ARPKD/CHF Newsletters from 1996 to 2000
Table of Contents

SPRING/SUMMER 1996; ISSUE NO. 1 4
INTRODUCTION TO THE NEWSLETTER 4
LETTERS FROM READERS 4
DIET CORNER 5
Fall/WINTER 1996; Issue No. 2 5
LETTER FROM THE EDITOR 6
HIDDEN GRIEF WHEN PARENTING A CHRONICALLY ILL CHILD 6
PREPARING YOUR CHILD FOR ORGAN TRANSPLANT 6
Mom's Thoughts 7
Dad's Perspective 7
TIDBITS 8
PREDNISONE—AN IMPORTANT MEDICINE YOU LOVE TO HATE! 8
COMMON SIDE EFFECTS OF PREDNISONE 9
"FAMILY NOTES" 9
SPRING/SUMMER 1997; ISSUE NO. 3 10
LETTER FROM THE EDITOR 10
RESULTS OF THE ARPKD FUNDRAISER 10
QUESTIONS YOU SHOULD ASK the salespeople if you are choosing an HMO for your child: 11
LIFE AFTER TRANSPLANT - Exploring the Myths and Realities 11
ORGAN & TISSUE DONATIONS: Some Facts To Know 13
SOCIAL WORKERS AS RESOURSES 13
Information and Referral 13
Advocacy 13
Counseling 13
How to get connected with a social worker: 14
WHAT TYPE OF PKD DO YOU HAVE? 14
THOUGHT FOR THE DAY..... 14
HOW NOT TO STARVE ON A RENAL DIET 14
FALL/WINTER 1997 ISSUE No. 4 16
LETTER FROM THE EDITOR 16
Dr. Campbell's Top Ten Ways On How Best To Get What You Want For Your Child From The Medical System 16
PARENTHOOD AFTER TRANSPLANT 18
TIPS AS AN ALTERNATIVE TREATMENT … 19
TOPIC: IS ARPKD REALLY A LIVER DISEASE? 19
WHAT IS CONGENITAL HEPATIC FIBROSIS? 19
FOUR MAIN PROBLEMS LIVER DISEASES MAY HAVE 20
BLOOD FLOW ABNORMALITY AND DUCTAL PLATE MALFORMATION 20

ARPKD/CHF Newsletters from 1996 to 2000
Table of Contents

WHAT ARE SOME OF THE CLINICAL MANISFESTATIONS OF DUCTAL PLATE MALFORMATION OR CHF? 20
WHAT ABOUT THE CHICKEN POX VACCINE? 21
SPRING/SUMMER 1998 Issue No. 5 21
THE FAMILY IS AFFECTED BY THE ILLNESS TOO 21
CAN MILK THISTLE HELP PROTECT THE LIVER? 22
GUESS WHO'S COMING TO DINNER! 23
QUESTIONS TO CONSIDER RE: HOSPITALIZATION 24
TRANSPLANT—Signs of liver graft rejection *by Jeffrey Punch, MD* 25
UNDERSTANDING THE TEEN YEARS 26
ABOUT SPLIT LIVER TRANSPLANTS 27
LETTERS FROM READERS 28
FALL/WINTER 1998 ISSUE NO. 6 29
LETTER FROM THE EDITOR 29
YOUNG TRANSPLANT RECIPIENTS—an age-appropriate guide to helping your child with a transplant 29
A noted physician finds tangible proof . . .WHEN SCIENCE INVESTIGATES PRAYER 30
SIBLINGS—The Children Who Sometimes Get Lost in the Raindrops 32
HELPFUL HINTS 33
SPRING/SUMMER 1999 ISSUE NO. 7 33
LETTER FROM THE EDITOR 33
A CHILLY PEAK AT THE NEAR FUTURE 33
WHAT IS POLYCYSTIC KIDNEY DISEASE? 34
AUTOSOMAL DOMINANT POLYCYSTIC KIDNEY DISEASE—(ADPKD) 35
AUTOSOMAL RECESSIVE POLYCYSTIC KIDNEY DISEASE—(ARPKD) 35
WHEN WILL YOUR CHILD NEED A LIVER TRANSPLANT? 36
About the liver 37
LETTER FROM A READER IN Hawaii: 37
IDEAS FOR SELF-CARE 38
FALL/WINTER 1999 ISSUE NO. 8 38
1999 Annual Conference-COMMENTS FROM ATTENDEES 38
CONGENITAL HEPATIC FIBROSIS—THE LIVER LESION OF ARPKD 40
Diagnosis 40
Treatment 41
GROWTH HORMONE THERAPY 41
ANYTHING BUT ROUTINE 42
GLOSSARY OF LABORATORY TESTS — A LOOK AT COMMON LAB TESTS 43
BLOOD TESTS: 43
URINE TESTS: 44
OTHER LABORATORY TESTS: 45

ARPKD/CHF Newsletters from 1996 to 2000
Table of Contents

BLOOD PRESSURE:.....45
WELCOME TO HOLLAND 45
SPRING/SUMMER 2000 ISSUE NO. 9 45
POLYCYSTIC KIDNEY DISEASE: AN OVERVIEW AND COMMENTARY *by Colleen B. Zak, RN* 46
Autosomal Dominant Polycystic Kidney Disease.....46
Autosomal Recessive Polycystic Kidney Disease.....47
Diagnosing PKD.....48
Health Maintenance for PKD:Interventions and Treatment.....48
Insurance.....48
PKD Research and the Outlook for the Future.....48
FOUR PKD MAGNET CENTERS of EXCELLENCE ESTABLISHED 49
POST-TRANSPLANT ISSUES: Missing a Dose: Does It Matter? 50
AN INTERVIEW WITH ELLIS D. AVNER, M.D.….....51
FUND-RAISING FOR ARPKD/CHF…. 53
"Study to block rejection of transplants seen effective".....55
QUESTIONS/ANSWERS 55
Transplant Q & A.....57
LETTERS FROM READERS 61
KIDS TALK! 66
FOR ARPKD LOSS ……..…..66
TO THE NEW ME 66
COPING WITH GRIEF 66
TIPS FOR THE BEREAVED COUPLE:.....69
SAD VACATIONS 70
COPING SKILLS FOR PATIENTS: Taking Control Control of Your Illness 70
RESOURCES.....71
BROCHURES 71
BOOKS TO ORDER.....72
OTHER PUBLICATIONS 72
WEB SITES:.....72
ORGANIZATIONS:.....73
BEREAVEMENT:.....74
QUOTE —.....74
DISCLAIMER.....74
HAVE YOU MOVED? 74
NEWSLETTER MAILING LIST 74
CREDITS AND ACKNOWLEDGEMENTS 74

SPRING/SUMMER 1996; ISSUE NO. 1

INTRODUCTION TO THE NEWSLETTER

by Colleen Zak

Hello. I am the mother of a child who has Autosomal Recessive Polycystic Kidney Disease (ARPKD). As parents of children with ARPKD, or as individuals with ARPKD, we share powerful common interests. Though each one of us may be at a different stage in the ARPKD process, from nearly normal to medically fragile, the initial grief that accompanies diagnosis, followed by the questions, fears, isolation, sadness, then the waiting and hope for the future, are universal.

There is a need to ease our journey through the roller coaster feelings and maze of medical and psychosocial issues. This is the place to voice yourselves, provide and maybe find solutions to your troublesome encounters, connect and network with others affected by ARPKD, and gather resources to provide us some control over our situations and guide our decisions.

My hope is to include families who have suffered the loss of loved ones afflicted with ARPKD. They are so much a part of the ongoing story of ARPKD. I truly believe a cure for ARPKD will be found. My greatest wish is for a cure to be found in time for my 4 year old son, Nolan, and all the other ARPKD children I have met. Maybe through our effort we will be led in that direction.

LETTERS FROM READERS

My husband and I have endured many confrontations with our son, 3 1/2, about taking his high blood pressure medication. About 6 months ago, we bought a pill dispenser. We thought that the more our son was involved with the dispensing, the more likely he'd be a participant at pill-taking time also. So, every Sunday, I give him his medicine container and he counts out 7 pills. Then he places each pill in its own compartment. (Of course, he also chooses which pill is going to be "Monday's pill", "Tuesday's pill", etc.) When it's time to take a pill he gets his pill box and retrieves his pill. We haven't had any confrontations in 6 months!
Submitted by Lisa Smith, Southbridge, Maine

Dear Parents,

My name is Donna Robbins. My little girl, Brooke, has ARPKD, and she was 12 years old last December 7th. When she was born she had grossly enlarged kidneys and respiratory distress. Within hours a medical team rushed her to the neonatal intensive care unit at Sparrow Hospital in Lansing, Michigan. At first her chances for survival were very slim, but after almost three weeks we took her home.

Through the next few months, one procedure after another was performed to insure the accuracy of the diagnosis, and much care was given to the management of her hypertension. Although medication could control the hypertension, nothing could be done for the Polycystic kidneys. Then, as if things weren't bad enough, when she was nine months old, a biopsy of her liver indicated involvement of that organ as well, and, as a result, pressure was already beginning to increase in the portal artery. The veins on her abdomen became very prominent during the first to third year of her life. (However, over the last 9 years, the process has slowed to a creeping pace.) We are on alert for a "bleed" in the abdomen or esophagus, but so far nothing has materialized.

There were many anxious moments spent in hospitals when she would become very ill with a virus of unknown origin, then pneumonia, and again with another virus. She fought each illness with the might of a warrior and defeated the enemy in spite of the odds. As time went by, she became stronger and her illnesses were not so severe as during the first few years of life. Maybe it was because I had learned to recognize the signs of something coming on, and was able to obtain rapid intervention. The Nephrology and pediatric team following the progression of Brooke's ARPKD are conscientious in every aspect of her care, and we have a genuine relationship with them. I attribute her well being and longevity to that fact; and that she has been provided a happy, stable environment, appropriate exercise and diet, and careful monitoring of her blood pressure.

It is very important that all involved in the medical, psychological and physical nurturing of your child work as a team to keep you

from being pulled in several directions. It is also very important that you, and your child (at an appropriate age), be allowed to participate in some of the decisions involving treatment strategies. There are many areas in which the child could be able to choose from a list of alternatives, all having the same end result. With involvement in the treatment process, there is a sense of control over what is happening.

Brooke is a beautiful, vivacious 12 year old with a gusto for life that won't quit and wants to do everything at once. Tap dancing and swimming are her favorite sports, and she does both very well. She knows the seriousness of her condition but keeps a healthy attitude for the future. Her fondest dream is ... "to be able to wear regular jeans after my enlarged kidneys are removed", and, "to meet Dolly Parton".

Even with all the support from caring doctors and family members, Brooke's life is not without adversities. Her peers make fun of her large abdomen by saying she is "fat", even though the rest of her anatomy is small. When she misses a lot of school because she is sick a lot she falls behind the class and is overwhelmed with all the make-up work. Other children enjoy visiting OUR home but none reciprocate with an invitation to THEIR home. I find it very hard to come up with any logical answer when Brooke questions "why?". The best I can do is surmise that they don't want to be responsible for a child with a medical problem. thus, my point being; it is of the utmost importance to educate the general public about ARPKD.

I would suggest that, if at all possible, join a group supporting ARPKD education and research; and, if there isn't one in your area, inquire about getting one started. If you don't know where to begin, contact the editor of this newsletter and I'm sure you will be pointed in the right direction.

You cannot imagine the relief I felt when I was finally able to start networking with other people in similar situations. It has given me a whole new perspective on life with ARPKD; and now I know there is a future...a VERY GOOD FUTURE...if we all pull together.

—submitted by Donna Robbins, Bath, Michigan

DIET CORNER

Suggestion:

Pepsi, Coca Cola, and most "dark colored sodas" contain stabilizers and preservatives in the form of Phosphoric Acid and Sodium Benzoate.

Both Phosphorus and Sodium are harmful to the kidneys. They should be avoided if possible.

The best choice, if you're looking for a drink, would be bottled water containing no sodium - such as Poland Spring or Appalachian Spring Water. Do not drink Perrier or any other "mineral" water. Also, avoid drinking tap water if it is "softened." If water is softened, sodium (salt) has been added to the water. If you eat out a lot in restaurants, most restaurants soften their water so beware. I would suggest ordering or bringing your own bottled water whenever possible.

Coffee and/or tea are OK in moderation (no more than 2 cups per day) EXCEPT if you have high blood pressure or are on high blood pressure medication. Both beverages cause blood pressure to rise. This puts added pressure on the kidneys and on renal function and is most counterproductive.

Remember to read labels and avoid beverages with added phosphorus and sodium.

Other OK drinks include cranberry juice, lemonade (real, not artificial) and apple juice.

POWERADE TO THE RESCUE

I am not a dietitian but my ARPKD child's doctor has been treating her lowered CO_2 (azotemia) with sodium bicarbonate tablets (instead of the traditional Bicitra or Polycitra). For the past year, we have been supplementing the sodium bicarbonate tablets with large amounts of PowerAde sports drink**. This treatment has kept her CO_2 numbers above the minimum of 20.** Other drinks with sodium citrate are also helpful. It has not affected her blood pressure either. If you have questions about this, write to me through this newsletter.

—Donna Robbins

Fall/WINTER 1996; Issue No. 2

LETTER FROM THE EDITOR

Hi, everyone!
It is nearly press time for the September, 1996 ARPKD/CHF Newsletter and I have yet to write "a letter". The last minute is here, but I am far from home. It is our family vacation and I feel relaxed. It seems I don't have a care in the world. I can almost forget that my 4 ½ year old son has ARPKD, until I realize my hesitancy to begin a conversation with the woman sitting directly across from me. Her children appear to be my children's age, but I don't speak. I wish not to witness another surprise reaction to Nolan's age and below average stature. Recently, some outsiders believe my sons, Nolan and two year old Evan, are twins. The need for daily growth hormone injections may be obvious to some, but, as Nolan's parent, it is an agonizing decision to make.
Yet, this is a minor situation in comparison to what other ARPKD families undergo.

So why do some children with ARPKD die soon after birth or experience a lifetime of medical problems while others have decades of relatively good health? The ARPKD "experts" can't answer that question nor the vast majority of questions in our unending search for information.

Nevertheless, I believe we can help the medical community answer our questions, achieve better clinical treatment, and eventually find a cure for ARPKD. We can do this by striving to support a medical symposium where ARPKD scientists and experts converge to discuss and brainstorm ARPKD; produce an educational video specific to ARPKD; provide an international "library" for ARPKD families and physicians. All of these goals can be attainable ambitions with the proper finances.

Several fundraising events are being evaluated. One of these is a children's book sale. Details are being compiled and that information will be mailed to you when completed. I would like to ask that everyone participate as this is the beginning of our ARPKD benefit project pursuit.

As always, I want to hear from you. Your thoughts, ideas, and suggestions widen my perspective, motivate me, and give me a clearer direction for our needs and goals.

Sincerely,
Colleen Zak

HIDDEN GRIEF WHEN PARENTING A CHRONICALLY ILL CHILD

The grief of parents whose child has a serious or chronic illness is often hidden. In the busyness of caring for all the medical and emotional needs of a sick child, parents can often put their feelings on hold. Because few people, including family, friends, and professionals, take the time to inquire about those feelings, parents can begin to believe that their emotional responses are abnormal and that no one feels as they do.
However, it is very common for intense feelings of grief to be triggered for parents, not only at the time of diagnosis, but also over and over during the illness. This can happen at medical crises, at times of missed opportunities for their child, and even at simple occurrences such as seeing another child who is healthy. Parental grief is not simply feelings of sadness and pain. It includes times of shock, confusion, numbness, memory loss, anger, fear, anxiety, and guilt. These and many other feelings are normal responses to dealing with an abnormal situation that has so many unknown factors. Because all parents seem to have a basic belief that a "good parent" should protect a child from illness and suffering, many parents blame themselves when their child gets sick. It is hard work to unlearn and ignore this kind of myth.
Family members also express their grief in many different ways. Often this can be a source of friction, especially if, for example, one parent needs to talk, while another needs to withdraw. It helps if each parent can recognize his or her style of dealing with grief, and can give one another the freedom to be different. Having other people to share with often helps parents to do this more easily.
Other methods for parents to deal with grief include: becoming as informed as possible about the illness and ways to live with it, being a part of the treatment team, joining a support group of other parents of chronically ill children, and learning to reach out for help to others in the community.—Reprinted from the *HOME CARE FAMILY NEWSLETTER*,
Children's Hospital, Philadelphia, PA

PREPARING YOUR CHILD FOR ORGAN TRANSPLANT

Mom's Thoughts

Where do I begin? First I should introduce myself to help you understand where I'm coming from. I'm the mom of Derek James Doyle — who at eight years old has already had two kidney transplants.

It's hard to think back on how I felt before Derek's transplants. Doctors, hospitals, nurses, and medications have been a way of life for us since Derek was six months old.

We feel we are very fortunate that if Derek had to be chronically ill, he grew up that way. He knows nothing else, and as a result he thinks this is normal. I believe we worked this to Derek's advantage; he has grown up with a healthy attitude about himself.

We tried to follow the wise words of Dr. Bernard Gauthier, Derek's pediatric nephrologist from Long Island Jewish Hospital. He told us, "The worst thing you can do for Derek is to make a problem of a problem."

We spent the first years of Derek's life not necessarily preparing him for his first transplant but helping him become a normal, healthy kid, so by the time he needed his first transplant, it was just another day in his life. We were all excited about his new experience, just as another child might anticipate his first day of school.

Derek was totally prepared for a successful transplant. He played surgery with his friends. He was able to take friends through each step of the process, explaining how the doctors would prepare him for surgery, what tubes he would have in him afterwards, and what meds he would be taking. We thought we had all the bases covered.

When we went through the pretransplant workup, the doctor also told us about the outcome statistics. We listened, but we didn't comprehend. We were totally convinced we were in the successful group. As a result of not being totally open-minded, we didn't totally prepare Derek. We never once talked about what happens if the transplant doesn't work.

That's what happened—four days after transplant. One minute Derek was chasing the nurses down the hall, and the next he was back in surgery having his dad's kidney removed and beginning dialysis. We weren't prepared for any of this.

I bring this up not to focus on the negative but to help you learn from our mistakes. You need to talk about the "what if" to help realistically prepare for your transplant.

We were fortunate, and it was not as devastating mentally for Derek as it was for us. Derek spent the balance of that summer in and out of hospitals while we got him started on home dialysis. Thanks to Derek, it turned out to be a great summer. The dialysis machine became the "tic toc." Thanks to my husband Donnie we always managed to find some humor in our lives, especially when it came to maintaining Derek's health.

Unfortunately, we had no other eligible donors in our family. Once Derek had recuperated from the loss of the first kidney and we had established a "new normal" in our life, we began our search for another kidney. This was a family decision, and I feel fortunate that our family bonded and became an even stronger unit. I think our salvation was that we always talked about everything—good and bad.

I remember when we first learned about what Derek had in store for him. He was just six months old (it was on the eve of his first Christmas), and I sat him on the kitchen table and explained to him as much as I could. I remember people asking if I thought he knew what I was talking about. In a way that didn't matter; I was telling the truth, and that was important to me. It helped establish our bond of trust. Fortunately, not quite one year after Derek's first transplant, a cadaver kidney was found for him. To get to the hospital on time, we had to arrange for a Lear jet. We made it!

Here's when our open communication almost got us in trouble. As we were wheeling Derek into surgery, he decided he didn't want to go through with it. Now what do we do? We knew we could lie and say OK, and he would never remember after surgery. But we also had six years of trust going for us, and many years ahead of us. We decided lying wasn't an option. Instead we asked for a little more discussion time and did some fast talking. The final decision was his.

Derek's first goal after surgery was to try to figure out how to turn his urine green, since it was April Fool's Day. The doctors had warned us that he may not start urinating for 24 to 48 hours because the kidney ended up being a little older than desired. Derek and his dad began calling his urine "liquid gold," and to this day the flow of the "liquid gold" is a momentous occasion at our house.

We flew home ten days later with a new bag of medications and a definition of normalcy we had already started adjusting to. Thirty days later we were back, being treated for rejection. There wasn't much time for euphoria, but we managed to view the situation positively and see it as important to making new adjustments.

After another thirty days, we were back again with another rejection. Then we decided it was time to become a "transplanted family." Derek was approaching school age, and in order for him to have a more normal life, we decided to move so we could be closer to our transplant center.

That turned out to be a good decision. Derek has had ten rejections, but they are not nearly as disruptive to his life as when we lived far away from the University. Derek has become very active in a local theater group and has even performed on stage with tubes hanging out of him. He has been in a school play at 9 A.M. and had a biopsy by 11 that same morning. We have become a very efficient team—doctors, nurses, transplant center, and family.

What's the big picture for Derek? The same as his daily focus: to keep growing mentally and physically so he can go out and feel good about himself and accomplish whatever he sets his mind to.

—Kathi Doyle

Dad's Perspective

The most constant thing in life is change.

I always felt that statement was fuel for thought—in case I wanted to change something in my life or if that "turn in life" decided for

itself there was going to be a change for me.
There was always the amusing minor change I could adapt to with a shrug or a smirk, hoping I either benefited from it or got away with something. But the day they told Kathi and me that our six-month-old son had a kidney disease and would need to have both kidneys removed, I neither shrugged nor smirked. Instead I tried to absorb every one of those new words they were tossing out and to begin to focus on a new world of blood chemistries and medications.
I listened intently, feeling I was preparing to stand up in a tornado. As Kathi and I walked down the hallway, we held each other and then cried for many minutes.
I think we did the most obvious and natural thing. We let our emotions out, and we did it together. After we regrouped, stopped, and caught our breath, we began to make new plans. We would have to have some crash courses in kidneys and how they work. And we would need to try even harder to instill positive values and goals in ourselves and our son.
Looking back, I learned that if the parents keep things in perspective and move on, one step at a time, the kids will do just fine. If you panic or lose your sense of balance in life, the children will have a much harder time. Most of us can get through anythin if we view it one day at a time.
I assume if you're reading this, you're going through similar "adjustments" or preparing to do so. If you're now at Step A and you care enough to get to Step B, you'll make it. Educate yourself. You don't have to be an expert, but get enough information to become comfortable with the new reality before you.
Don't be afraid to ask questions, and don't neglect or ignore the nagging "what ifs" that keep you awake at night. If you care and love, you can make it. Lots of others have, including us, and we're here to help if you need us.
—Donnie Doyle

—"Reprinted with permission from *Encore,* a quarterly magazine for individuals who have undergone transplantation. For information, please write to: Chronimed Pharmacy, 13911 Ridgedale Drive, Minnetonka, Minnesota, 55305.

TIDBITS

Did you know that cow's milk and baby formula have three times as much protein and sodium as breast milk? Both protein and sodium make the kidneys work harder than breast milk. (Please be aware that growing children need protein for growth. If you are thinking of restricting protein in the diet, please discuss this with your Pediatric Nephrologist first.)
For infants and children who cannot swallow pills, here is one solution. Your local pharmacy may be able to make a "suspension" specific to your child's medication and dosage needs. This medication is in liquid form and may be flavored as watermelon, raspberry, banana, or grape. If your pharmacy is inexperienced or unable to provide a suspension, you may try Ridge Road Pharmacy in New Jersey at 201-438-2218, or fax 201-438-0468 for assistance.

PREDNISONE—AN IMPORTANT MEDICINE YOU LOVE TO HATE!

A commom medicine used after transplantation is prednisone, which belongs to a group of drugs called corticosteroids. Prednisone is actually its generic name; you may know it by a brand name such as Deltasone® or Orasone®.
Corticosteroids are important for treating a number of health conditions besides transplantation. For example, corticosteroids are used for asthma, gastrointestinal illnesses such as Crohn's disease and ulcerative colitis, and arthritis. Although very effective, corticosteroids have many side effects. Thus, prednisone is a drug many people love to hate!
Prednisone is a manufactured medicine that is similar to the hormone cortisol, which is made in your adrenal glands. Located next to the kidneys, adrenal glands make about 20 milligrams (mg) of cortisol each day—equal to about 5 mg of prednisone.
Following organ transplantation, prednisone is prescribed with other drugs to suppress the body's immune system and prevent rejection. Without prednisone and other immunosuppressant drugs, the immune system would recognize that the transplanted organ is different from your other organs and attack it. Specifically, prednisone stops the production of interleukin-1, a protein used by cells in the immune system to "talk" to each other. Prednisone also decreases inflammation and swelling.
Most often, prednisone is given as a tablet that you swallow. But it can also be given intravenously (through the veins) when someone cannot take medicine by mouth or when it is being used to treat a rejection episode. Doses used right after transplantation or during rejection are pretty high. A typical goal is to decrease the prednisone dose to a maintenance level of 5 to 10 mg per day by one year after transplant.
In the following section are prednisone's most common side effects, and tips to try to make them more tolerable. Most people find the side effects of prednisone are more bothersome and frustrating than any other medication. Fortunately, when the prednisone dose is lowered, the side effects also diminish. The list of side effects is long and not very pleasant, but keep in mind the good prednisone does.
It is important to never stop taking your prednisone or lower your dose unless your doctor says so. Suddenly stopping prednisone may

make you feel worse than before by causing extreme tiredness, nausea, vomiting, headache, dizziness, fainting, and joint and muscle pain.
Although prednisone may be a medicine you love to hate, try to remain patient and learn about it and your other medications by talking to your transplant staff, doctors, pharmacist, family, and support group. Prednisone's benefits outweigh any side effects in the long run.

—Gina Lemke, Pharm.D.
Chronimed Pharmacist

COMMON SIDE EFFECTS OF PREDNISONE

Nausea, vomiting, stomach pain
Prednisone can irritate the stomach lining, so it is important to take it with food or milk. Since it can also increase acid in your stomach and lead to ulcers, you may be asked to take an antacid such as Maalox□ or prescribed a medicine called ranitidine (Zantac□) or famotidine (Pepcid□). If the nausea and vomiting continue, call your transplant center or doctor.
Increased appetite
Many people on prednisone say they can eat and eat and never get full. Of course, if you do eat and eat, you'll gain weight, so it's important to stick to a well balanced diet and exercise program.
Weight gain, swollen ankles and feet
You may also gain weight because prednisone causes the body to hold on to sodium (sodium retention). When the body retains sodium, it retains extra water causing swelling of the ankles and feet. Extra fluid can also increase your blood pressure, so it is important to limit your salt intake.
High blood sugar
Prednisone causes the body to make more sugar. A diet suitable for diabetes will be important, as well as regularly checking your blood sugar levels. (A dietitian can help with eating plans.) If you had diabetes before transplantation, you may need to take more insulin because of the prednisone. Others who didn't have diabetes before their transplant may need to take insulin or oral diabetes medicine.
Muscle weakness and pain
Prednisone may weaken muscles, especially in the thigh and upper arm. Therefore, it is very important to strengthen the muscles with regular exercises. If leg and arm pain does not go away with exercise and stretching, call your transplant center or doctor.
Osteoporosis
Exercise is also very important in preventing osteoporosis and bone fractures. You may be asked to take calcium supplements or vitamin D such as Rocaltrol to strengthen your bones.
Mood swings, irritability
Prednisone may cause severe mood swings, especially when the dose is high. You may feel 'up' one day and depressed and blue the next. Try to stay positive and remember that you will feel more like yourself as the prednisone dose is lowered. It is also helpful to talk to your transplant team, family, and support group about your feelings.
Eye problems
Blurred vision is common after transplant when prednisone doses are high, but usually improves after six months. If your vision continues to be blurry or you have decreased vision, you should see an eye specialist (opthalmologist). Other eye problems such as cataracts may also occur.
Acne, dry skin
Prednisone may cause skin changes such as acne on your face and back. Keep your skin clean by washing two or three times a day if possible. Some people may experience dry and sensitive skin. In this case, it is also important to keep the skin clean as well as protected from bruising and sun exposure.
Higher risk for infection
Because prednisone and the other immunosuppressant medications decrease the body's ability to fight infection, you have a greater chance of becoming sick than other people. Call your transplant center or doctor to report any fever or illness.

—"Reprinted with permission from *Encore*, a quarterly magazine for individuals who have undergone transplantation. For information, please write to: Chronimed Pharmacy, 13911 Ridgedale Drive, Minnetonka, Minnesota, 55305."

"FAMILY NOTES"

Dear fellow ARPKD parents,
Please allow us to introduce ourselves. We are Dave and Christy Runner. We live in Riverside, CA, about sixty miles East of Los Angles. We have four daughters, Suzanne 16, Jennifer 15, Vanessa 12, and Rebekah 10. Both Vanessa and Rebekah have ARPKD. Vanessa was diagnosed when she was nine weeks old and Rebekah was diagnosed at 5 years. Vanessa is currently taking Vasotec, Cimetidine, Sodium Bicarbonate, Feosol, and a daily vitamin. Rebekah is also taking Vasotec, and a daily vitamin. They see their pediatric nephrologist every six months at Loma Linda Medical Center. Except for their periodic check-up, they lead a very normal life.
Rebekah is in the fifth grade and is a straight A student. She likes playing catch with her Dad, playing the piano, and eating at McDonalds!! Vanessa is in the seventh grade and loves to socialize a little to much. She loves to tap dance and talk on the phone. All four of our girls know about ARPKD and we try to answer all their questions. Suzanne and Jennifer take a big interest in their sisters condition and are very helpful with their doctor visits. ARPKD has made us a stronger and closer family. We try to teach all our daughter's to live life to the fullest and to keep a positive attitude for their future's!!!

—Dave and Christy Runner, CA

SPRING/SUMMER 1997; ISSUE NO. 3

LETTER FROM THE EDITOR

Lord, give me peace. Or a miracle to heal my son of PKD. That's the big one I pray for every Sunday and often in the evening. Most days are upbeat, productive and full of contentment. There is much hope for the future. For days, weeks, even months at a time, there is no anxiety and little fear. Till it is time to visit the nephrologist.
It usually starts several days beforehand. Moments of uneasiness that occur more frequently as the scheduled time approaches. What seems like irrational feelings of apprehension and dread. No matter how well your child with this chronic disease is doing, those feelings are looming in the back of your mind. You have been told that eventually kidney failure and liver fibrosis will occur. It's coming, that much you know. This waiting, at times, can be your own personal hell. Going home from the doctor's visit is always a welcomed relief.
Then a lab value reveals slight changes for the worst, again the fear returns. Oh, how you wish you could forever rid yourself of the predicted outcome. This insidious deterioration of your child's kidneys and liver. A fear of death. The mind goes into overdrive and begins to analyze everything. Is that why he often seems so tired these days? Perhaps that is the reason he is less interested in food lately. Oh he looks pale....and so on.
It seems there is nothing you can do but wait for the inevitable to occur. But is that correct? Perhaps there is something you can do. Perhaps you could start by altering your attitude and perception you may have. Learn everything you can about your child's condition. Ask questions. Empower yourself with knowledge. When you are well-informed about your child, then you can advocate in their best interests. Get involved. Write an article for the newsletter or your local paper. Start a Support Group. Organize a fund-raiser. Educate others about this progressive disease. Your initial involvement may be the most difficult step, but please remember, you can make a difference. Personally, I can not just wait while the weeks turn into months and the months into years. There are far too many unanswered questions about this disease for me to wonder about. How would I forgive myself to think, that if I could have made any difference for the good of my child, I chose instead not to get involved. To always play it safe, accomplishes little. Certainly, there are not enough PKD answers for even the PKD "experts".
Yes, I do have moments of fear and worry. When I shake loose from it's grip I am more determined to make a better life for my child. I am his most effective advocate. I must do everything that I can to improve his chances in this life. Every effort may push closer to the realization of better clinical treatment or a cure. I encourage you to do the same. Close the door on the darkness and open the window for change and opportunities of improvement, by becoming involved. It could be very therapeutic for you and your loved ones.

—Colleen Zak, Editor

RESULTS OF THE ARPKD FUNDRAISER

A total of 50 catalogs and accompanying fundraising materials were distributed to ARPKD families for participation in the ARPKD fundraising children's book sale. From those families, sixteen participated in the fund-raiser. A total of $3106.25 of "Usborne" products were sold by participating families. Costs incurred to purchase "Usborne" catologs, postage fees, photocoping of letters, order forms and additional materials came to a total of $176.10. The ARPKD project profit of 40% of the $3106.25, minus the costs to organize the fund-raiser, came to $1066.40. Below is a summarization of the fundraiser and its results.

Most families sold an "average" amount of merchandise between $100.00 and $200.00 dollars, with three families selling less then $100.00 and two families selling in the $400.00 range. Lisa Brudos, from Illinois sold a whopping $490.00 in "Usborne" products and was the winner of the 5% in free "Usborne" merchandise, which came to $151.46.

Congratulations Lisa!!!

I would like to thank everyone who participated in this fund-raiser, your efforts are appreciated. The plan is that the fundraising results will help with the production of educational video specific to ARPKD. If anyone would like to help financially contribute to the production costs of this, please contact the ARPKD newsletter editor.

Total "Usborne" products sold.....$3,106.25

Cost of "Usborne" catalogs and S/H.....46.00

Photocopying of materials.....12.50

Shipping of packets to families.....106.85

Shipping result to "Usborne".....10.75

Total ARPKD project profit.....$1,066.40

QUESTIONS YOU SHOULD ASK the salespeople if you are choosing an HMO for your child:

- 1. Can I see the same doctor or dentist I have now if I join?
- 2. Is the hospital where I take my children part of your health plan?
- 3. Can each of my children have their own doctor, or do they need to see the same one?
- 4. What do I do if I am out of town and my child needs to see a doctor?
- 5. If I don't like my doctor, how can I change?
- 6. Are the hospital and doctors in your plan near my home?
- 7. If I want to stop using your plan, how do I quit?

To choose the best plan for your child or family, ask questions about the plan, don't sign up until you understand the plan, and check to see if your doctor takes the plan. If you are not sure, take the salesperson's card and tell them you will call when you are ready.

LIFE AFTER TRANSPLANT - Exploring the Myths and Realities

The optimists call it an opportunity for a new life.The pessimists say it's trading one set of problems for another. But any way you look at it, life after an organ transplant is very different.

Surgeons often promise those close to dying and waiting for a new organ that transplant will mean a new life or a second chance at life. Surgeons prepare the potential recipients fairly well for the complicated and often lengthy surgery. Fortunately, most survive.But unfortunately, recipients are not always well-prepared for their "new" life. The question remains: **Is that new and different life necessarily better?**

"Absolutely," said Joyce Willig, a Fairfield, Connecticut, resident who received a new liver and new life at Pittsburgh's Presbyterian University Hospital 12 years ago.Willig is the cofounder and co-president of the Connecticut chapter of the American Liver foundation and a founding board member of TRIO (Transplant Recipients International Organization). "And," Willig said, "I don't agree with the uninformed that you are trading one set of problems for another. Life for me is far superior."

John Fung, M.D., Ph.D., chief of the Transplantation, Surgery Division at the University of Pittsburgh's Presbyterian University Hospital, said he doesn't promise his patients a new life. "Transplantation offers not a new life, but rather another opportunity for a better quality of life."

"Most of the surveys show that the vast majority of transplant recipients have a better quality of life," Fung said. But with that new chance of a better quality of life, patients are "trading the disease process for the risk of the transplantation procedure and all of the attending complications from the medications they will be on."

Cyclosporine, Imuran, the experimental FK506, and the steroid prednisone are the four most common drugs used to prevent organ

rejection. Each carries a host of possible side effects. These medications "each affect some part of the body's ability to fight infection and rejection and each has a different set of side effects," Fung said. The effects range from weight gain and puffy cheeks to more serious problems, such as an increased risk of skin cancer, chance of diabetes, anemia, kidney toxicity, osteoporosis, hand tremors, and even seizures or death. "Not everybody gets all of the side effects," Fung added. "Most people have some but not all of them. All we can do is give them some type of an expectation of what their quality of life will be like." A lot of their adaptation depends on their motivation and expectations based on what they've heard before their transplant, he said.

"Attitude is everything," Willig said. "Many of the people I come in contact with are anxious." Willig receives at least a dozen calls a month from people who have had or who are waiting to have a transplant. "I would much prefer to emphasize the wonderful quality of my life over the past 12 years since my transplant than to dwell on the ups and downs of the recovery period associated with any operation. I don't want them to give up. I don't want to deter anyone from going for the gold. I have a positive attitude, and I work hard at it. I want them to know how good it is now," She said. "Everybody experiences some changes in their life. Some people adapt easily and some people don't," Fung said.

"No one goes through this experience without some kind of a change and a different view of life," Said Mary Grace Fitzgerald, R.N., M.S.N. Something the recipient has a difficult time adjusting to his or her new life.

Fitzgerald is a psychiatric nurse clinical specialist with the liver transplant service at Presbyterian University Hospital. "I get phone calls from doctors, nurses, roommates, and family members. What I essentially do is to try to help the patient and the family get through a tremendously stressful time."

"Sometimes people have expectations that they will be able to do things they weren't able to do before their surgery," Fitzgerald said. Sometimes these expectations are fulfilled, and sometimes they are not.

"There is a long road to recovery. The patient has to work at it," Fung said. Last year, the surgeons at Presbyterian performed about 400 liver transplants. Of those 400, 15 percent did not survive. Of the remaining 85 percent, only about 10 to 15 percent needed some help with adjusting, Fung said. "I was told from the beginning that I was going to die within two years," Willig said. "Adjustments to life after my transplant were far easier for me than living with a chronic, fatal disease-primary biliary cirrhosis. There was no adjusting to that."

In some ways, there is a myth surrounding organ transplantation. There are many things people can't go back to, Fitzgerald said. Take jobs and careers, for instance. "The typical type-A man has a work, work, work attitude, and all of a sudden he is sick with liver disease and in the hospital. Before his liver disease he never had to face death or even spend enormous amounts of time with his family. Work was what he did, and that was how he identified himself." Depending on the type of job, some people can return to work. Before her transplant, Willig held a part-time position as a writer for the Connecticut Jewish Ledger. Today she is the editor of TRIO's quarterly newsletter "Lifelines" and most of her writing now is transplant-related. "I've become a professional speaker promoting organ donation and liver wellness. That's become a new career and an extremely rewarding one."

Some recipients are not as lucky as Willig. Those who held positions in health care or heavy duty construction or those who worked with toxic chemicals may be discouraged from returning to their positions, Fitzgerald said.

But every patient and activity is judged on an individual basis, Fung said. "What we try to do is give them reasons not to do things on a medical basis." He encourages good hand washing and the warring of gloves or even face masks when around a hazardous chemical. "If there is a question about an activity, it should be brought up before resuming that activity," he said.

"Many liver patients end up on disability before their transplant, and then when they are well they don't have a job to go back to," Fitzgerald said. What she does then is to try to redirect recipients or help them make some adjustments to their past lifestyle. For example, hands-on gardening is risky for recipients because of the possibility of coming in contact with bacteria or fungus. But some recipients can continue gardening by wearing gloves when gardening and using a nail brush to scrub under the nails afterward. Taking "gardening away would be like taking their life away. That's who they are," Fitzgerald said.

"We are enormously changed by the transplant," Willig admits.

Fitzgerald tells the story of a woman in her mid-60's who had a liver transplant. The woman was hospitalized for a long time, during which her husband assumed all of the household duties. Once the woman felt better, she wanted to resume cooking, but she couldn't find the potato peeler . When she asked her husband where her potato peeler was, he argued it was now his potato peeler and he had rearranged the kitchen the way he wanted it. He said he enjoyed cooking and intended to continue. The wife said that was fine with her and she turned and went upstairs to start writing a book, something she had always wanted to do. "They reversed roles very nicely," Fitzgerald said. But in some situations it is not so easy.

Most of the patients Fitzgerald sees are "not mentally ill." They are just having an awful time adjusting to the medical system, their illness, the side effects of medications, and the fact that they no longer control their body. "It's very difficult to give up control of themselves," she said.

Willig never suffered the adjustment problems that some recipients have. But she, too, has had her share of side effects. Sometimes, she said, it's as if she takes a step backward. But, she adds, she is still, always, moving forward.

Willig was hospitalized for two months at Yale-New Haven Hospital before spending five months at Presbyterian University Hospital for her transplant. Because of too much bed rest back then, she developed a permanent foot drop. It took four months to learn how to walk again.

Today, 12 years later, she wears a leg brace and walks with a slight limp. "It's the only visible indication that I've had a transplant. I'm not going to be a ballerina, but I am able to get where I want to go, when I want to go," she said with a laugh. Willig has a permanent hearing loss caused by having to take too many antibiotics. She's been diagnosed with osteoporosis and had to have a hip

replacement because of it.
Despite everything, Willig maintains an inspirational positive attitude and only views her transplant as a positive thing. She meets and talks to people all over the country about organ transplantation. "I tell them to have hope, have faith, and to believe in miracles. The transplant was my miracle and life is much more pleasurable now."
by Jackie K. Shea

Jackie Shea is a free-lance writer who lives in Meriden, Connecticut, with her husband Mark and their two daughters, Jessica and Lisa. Jackie underwent a liver and bone marrow transplant on June 7, 1992, at Pittsburgh's Presbyterian University hospital..

—Reprinted with permission from *ENCORE*, Spring 1994 Issue. For information please write to: Chronimed Pharmacy, 13911 Ridgedale Dr., Minnetonka, Minnesota 55305 (per their request)

ORGAN & TISSUE DONATIONS: Some Facts To Know

- Donors can range in age from newborn to 75.
- You can designate your organs and tissues for donation by signing and carrying an organ donor card.
- You must inform your family of your wishes to be an organ and tissue donor. Consent from next of kin is required before donation can proceed.
- One donor can help or save the lives of as many as 25 transplant patients.
- All major religions support organ and tissue donation.
- Organ and tissue donation can occur only after an individual has died.
- Donation of vital organs is considered when a person is brain dead. The brain has permanently ceased functioning, while the heart, liver, lungs, kidneys and pancreas continue to function with artificial support.
- Tissues, such as eyes, skin, bone and heart valves can be donated up to 24 hours after death.
- Donation can be considered only after every effort has been made to save the patient's life.
- Organ and tissue donation does not interfere with funeral services.

For more information or a donor card, please contact:

1-800-KIDNEY-1
or
Delaware Valley Transplant Program
Rodin Place, Suite 201, 2000Hamilton Street
Philadelphia, PA 19130-3813

SOCIAL WORKERS AS RESOURSES

You may be hesitant to call on the services of a social worker because of confusion about how a social worker can assist you. A common misconception is that social workers only become involved in a child's case when the parents are doing something wrong. This is not true! Asking for help from a social worker does not mean that you are weak or that you are a bad parent. Caring for your chronically ill child can be a complex and stressful process and it is reasonable to expect that you and your family may need assistance dealing with the many issues involved.

There are 3 main areas in which a social worker can assist you:

Information and Referral
First, a social worker can help you locate the resources you need for your child. Whether that means something like finding respite care or seeking financial resources for specialized equipment, involving your social worker can make the process easier.
Advocacy
You have likely found that caring for your child involves dealing with many different agencies and professionals. At times this can be confusing and frustrating. Sometimes you may feel that you are running into obstacles in providing what your child needs. In such cases, a social worker may be helpful for advocating for you and your child to receive the required care and services.
Counseling
Children's Hospital social workers are sensitive to the issues that the families of chronically ill children face. You do not have to be

"going crazy" to ask for help. A social worker may be helpful in assisting parents to handle the emotional strain of caring for a chronically ill child.

How to get connected with a social worker*:*

If your child is an inpatient, you can tell the nurse that you would like to see a social worker. Each inpatient unit in the hospital has a social worker assigned to it. If your child is an outpatient, call the social work office at your local hospital. The secretary or intake social worker will be able to refer you to a social worker who can best help you.

—Written by Trish Franklin, Social Work Intern. Reprinted from the August-September 1995, *"HOME CARE FAMILY NEWSLETTER"*. The Children's Hospital of Philadelphia.

WHAT TYPE OF PKD DO YOU HAVE?

Infantile PKD, Early Childhood PKD, Late Childhood PKD, Adolescence PKD, Early Onset PKD, Adult PKD, ect...

What do all these names mean? How many types of PKD are there? This is sooo confusing! Here is the explanation. Either a person has Autosomal Recessive Polycystic Kidney Disease or Autosomal Dominant Polycystic Kidney Disease.

In the past, when the diagnosis of ARPKD was made, that diagnosis included the age of discovery, when the onset of symptoms occurred. For example, if an infant was discovered to have been born with ARPKD, the diagnosis of Infantile PKD would have been given. If that same child was born without symptoms and no ARPKD problems presented until adolescence, the diagnosis would have been Adolescent PKD.

It is now known that ARPKD is a continuum of a disease process, with kidney and liver symptoms that vary greatly from one individual to another. This is the reason why the term Infantile PKD is used today. Antiquated medical information still exists in medical textbooks describing and defining ARPKD.

As for Autosomal Dominant Polycystic Kidney Disease, there are children with this "Adult" form of PKD who are seen by Pediatric Nephrologists and may be labeled with the diagnosis of Early Onset PKD. Characteristically, symptoms do not become present until the 3rd or 4th decade in life.

Within the last few years, three types for Autosomal Dominant Polycystic Kidney disease have been defined, hence the names PKD-1, PKD-2, and PKD-3.

The gene for ARPKD is close to being identified.

THOUGHT FOR THE DAY.....

Making the decision to have a child is momentous - it is to decide forever to have your heart walking around outside your body. - Author Unknown

HOW NOT TO STARVE ON A RENAL DIET

(The following suggestions are only to be utilized if there is considerable loss of renal function. Renal diet restrictions are <u>not necessary unless prescribed by your physician</u>. Please refer any questions you may have to your child's specialists.)

The diagnosis of renal disease can be overwhelming. Just when you thought you were beginning to cope with the situation, in walks the dietitian to talk about your diet. Your new diet can be controlled in any or all of the following: Protein, sodium, potassium, phosphorus, and fluid. (For children on a renal diet: Protein is not controlled or restricted but rather is given in moderation as protein is needed for proper growth.)

At first glance, the *avoids* list look longer than the lists of foods allowed. It seems, all of your favorite foods are on the wrong list. Your old standbys of canned soup and cold meat sandwiches top the *avoids* list. Then comes the question, is there anything left?

There are no doubts that following a renal diet is not an easy task. Usually, it means giving up a lot of your old favorites. In the positive, it means making better choices, putting the "right" foods in the grocery cart, & changing the way you think about food.

Today, people are concerned about weight control and lowering their serum cholesterol and triglyceride levels. Certainly, this is a very appropriate goal, and one of the best ways to achieve it is to follow a low fat and low sugar diet. Thus, many of us have been making food choices that are sugar free and as low in fat as possible. If you are following a diet that requires moderation of protein,

many of your ideas about food will have to be reevaluated. You often have to think fat and sugar to supply enough calories to avoid unwanted weight loss, that could ultimately lead to malnutrition.

Now, let us look at each food group in your renal diet individually. It is best to stop looking at the *avoids* list and start concentrating on the foods allowed. Educate yourself, read labels, be creative and think fats and sugars to supply those necessary calories.

Meat and Meat Substitutes

The recommendations for the meat group can be quite shocking when you think that an 8 ounce steak could wipe out your meat allowance for two days. While providing you with two much protein it failed to give you substantial calories. Therefore you must think of ways to supply those missing calories, without increasing your protein intake above the recommended level. To get the most from your meat servings: fry your meat and eggs in oil or margarine, top a broiled hamburger with mayonnaise or sautéed onions, make meat salad sandwiches by adding mayonnaise to tuna, salmon, turkey or chicken.

Milk and Milk Products

The milk list can be shocking as well. Chances are you only have one serving listed here, but do not underestimate its caloric contribution. A half cup portion of skim milk provides 40 calories and whole milk provides 80 calories, but what about using half and half on your cereal for 160 calories. If you do not care for cereal, use that cream to make some pudding or pour it over fruit.

Undoubtedly, you will find that your milk allotment is not sufficient for your needs. Substitute liquid non-dairy creamer for milk in all of your recipes (pancakes, creamed soups, puddings, ect.). Liquid non-dairy creamer is protein free and can be used without adding unwanted protein in the diet.

Starches

The starch group holds many options for ways to boost calories, as they can act as carriers for fat and sugar. Just look at the possibilities: Hot biscuits and muffins with margarine and jam, jelly or honey sandwiches, pancakes or waffles with extra margarine and syrup or non-dairy whipped topping, sugar sweetened cereals with liquid non-dairy creamer, rice with margarine, rice with liquid non-dairy creamer topped with cinnamon and sugar, crackers topped with margarine and jam, noodles with lots of margarine (can vary flavor by adding a variety of herbs), macaroni salad with extra mayonnaise, cold pasta with vinegar and oil dressing to make pasta salad (can add a few chopped vegetables for flavor), warm tortillas spread with margarine and sprinkled with cinnamon and sugar, popcorn with margarine, popcorn balls, jelly filled donuts and iced sugar cookies, layer cakes split into four layers to hold extra frosting, graham crackers held together by frosting, Rice Krispie treats.

Fruits and Vegetables

In the vegetable and fruit list, there are also wise choices to be made. Use mayonnaise or low sodium salad dressings such as vinegar and oil on salads or as a dip for fresh vegetables, add margarine to hot, steaming vegetables, stir fry fresh vegetables in oil, top allowed portions of potatoes with margarine, gravy or a small amount of sour cream, drizzle allowed portions of warm potatoes with oil and vinegar dressing, then toss with mayonnaise, chopped onion and celery for a high calorie hot potato salad (season with pepper and herbs to taste), choose fruits canned in heavy syrup, generously sprinkle fresh fruit with sugar and serve it with non-dairy whipped topping or liquid non-dairy creamer.

Beverages

The fluids you drink in your diet can also be a tremendous source of calories. Add sugar and non-dairy creamer to your coffee and tea, drink Hi-C, Koolaid, artificially flavored lemonade and carbonated beverages that contain sugar, instead of diet beverages (one quart of Koolaid, as prepared by the package instructions, will provide 400 calories). Try popsicles or fruit ice for a cool summer treat. If you have been asked to limit your fluid intake, drink only the allowed amount of high calorie beverages.

By now I am sure that you have the idea of thinking fat and sugar. However, that is not the only way to boost calories while following a diet the requires moderation of protein. There is a special group of foods called low protein products which may be used with a diet requiring moderation of protein. All of these foods have no protein or insignificant amounts, which allows them to be used, as needed, to provide extra food and calories in the diet. Also, use of these products allows you to not be so dependent upon fats and sugars for calories. These products include: Low protein bread, crackers, cookies, pasta, rice and gelatin. Ask your dietitian for more details and how to obtain these products.

It is very important that your diet contains enough calories to prevent unwanted weight loss. Lost weight is difficult to replace, so start right away! To successfully follow this diet, you will need to be creative and use your imagination. Start by using these tips. Of course, you cannot use all of these suggestions every day, but they should get you thinking about ways to increase food and calories in your diet, to help get rid of those hunger pangs and avoid unwanted weight loss.

Reviewed by:

Barbara Griggs, M.A., R.A., Renal Dietitian,The Children's Hospital of Philadelphia Philadelphia, Pennsylvania

FALL/WINTER 1997 ISSUE No. 4

LETTER FROM THE EDITOR

Dear ARPKD families:

Taken out from storage recently was the unfinished Peter Rabbit baby blanket. Begun six years ago, the nearly completed cross-stitch baby blanket holds many memories, as it represents many things. From happy, anticipated thoughts of a first-time pregnancy to broken dreams of motherhood.

My hopes of mothering were shattered at 33 weeks gestation with the diagnosis of "Infantile Polycystic Kidney Disease," a term used to describe autosomal recessive polycystic kidney disease (ARPKD), and the grim prognosis which included: "these babies only live a few months to no more than one year after birth … with "Potter-type faces (facial deformities) and postural deformities."

With the knowledge that the child I was carrying would certainly die, and that future pregnancies were destined for a 25% probability of an ARPKD child, I mentally swore off the lifelong dream of motherhood. Pondering what life would be like without children produced feelings of bitterness. The blanket was tossed into the nursery and the door remained shut for a long time. I had lost hope in the future.

I view many things differently now, having since recovered from the anguish and trauma of those days. Indeed, the events seem distant and far away. Only when I hear a similar story, or one in which PKD parents are misinformed with antiquated information, do I recall with clarity the feelings of disbelief and grief. It is a gentle reminder of the continued need for PKD education and research.

In spite of this, my outlook for the future is no longer one of doom and gloom. Many, many children with ARPKD survive and lead productive and happy lives. A good example is my first child, who survived and is doing well with ARPKD. He is happy, curious, energetic, and exuberant. His kidney function remains within normal limits and his quality of life is excellent.

I now have a clearer and better understanding of ARPKD, which has changed my outlook and attitude greatly. So much so, that I have gone on to have another child, and a third is on its way. There was a 75% chance that our second child would be healthy, and he is. As yet, we do not know the outcome for our third child

Some have wondered aloud, "How could you consider having another child if there is a chance he or she may have ARPKD?" It was not an easy decision, but because I could not imagine life without my oldest child—who is so full of life, has so much to offer and has such potential—how could I not?

One thing I know, our child—with or without ARPKD—will be a welcome addition to our family. As ARPKD parents know, their ARPKD children are very special. With the passing of time, I have learned an important lesson: special children with special needs, whether minor or major, widen our perspectives and teach us many things, including love, tolerance, and patience. They strengthen our character as no other situation in life can. "Special children" have "purpose," even if some of their abilities are small.

The future for children with PKD looks brighter each year. The benefits of research are constantly moving technology forward, improving clinical treatment, dialysis and transplantation. Even a PKD cure is now foreseeable. There is so much hope in what the future will bring.

We can make a difference. There is power in numbers, and the more voices heard, the louder the message. On June 19, 1998, hundreds of PKD families and friends will converge on Washington, D.C., to visit their congressional representatives and senators during the 9th Annual Conference on PKD. They will voice their concerns about the need for increased funding for PKD research. Won't you join me and many others to voice your concern that there is not enough money being allocated for PKD research? I hope to see you there!

Best Wishes,
Colleen Zak, Editor/

Dr. Campbell's Top Ten Ways On How Best To Get What You Want For Your Child From The Medical System

By Robert Campbell, M.D.

10. **KNOW THE RULES**

One of the wisest investments you can make in your relationship with those who provide healthcare for your child is to learn the office policies of your physician and the rules of your health plan. Inquire about your physician's hours, availability for emergencies and nuances of the office billing policies. If this information is not available in writing, for accountability note the name of the individual who gives the information. Once you know the "rules" it is less likely you will get lost in "the system".

9. HELP PEOPLE HELP YOU

In a perfect world, you can call your doctor's office and have your child be been that day at a convenient time for your schedule, but the reality is much harder to handle. Your doctor, especially if he or she is a surgeon, has certain defined availability during the work week for office hours. A physician's office schedule tends to be chaotic, especially with busy doctors, since emergency patients are

often “worked in” the schedule (when there is no room for them) so that delays invariably occur.

When you call your doctor, clearly state, to the best of your ability, what your child’s problem is so that the office can determine if you need a routine appointment or urgent care. The urgency of your child’s problem should be a *mutual* decision between you and the doctor’s staff. Provide clear information.

Consider being willing to pay the price of inconvenience if the staff needs to work your child in for an appointment with short notice. If your doctor is not available, ask to see an associate doctor or a doctor in another office. If you’re not satisfied with the appointment scheduling, you can ask to have your doctor call you to discuss it but be very clear why the office recommendations will not work for you.

If office hours are not available, your doctor may be able to see your child at the emergency room although this may add waiting time and expense. The addition of “surprise patients” to a busy office schedule is stressful for the doctor’s office and patients and often leads to delays as the doctor is pressured to rush to get everyone seen in a timely fashion.

8. USE THE PHONE EFFECTIVELY

A physician may have 10-20 phone calls to return each day and the more detailed and helpful your message is, the more likely it will rise to the top of the pile.

Instead of a vague request to just call about your child, detail in the message what you need so the doctor can prepare for the call through review of your child’s records or test results.

State how soon you wish your call to be returned and give work and home phone numbers, as well as pager numbers so that you can be reached when there is time available to return the call.

If you don’t hear from your doctor within the expected time, don’t hesitate to call again to see why you have not gotten a response. If your message is detailed enough, you may get a very speedy response if the doctor can direct one of his staff to call you with the answers to your questions.

7. FIND ALLIES

Take a moment to chat with the people who work with your doctor. Give them a chance to get to know you and your child. Acknowledge and thank the staff when you get the extra attention you need. Your communication with your doctor’s office will be more effective if people there know your family personally.

6. DON’T FEEL LIKE A NUMBER IN THE WAITING ROOM

If excessive waiting has been a past problem, call half an hour ahead of your child’s appointment to see how the doctor’s schedule is progressing. You may choose to reschedule or the nurse may ask you to come in later when the doctor is back on schedule.

When you get to the office, ask how long before your child will be seen. Get specific times, not “in a while” or “son”. Bring something to read or do while you’re waiting.

Delays are an unfortunate reality of the system. Surgeons can’t leave an unexpectedly lengthy surgery to get to the office on time. The compassionate physician must sometimes take longer with a family. Your time, however, is just as valuable as you doctor’s time and it is your choice to wait until your doctor is available or go to another physician with waiting times that work better for you. If you do choose to leave before being seen, be sure that your child’s condition will not be worsened by a delay in seeing a doctor.

5. DO YOUR HOMEWORK

Do every you can to make your child’s appointment with the doctor go smoothly. Have the proper paperwork and authorization from your primary physician (if necessary).

If this is a first appointment wit a new physician, bring old medical records and bring a list of all current medications and dosages.

4. HAVE QUALITY TIME WITH YOUR CHILD’S DOCTOR

Be as clear, concise, and straight forward as you can in describing your child’s problem. Let your child tell his or her story and try not to interrupt. Often, the diagnosis can be sorted out from the information you give. After you finish, the doctor may ask questions which require brief answers. In respect for patients being seen after you, wait until the end of the appointment for any long chats.

3. BE OPEN & DIRECT

Be completely honest with your doctor since that is the best way to insure no important details are forgotten. Your child’s privacy will be protected. If you are uncertain of your doctor’s knowledge of your child’s problem, be open with him or her and ask to see experts in the problem field.

2. **EXPRESS YOUR CONCERNS CLEARLY**

Your child may have been referred to a specialist because of an abnormal lab test or for some other reason unclear to you. Clearly express what is concerning you to your doctor. If you’re afraid test results mean something like cancer, express these fears so your doctor can address them.

1. NEVER GIVE UP

Pursue all avenues, enlisting allies as needed. The medical system has its own "chain of command". Fill out any necessary paper work and get it into the proper hands, pursuing appeals and letters of support as needed. Work your way up the ladder of the system. If you've exhausted all options and can't get what you need for your child, get your doctor to go to bat for you and your child. Keep talking to people who may be able to help you, enrolling them in your cause, and NEVER, NEVER, GIVE UP.

—Robert Campbell, M.D. is an Associate Professor in the Department of Orthopaedics at the University of Texas Health Science Center at San Antonio. He has held numerous positions in many Orthopaedic Societies and among his many honors was selected in 1992 as one of the ten best pediatric specialists in the United States by Child Magazine. Dr. Campbell was the recipient of a NORD Research Grant to assist in his development of the Titanium Rib which expands the chest cavity in children born without ribs, giving their lungs room to expand.

—The preceding article appeared in *NPPSIS* (National Parent To Parent Support & Information System, Inc.); Summer, 1997 issue.

(The following information derived from the *Alliance of Genetic Support Groups*, June 17, 1997 newsletter)

PARENTHOOD AFTER TRANSPLANT

By Vincent T. Armenti, MD, PHD and Michael J. Moritz, MD

In January, 1991, we established the National Transplantation Pregnancy Registry with the assistance of Sandoz Pharmaceuticals. This registry is a storehouse of information abut transplant recipients who have become pregnant or fathered a pregnancy. By collecting information from many recipients all across the country, we are trying to answer the complex questions that revolve around the transplant recipient and the transplanted organ when there is a pregnancy.

Over the first 15 months, we have received responses from 446 transplant recipients (628 pregnancies). Most of these pregnancies (80% for women; 94% for male-fathered) have resulted in live births. The responses are:

The average age of the female recipient was 27 years at the time of pregnancy and the average age of the male recipient was 32. While the interval between transplant and pregnancy ranged from weeks to 19 years, the average for both men and women was three to four years.

So far, the risk of a miscarriage or a stillbirth seems to be similar to that for the general population, but we still need more information before we can be certain.

A premature birth, which is defined as a pregnancy of less than 37 weeks, occurs in approximately 10% of the live births in the general population. For the fathers in our registry, the chance of premature offspring was the same (10%). For the females in the registry, 58% of the live births were premature. Most of the premature babies were only four to five weeks from term, when babies are relatively well-developed, so most of the babies have done well.

Of the babies of female recipients, 26% were reported to have complications compared to 11% of the babies fathered by male recipients. Most of the complications in the babies of female recipients can be attributed to the higher rate of prematurity. There has not been an increased number of birth defects reported compared to the general population.

We have not found an association between the degree of prematurity or number of newborn complications and any of the "standard" immunosuppressive drugs, namely prednisone, azathioprine (Imuran*) and cyclosporine A (Sandimmune*). We seem to be seeing an increased risk of lower birth-weight, prematurity, and complications when there is a shorter interval from the time of transplant to conception. Waiting one to two years after transplant before conception seems advisable.

For many transplant recipients, a successful pregnancy may be attainable. However, there are risks which vary from person to person. The pregnancy can jeopardize the mother's health or the function of her transplant. Again, the risks to the baby are not completely determined. We strongly recommend seeking the advice of your doctor before considering pregnancy. In addition to waiting for at least one year after the transplant, good control of blood pressure, and stable, excellent transplant organ function should be present. Finally, pregnancies in female recipients should be considered "high risk," and obstetric care should be sought with the guidance of your doctor.

The experiences of many more recipients will have to be added to the pregnancy registry before we will be able to fully sort out all the issues involved in pregnancies following transplant. . . . . .

About the Authors

Vincent T. Armenti, MD, PHD is assistant professor of surgery at Thomas Jefferson University in Philadelphia, where he completed a fellowship in transplantation. He is a member of the Greater Delaware Valley Society of Transplant Surgeons and the Teratology Society, a group interested in the study and prevention of birth defects,

Michael J. Moritz, MD is associate professor of surgery at Thomas Jefferson University in Philadelphia. He is section chief of liver transplantation and acting director of the transplantation program. He is a member of numerous societies devoted to transplantation, kidney disease and liver disease.

TIPS AS AN ALTERNATIVE TREATMENT …

My Story by Donna Robbins

Back in April of this year, our lives took a sudden, traumatic nose dive and we were shaken back into the reality of ARPKD and it's other ugly side, Congenital Hepatic Fibrosis. Our daughter, now almost 14, was diagnosed at birth with ARPKD, and with Hepatic Fibrosis at 9 months of age. We had known for years that she would some day succumb to portal hypertension and subsequently bleed from esophageal or gastrointestinal varicies. The bleed occurred in her stomach and we had no warning that it was coming except for the increasingly debilitating abdominal pain she experienced on a daily basis. She had been scoped for esophageal varicies several years earlier and found to have two "hot spots" that more than likely would bleed in the near future. As time went on and no bleeds occurred, we became complacent and unwary. Her kidney involvement was progressing but not at the pace we first anticipated. Medications had become a way of life as well as pain, frequent illnesses, restrictions, special diets, and set-backs.

So when early one morning she came into our room and announced she had vomited blood we took it in stride with an, "okay, I'll come look" (not really expecting anything of significance). As it turned out the volume WAS significant and we knew we had an emergency on our hands. On the way to the hospital and a span of about 2 ½ hours she lost even more blood and quickly turned critical. She was given whole blood transfusions and a medication to, hopefully, stop the bleeding.

The medication worked. A day later she was stable enough to have the banding procedure to tie off bleeding varices in her stomach and her condition steadily improved.

At this time the doctors presented us with a relatively new treatment that might buy some time and stop the portal hypertension causing the bleeding. The procedure is called transjugular intrahepatic portosystemic shunt or TIPS and is being used in patients with cirrhosis and other forms of advanced liver disease with recurrent variceal bleeding. It offers a less invasive alternative to surgical shunting and does not require an open abdominal operation.

It was explained to us that TIPS would divert the blood from the high pressure portal vein to the low pressure hepatic vein much the same as a partially decompressive surgical portosystemic shunt. A catheter is fed through the hepatic vein via the right internal jugular vein and connects with the intrahepatic portal vein using fluoroscopic imaging to guide the way. An expandable metallic stent is then anchored in place inside the liver and portal pressure is reduced. Since this is a rather complex radiological procedure, placing the stent inside the liver, we needed to be aware of the consequences that could result. After weighing the alternatives, we decided to proceed.

The affects were immediate, lowering portal pressures from 20 to 9 instantly. Her only scar is from the tiny ¼ or so inch opening in her neck (at the jugular vein) the entrance for access to the liver. Within days she was no longer complaining of abdominal pain and, as her energy level built back up after having lost so much blood, she said she has never felt better.

The TIPS has to be monitored by ultra-sound on a regular basis for patency and has, on one occasion, narrowed and had to be revised. The hospital stay for the revision was only overnight. Coumadin thins the blood and, at the right level (a PT and INR is done weekly), will help in preventing the narrowing or stenosis of the TIPS over time.

We really haven't had enough time to analyze the data but having been there, done that, we would certainly recommend the procedure as a predecessor of a more invasive alternative.

TOPIC: IS ARPKD REALLY A LIVER DISEASE?

THE FOLLOWING ARE NOTES TAKEN FROM DR. DAVID PICCOLI'S LECTURE, PRESENTED DURING THE ARPKD SUPPORT GROUP MEETING HELD MARCH 27, 1997, AT CHILDREN'S HOSPITAL OF PHILADELPHIA IN PHILADELPHIA, P.A..

Autosomal Recessive Polycystic Kidney Disease (ARPKD) and Congenital Hepatic Fibrosis (CHF) ….. From what we know, these are genetic disease processes based on one gene site, and not two different diseases within the ARPKD population. Though a person may have CHF and not ARPKD, if an individual has ARPKD, it is thought that they always have some form of CHF. Yet, the ARPKD person may be asymptomatic from CHF (without CHF symptoms) for a lifetime. Symptoms and outcomes vary greatly within the ARPKD and CHF population, and the disease processes do not look the same from one individual to another.

Usually a person has predominately liver or kidney involvement and manifestations. To have clinical symptoms from both the kidney and the liver is rare, though it does occur. Those who have more liver involvement are often not diagnosed the first year of life. They usually present with bleeding from 6 years of age and up. But, there is not the ability to predict when or if this bleeding will occur or the course of CHF.

WHAT IS CONGENITAL HEPATIC FIBROSIS?

CHF is a bile duct disorder. The bile ducts, develop with the portal vein, which is the "plumbing system" that drains hugh amounts of blood into the liver. When there is an abnormality within this "plumbing system of the liver" due to "ductal plate malformation"

and fibrosis, the blood running through the liver may be slowed. Fibrosis is scar tissue and very different from cirrhosis, though either, if severe enough, has the potential of causing portal hypertension. If blood flow restriction is significant, then the blood backs up. Where this backup occurs is where the problems occur.

FOUR MAIN PROBLEMS LIVER DISEASES MAY HAVE

1. Inability to purify and excrete waste products from the liver properly, i.e., as with jaundice.
2. Problems with synthesizing compounds, i.e., unable to produce compounds needed for blood clotting abilities.
3. Lack of regulatory function, i.e., can't control blood sugar level, hormones, blood pressure, etc.
4. Blood flow abnormality and blood flow backup, (yet liver excretes, syntheses, and regulates normally). THIS IS THE PROBLEM THAT IS SEEN WITH CHF.

BLOOD FLOW ABNORMALITY AND DUCTAL PLATE MALFORMATION

This blood flow abnormality is not caused by infection, toxins during pregnancy, or environmental factors. It is an inherited "ductal plate malformation" which occurs, early in the formation, as the fetus grows. This maturation of the bile ducts and portal vein is genetically programmed to grow abnormal in configuration. The area surrounding the bile ducts and portal vein (which is the huge blood vessel that goes through the liver) is filled with fibrosis (scar tissue). (This same inherited defect is going on in the kidneys, but the two organs react totally different.) Yet, CHF may be detectable only by microscopic examination for a lifetime, as some individuals never have clinical symptoms.

In the past 10 to 15 years, CHF has been called different things, including Infantile PKD and Infantile Poly Cystic Disease (IPCD). There are other disease processes, unrelated to ARPKD, that mimic CHF and look just like it, so the CHF diagnosis is not quite as acurate or specific as DUCTAL PLATE MALFORMATION (DPM). This is the abnormality seen in the liver with ARPKD. DPM has a highly variable clinical outcome. There are no rules and no way to predict the outcome or prognosis. ARPKD individuals may have CHF so mildly that they never experience any of it's consequences.

So, an ARPKD individual may only have symptoms from either their liver or kidney. This means that, clinically, they may only need treatment for the DPM (liver involvement), or ARPKD (kidney involvement) throughout their lifetime. If you don't hear much about liver disease in your child, you probably won't in the future; and, if there is little mention of kidney problems, the probability of later kidney involvement is unlikely. Yet, having clinical symptoms from one organ does not protect you from having manifestations from the other. Once again, the course of this disease is unpredictable.

There is some correlation between the age of when ARPKD is diagnosed and what the manifestation of disease processes will produce. Often ARPKD, with kidney involvement, is discovered prenatal, at birth, or soon afterward. DPM, or liver involvement, may be discovered as late as adolescence. The older an individual upon diagnosis, the more likely manifestations will occur from the liver and not the kidneys.

WHAT ARE SOME OF THE CLINICAL MANISFESTATIONS OF DUCTAL PLATE MALFORMATION OR CHF?

Hepatomegaly - an enlarged liver from pressure within due to fibrosis and ductal plate malformation.

Splenomegaly - an enlarged spleen due to the blood flow backup from the liver.

Portal Hypertension - result of increased pressure within the portal vein (the "huge plumbing tube" that goes into the liver). Blood must flow freely from high to low pressure areas. If not, the resistance to flow cause blood to backup into surrounding areas. Portal hypertension may occur within the portal vein before entering the liver, within the liver, or past the liver site. This hypertension is different from systemic high blood pressure (which is measured with a cuff on the arm). The lower pressure areas that blood may spill into and eventually cause problems are the spleen, stomach, esophagus, large and small intestines, and rectum (where hemorroids may occur). Portal hypertension may improve or worsen with time.

Esophageal Varices - result of Portal Hypertension. The low pressure blood vessel walls in the esophagus are weak, thin, and prone to stretching and have the potential to distend. With enough tension, they may rupture causing large amounts of bloody vomitus, black, tarry stools, or coffee ground-like stools. Once the "dam breaks", the pressure drops and the bleeding usually stops; however, by then massive amounts of blood may be lost. (It is not inevitable that because a person has esophogeal varices, they will rupture.)

Bile duct dilatation - occurs because of the bizarre bile duct configuration and has also been called Caroli's Syndrome or Disease. Normal bile ducts are threadlike or hairlike in size and shape, whereas dilated ducts look more like big sacks. Bile may pool in the cavities of the big, dilated ducts. Instead of bile flowing in and out, which is how the other bile ducts work, the bile may move sluggishly.

Cholangitis - infection caused by stagnation from bile sitting inside a dilated bile duct(s). This is usually treatable by antibiotics. For more resistant infections, radiological or surgical procedures may be needed.

Liver transplantation is not a common outcome. It is best to keep the original liver as long as possible. Except for potential blood flow problems and infections, the liver has the continued ability to excrete, synthesize, and perform regulatory functions. Shunts, which are surgically inserted to take high pressure blood to lower pressure areas, if successful, may work for a lifetime. If a shunt is to long or crooked, it may clot within a few years; the more direct a route, the better.

Pediatric transplantation data is encouraging.

WHAT ABOUT THE CHICKEN POX VACCINE?

By Jorge Vargas, M.D.

Most of the time, varicella (chicken pox) is a very benign and mild disease. In some cases, however, children in particular may become severely ill from this highly contagious viral infection. Chicken pox occurs year around with no major seasonal variations, however, it is mostly seen from January to May. In this country, almost 4 million cases occur every year and between 4 to 9 thousand cases require hospitalization. In a very small number, the infection proves to be fatal.

Severe complications of varicella include pneumonia, encephalitis and other bacterial infections, and may occur in normal, healthy children. Obviously, children who are either taking medications to suppress the immune system or undergoing chemotherapy are at a higher risk of developing more severe infections and/or complication. It was with this group in mind that a vaccine was developed which has proved to be very efficacious.

This vaccine is generally recommended for toddlers between ages 12 to 18 months who have not had chicken pox, as well as older children, adolescents, and adults who may be exposed. Although the vaccine does not provide 100% protection against varicella, cases that occur after vaccination are generally very mild. Several studies have addressed the issues of the length of immunity, which is not permanent, and on the safety and need for booster doses of varicella vaccine 4 -6 years after the initial immunization.

A major discussion is underway because of the lack of good data on the safety of the vaccine in children receiving immunosuppressive agents. Studies have been carried out in countries like Canada, Japan, and the European Union, and the results are being analyzed. There are studies showing the safety and efficacy of the vaccine in children with debilitating diseases, or children who receive chemotherapy. While the Academy of Pediatrics is developing clearer policies and better guidelines for the use, schedule and need of monitoring regarding the efficacy of the vaccine, it is important to understand that at this point in time children who are potential candidates for a liver transplant, children who are already on a waiting list for transplantation and their siblings who have not had chicken pox, should ALL be vaccinated before a transplant.

Varicella virus has an incubation period of 11 days to 3 weeks. Even before the infection is apparent by the presence of skin lesions, it is transmitted in the air by respiratory particles and secretions. Patients are most infectious during the 24 hours before the skin lesions appear and until all lesions are dry and crusted-over completely.

If a child has not had the vaccine or the illness and is receiving immunosuppressive agents to prevent rejection of a transplanted organ, and this child comes in close (and I emphasize *close*) contact with a child that subsequently develops chicken pox, it is advisable that the child receive a special dose of antibodies to fight the potential infection, to be administered within 72 hours of the contact. This is a specific antibody against the varicella virus called VZIG, and it is given by intramuscular injection in an amount proportional to the child's weight. It is not 100% efficacious, but administered on time may completely prevent the disease or make it much milder. These antibodies, as they were not actively produced by the patient, do not last and only cover a period of approximately 5 - 6 weeks, making it necessary to re-administer the treatment in cases where new contact occurs beyond this time.

If a child on immunosuppressive agents develops the disease, we always suggest treating the child in the hospital with intravenous medications designed to kill the virus. We also reduce the amount or dose of immunosuppressive agents while the disease is active, keep a close eye on potential complications and treat them quickly as they are presented. Frequently an antibiotic treatment for bacterial infections of the skin lesions is also prescribed.

Lastly, having the infection generally confers a child or patient with sufficient immunity to be protected for life. However, in patients whose immune system is depressed by medications, this is not usually the case. It is not rare to see either recurrence of the chicken pox or recurrent episodes of the so-called "shingles," which is caused by the same virus.

Dr. Vargas is Associate Professor of Pediatric Gastroenterology/Nutrition at UCLA Medical Center. He is also a member of the Scientific Advisory Board of *CLASS*. Reprinted with permission.

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THE FAMILY IS AFFECTED BY THE ILLNESS TOO

By HOLLY SWARTZ, MD

ABOUT THE AUTHOR

Holly Swartz is a native of Pittsburgh now residing in New York. She graduated cum laude from Harvard University and received an

MD from Albert Einstein College of Medicine.
She is a member of the medical honor society, Alpha Omega Alpha, and is currently in the psychiatry residency training program at Cornell University/The New York Hospital in Manhattan.

Laura's daughter was doing well after a kidney transplant. When the doctors told Laura that her daughter could resume a normal life, Laura decided that she would do exactly that: Laura sent her daughter back to school immediately and insisted that she and her husband resume their busy social schedule. Although she rarely discussed the surgery or fears that her daughter would relapse, Laura dreamed about it every night. When her daughter developed a fever and had to be readmitted to the hospital, Laura had difficulty recovering her equilibrium. Despite assurances that her daughter was doing very well, she felt compelled to take her daughter's temperature twice a day. She refused to participate in any leisure activities and found herself spending less and less time with her husband. Although she no longer dreamed of her daughter's illness, she stopped sleeping well at night.

When an individual falls ill, the person afflicted by the disease is rarely the only person affected by the disease. Inevitably there are others—parents, children, spouses, friends—whose lives are also altered by the chronic illness. Commonly, these family members experience powerful feelings—fear, guilt, sadness, anger—in response to their loved one's illness.

At times, they can feel quite isolated—as if it were unusual to have strong feelings about another's illness. It is helpful to acknowledge that these reactions are accepted—even expected—from family members. It can also be useful to learn to recognize some of the typical coping strategies employed by family members in this setting.

DENIAL: The pain of seeing a loved one suffer can be so intense that a family member unconsciously minimizes the significance of the illness. While the family attempts to maintain a veneer of normalcy, often the hidden feelings resurface in other places. In Laura's case, when family life prematurely returned to normal, Laura experienced disturbing dreams. While this coping style can help the family achieve a higher level of functioning, it can also interfere with the processing of important emotional experiences.

MARTYRDOM: Sometimes families become so preoccupied with the detail of their loved one's illness that they lose sight of their own lives. While the intent is to assist the ailing family members by doing everything possible, the effect is often to prevent the caregiver from participating in his usual activities. When her daughter returned from the hospital a second time, Laura's strong feelings (perhaps guilt; perhaps fear) prompted her to give herself over to her daughter's care. While taking her daughter's temperature twice a day might have permitted the early detection of infection, the net effect was to compromise Laura's quality of life while only marginally improving her daughter's health care. Although this coping style may facilitate the active participation of family members in the patient's care, it may interfere with their ability to meet their own health needs.

BLAME: Most family members experience a degree of anger—both toward the individual who has fallen ill and toward a cosmic order that would permit such suffering. Because it can be difficult to express those feelings directly, sometimes the anger is displaced onto others. Family members may find themselves snapping at co-workers, arguing with grocery clerks, lashing out at their children, and battling the doctors. Sometimes, like Laura, they turn the anger inward. Although the indirect expression of anger can permit the discharge of painful feelings while shielding the patient from the same, it can also lead to misunderstanding and disruption of important support systems for the family member.

Like Laura, most individuals respond to the stress of chronic illness with a combination of denial, martyrdom, and blame. Many families use this repertoire of coping styles both to effectively manage their own reactions to illness and to facilitate patient support. The outcome can result in improved psychological health for both the patient and the family.

There are times, however, when these strategies fail or are harmful. At the point when a family member feels overwhelmed or develops symptoms of psychological distress (disturbed sleep patterns, changes in appetite, loss of interest in pleasurable activities), it is important to seek the advice of a mental health professional. Although the family member is not the patient, the family member is nevertheless entitled both to strong feelings and to appropriate health care.—

—from *Stadtlanders Lifetimes,* Spring, 1992.
Reprinted by permission of Stadtlanders Pharmacy.

CAN MILK THISTLE HELP PROTECT THE LIVER?

(This is a question you may want to ask your GI Physician.)

In more than three hundred clinical and experimental studies (mostly conducted in Europe), Milk Thistle is an herb that has shown considerable promise as a liver protectant and has been used for centuries to treat liver disorders and to strengthen the liver.

The seed of Milk Thistle (Silybum Marianum) is the active component which has been shown to consist of a large number of flavonolignans. It has been shown that Silymarin slows the entrance of toxins into liver cells and protein synthesis is also stimulated, therefore helping to accelerate the regeneration process and production of liver parenchyma cells. Research also indicates that Silymarin seems to protect liver cells by neutralizing free radicals which are formed in the liver.

In Europe, (where herbal use is common place), the German Commission E, an agency that evaluates the safety and efficiency of herbs, endorses use of Milk Thistle as a supportive treatment for chronic inflammatory liver conditions, including inflammation

of the bile duct and cirrhosis. There have not been any studies done on whether Milk Thistle is helpful with regard to Congenital Hepatic Fibrosis. However, it may be worthwhile asking your physician whether it might be used as an aid to relieve the daily stress of the liver's job.

There have not been any toxic side effects reported on Milk Thistle. However, some people may experience loose stools in the first few days of use. Milk Thistle can be obtained at your local health food store. An important note is to make sure if you purchase a tincture (a liquid form of the herb), do NOT buy a type preserved in alcohol. Purchase the type that is preserved in glycerin, so that your child will not be consuming the alcohol.

If you and your GI physician are interested in obtaining more information concerning Milk Thistle, one good source of studies on herbs is the Herb Research Foundation, 1007 Pearl Street, Suite 200, Boulder, Colorado 80302, or telephone 303-449-2265. As with any herb/drug, being educated and armed with accurate and reliable information first, before using something, is of the utmost importance.

Before you take any herb or drug, always discuss the matter with your physician first. Your doctor will be able to advise you whether this herb could be helpful, or detrimental in your particular case. He/she can also recommend the appropriate dosage.

— Beth P. Hall, ARPKD Mother, Buffalo, NY

GUESS WHO'S COMING TO DINNER!

REPRINTED BY PERMISSION OF ENCORE, SPRING 1995, VOLUME 5, NUMBER 2 ISSUE; A QUARTERLY MAGAZINE FOR INDIVIDUALS WHO HAVE UNDERGONE TRANSPLANTATION.

If you're not careful, you may have some unwelcome guests at tonight's dinner table. They go by the names *Clostridium perfringens, Staphylococcus aureus, and Shigella.*

These party crashers are just three of the many microorganisms that can contaminate food and cause a variety of illnesses. In fact, tens of millions of cases of intestinal illness occur each year in the United States. For most people, the worst that happens is a short-lived case of abdominal cramps, diarrhea, or vomiting. But for others, especially those with suppressed immune systems, food-borne illness can cause serious reactions and dangerous infections.

The organisms are in a wide range of foods, including meat, milk, and other dairy products, coconut, fresh pasta, spices, chocolate, seafood, and even water. Egg products, tuna, potato and macaroni salads, and cream-filled pastries are also targeted by organisms. Poultry is the food most often contaminated. It's estimated that 60 percent or more of raw poultry sold at retail stores probably carries some disease-causing bacteria.

The idea that the food on the dinner table can make someone sick may be disturbing, but there are many steps you can take to protect yourself and your family. It's just a matter of following basic rules of food safety.

Above all, you should not consume unpasteurized milk or raw or undercooked eggs, poultry, fish, shellfish, or meat. However, prevention of food poisoning really starts with your trip to the supermarket.

Look for cleanliness at meat and seafood counters and salad bars. For example, cooked shrimp lying on the same bed of ice as raw fish could be contaminated. Buy only Grade A or better eggs, and avoid ones that are cracked or leaking.

Don't buy any foods whose "sell by" or "best used by" date has passed. Read the label to see whether a food contains raw or undercooked animal-derived ingredients. Caesar salad dressing, for instance, traditionally uses raw eggs. Buy only milk and cheeses labeled "pasteurized." (Firms may sell cheese made of raw milk provided it has been aged for over 60 days. To be safe, transplant patients should avoid this as well.)

Keep groceries safe

Put raw seafood, poultry, and meat in plastic bags so drippings can't contaminate other foods in the shopping cart or bag. Take groceries directly home and refrigerate cold foods. Hot foods from the deli should be eaten, kept hotter than 140º F, or refrigerated right away. Leaving foods unrefrigerated for even a few hours fosters bacterial growth. Refrigerator temperature should be 40º to 45º F, and the freezer should be zero.

Store eggs in their original carton in the main section of the refrigerator. Don't put them in the egg section of the door because the temperature there is higher.

Don't crowd the refrigerator or freezer so tightly that air can't circulate. Check the leftovers in covered dishes and storage bags daily for spoilage. Anything that looks or smells suspicious should be thrown out. A sure sign of spoilage is the presence of mold, which can grow even under refrigeration. Mold is deceptive in that only a small part is visible. The large part extends below the surface of the food. However, it is possible to save a part of the food by cutting off and discarding the visible blemish along with a large section of the food around it.

Cleanliness is next to .....

Thirty percent of all foodborne illnesses result from unsafe handling of food in the home. The first cardinal rule of safe food preparation is: Keep everything clean. Wash hands, utensils, counters, and cutting surfaces with hot soapy water between preparation of different foods, particularly after handling raw eggs, meat, poultry, or fish.

Which is better, wooden or plastic cutting boards? Recent research confirms the conventional belief that plastic is safer than wood for cutting meat and poultry. However, wooden cutting boards used exclusively for raw meat and poultry are acceptable. Use a

different board for cutting other foods such as produce and bread. Do not put cooked meat on an unwashed plate or platter that has held raw meat.

Wash fresh fruits and vegetables with water, using a brush, if appropriate. Protect yourself with a plastic sealing bandage or plastic gloves if a hand has a cut or open sore. Wounds are easy entry points to the body for bacteria.

Use thermometers

The second cardinal rule of home food preparation is: Keep hot foods hot and cold foods cold. Promptly refrigerate or cook foods, including vegetables, after you cut them up. Bacteria can grow at temperatures above 40º F and below 140º F, so temperature is vital in keeping food safe. Cooked foods should not be left standing on the table or kitchen counter for more than two hours.

The Food and Drug Administration recommends cooking beef and lamb to at least 140º F, pork to at least 150º F, and poultry to 165º F. Use a meat thermometer to ensure complete cooking. Follow the recipe for seafood, but don't undercook it. Avoid lightly steamed mussels and snails, for instance. Fish should be flaky, not rubbery, when cut. Never eat oysters on the half shell, raw clams, sushi, or sashimi.

Cook eggs thoroughly until both the yolk and white are firm, not runny. Consider using pasteurized eggs instead of shell eggs whenever possible.

Reheat food or heat partially cooked foods all the way through to at least 165º F. Follow the recipe's time and temperature requirements, and check with a meat thermometer. When using a microwave, observe the recipe's standing time and directions about turning the dish. When using a barbecue grill, precook meat and poultry.

And here are just a few more parting tips to keep your favorite dishes safe. Don't thaw meat and other frozen foods at room temperature. Instead, move them from the freezer to the refrigerator for a day or two; or defrost in cold water (changing the water every 30 minutes), in the microwave, or during the cooking process. Never taste any food that looks or smells "off," or comes out of leaking, bulging or severely damaged cans or jars with leaky lids.

Though all these dos and don'ts may seem overwhelming, remember, if you want to stay healthy, when it comes to food safety, the old saying, "rules are made to be broken" does not apply!

—This article is adapted from *FDA Consumer,* a publication of the Food and Drug Administration. For more information on food safety, call the USDA Meat and Poultry Hotline at 1-800-535-4555

QUESTIONS TO CONSIDER RE: HOSPITALIZATION

Many people wonder, "What are my rights in the hospital setting?"

According to the Association for the Care of Children's Health, Bethesda, MD, 1991, individuals and their families have the following rights in the hospital setting:

- **Respect and personal dignity**
- **Care that supports them as a family**
- **Information they can understand**
- **Quality health care**
- **Emotional support**
- **Care that respects the child's growth and development**
- **Make decisions about their child's care.**

In order to serve as advocates for themselves and/or their children and fully understand and participate in their care, people can benefit from some prior knowledge about routines and procedures. The following questions can be asked to help develop realistic expectations of the hospital experience and to help in preparing for surgery:

1.When and where do we report when we come to the hospital for preoperative testing?
2.Who will examine me/our child at that time?
3.What forms, insurance information, and other types of information should be brought along?
4.What tests will be involved and is there any way to prepare for these?
5.Will an anesthesiologist be available for questions? Can we ask about the medication used to anesthetize during surgery, and the last time and type of meal prior to surgery?
6.What are the rooms like on the floor? Is it possible to have a tour before admission or to look at the accommodations?
7.How many people generally share a room?
8.Are there bathrooms attached to each room or shared by several rooms?
9.Are parents or immediate family permitted 24 hour unlimited visitation?
10.Can parents stay in the room with their children? What type of sleeping arrangements are provided? Do parents need to bring any of their own supplies (pillow, sheets, etc)?
11.What are the policies and hours for other visitors?
12.Is sibling visitation permitted and what are the restrictions on it?

13.Where should families wait during surgery? Does anyone keep them informed about the progress during this time? Does the surgeon meet with them afterward to discuss results?
14.Where will the I/child go after surgery and for how long? Is family allowed in this area?
15.How will I/child look after surgery? Will there be any special equipment required?
16.How much discomfort is expected and how is it managed?
17.What is the exact procedure that is being done and how long will the hospitalization last?
18.How will I/child be able to eat after surgery? How long until food can be consumed?
19.What kinds of after-care will be expected? Who will be responsible?
20.How long until normal activities can be resumed?
21.What do we need to bring to the hospital?
22.Is our child permitted to take a special toy into the operating room?
23.Are we permitted to accompany our child to the operating room? Can we be present for the induction of anesthesia?
24.Are showers provided for parents who stay overnight?
25.Are there playrooms and child life programs for the children while they are in the hospital?
26.Are families permitted to take their children out of the room during the hospitalization? Where are they allowed to go?
27.Where can people eat while visiting patients?
28.Is it necessary or encouraged that someone always be with the patient?
29.What are the facilities for parking or public transportation?
30.Is it inadvisable to bring much money to the hospital? Are valuables at risk for theft?
31.After discharge, how long should the recuperation take?
32.How much monitoring and attention will be required?
33.When and what types of postoperative visits will be necessary?
34.Will any equipment still be in place after discharge?
35.Who should be contacted in case of emergency?

TRANSPLANT—Signs of liver graft rejection *by Jeffrey Punch, MD*

Here is a list of signs and symptoms that may indicate liver graft rejection:

- **Fever greater than 100°**
- **Fatigue or excess sleepiness**
- **"Crankiness"**
- **Headache**
- **Abdominal swelling, tenderness, or pain**
- **Decreased appetite**
- **Jaundice (yellow skin or eyes)**
- **Dark (brown) urine**
- **Itching**

None of these symptoms are specific for rejection; but they are important enough that when they occur, they should prompt a call to your transplant center. Most centers have transplant nurse specialists who take such calls and decide whether the situation warrants further investigation or should be observed for the time being.

It is very important to realize that rejection of transplanted organs is quite variable. Some patients will feel perfectly well, only to discover that their graft is being attacked by their immune system. In fact, it is more likely than not that there will be minimal or no symptoms of rejection.

Since rejection may have no symptoms at all, the standard strategy for post-transplant care is to regularly run blood tests that may be early indicators of liver graft rejection. In the beginning, these tests are run daily. For the first month or so after a liver transplant the tests are run at least weekly. Gradually the interval between measurement is increased as the months and years pass.
The common blood tests include bilirubin, AST (also called SGOT), ALT (also called SGPT), GGT, alkaline phosphatase, and LDH. These lab tests are often grouped together and called "liver function tests" or "LFT's."

The truth, LFT's are not measures of liver function per se, but are indicators of liver "well being." The tests are not perfect, however. LFT's can be extremely abnormal despite the fact that the liver is perfectly fine. They can also read normal even though the liver is barely working! Interpretation of these tests, therefore, requires some expertise in liver transplantation as well as detailed knowledge of the patient's previous laboratory studies and medical history.

When rejection is suspected it can be confirmed by a liver biopsy. In some instances a biopsy is not needed because rejection is strongly suspected. In other situations, a biopsy is critical.

The chief problem that must be differentiated from rejection is infection. Since the treatment of rejection (increased

immunosuppression) can make an infection worse, it may be important to confirm the diagnosis of rejection with a biopsy prior to proceeding with treatment.

Another important thing to know is that most rejection happens in the first year after a transplant. This is particularly true in the case of liver transplant rejection. In fact, most liver graft rejection happens in the first three months after the transplant. As long as the immunosuppression drugs are continued and taken properly, the risk of rejection of the liver is very low after the first year.

Acute rejection is a process where the immune system tries to destroy the transplanted organ. Acute rejection causes a sudden change in the function of the transplanted organ. Without treatment irreversible graft loss will result in days to weeks.

Acute rejection is usually defined by a biopsy of the transplanted organ. If the biopsy shows injury to the organ caused by lymphocytes, the patient is said to have acute rejection. (Lymphocytes are small round cells that exist throughout the body. They are thought to be the main cells that cause immune responses to transplanted organs.)

Fortunately, acute rejection will respond to treatment in more than 90% of cases.

Typical treatment for acute rejection begins with high dosages of steroids, called "pulses." If this does not successfully resolve the rejection, the next step is usually OKT3, an antibody made in mice that binds to human lymphocytes, and in so doing is very effective against rejection. Other options include changing from cyclosporine (Neoral) to tacrolimus (Prograf) or adding mycophenolate (Cellcept) if the patient is not already on Cellcept.

The most common time for an acute rejection is in the first month after a transplant. The next most common time is during the second or third month. Acute rejection occurs only rarely after the first year; however, if the patient suddenly stops taking all immunosuppression, acute rejection can occur even many years after a transplant.

In the uncommon cases where acute rejection does occur after the first year, there is often a viral infection that precedes the rejection episode. The viral infection may stimulate or "rev up" the immune system so much that it begins to attack the transplanted organ.

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This article appeared in ***Class Notes***, Fall, 1997, issue; a publication of The Children's Liver Association for Support Services.

UNDERSTANDING THE TEEN YEARS

by Deborah Miller, MSN, RN, CNN

The adolescent period is often a difficult time for families, and when you have a child with a chronic illness, the difficulties are compounded. The adolescent with chronic kidney disease, or the "renal teen" is no exception. A major challenge for this age group is the need to progress from dependence on family to dependence on self This is often in direct conflict with the parent's need to protect the ill child. In most families, the parents have had the responsibility for ensuring proper care including making appointments, getting the prescriptions and being the "historian" at the doctor's office. These parental roles are long established, and as the child starts to make the transition into adolescence an increase in family conflict may occur.

In order to understand some of the factors at work, let us divide adolescence into three stages; each stage includes distinct developmental processes. Once the processes are identified, let us look at how illness exerts its influence, and then at some approaches families can use with their child. Keep in mind that these are generalities and children are not usually this predictable.

The early adolescent (age range 11 - 14) is in the midst of puberty. The child is preoccupied with self, particularly the questions of "am I normal" and "what can I expect." The parents still exert a great influence on this age group. The child moves from elementary school to middle school at this time, and with this tremendous change comes a greatly expanded peer group. While there are more choices for friends, there are also more chances for rejection. Young renal teens are exquisitely aware of the biologic changes occurring during puberty and sensitive to their particular timing and rate in relation to their peers. Many adolescents need a great deal of reassurance at this time.

Mid-adolescence is more complex. This child is the 14 - 16 age range and is very concerned with independence as well as self image. This is often when risk-taking behavior is seen, as the child is concerned with "who am I" and "do I have power." Acceptance is important to the mid-adolescence and peers exert a great influence. With the transition into high school, the teen has an even larger pool of peers from which to choose. Middle adolescents spend much less time with their families than early adolescents, and when not with their peers, they are often alone in their rooms. The tendency to stay away from the family is consistent with trying to establish a separate identity.

The late adolescent (17 and older) is more future oriented. This age group is increasingly concerned with issues of intimacy and career. The questions asked are "am I smart enough," "am I attractive enough" and "what can I do." The need to establish more intimate relationships drives social interaction and there is a greater influence exerted by "partners." This change in thinking may

decrease the number of friends, but may increase the intensity of the friendships. The older adolescent is searching for direction, and feels pressured to make "like decisions." The search for identity intensifies during the senior year of high school with graduation creating a deadline for decision making.

These are simple role definitions and there is a great deal of overlap between stages. But how does chronic illness come into play? The most obvious influences are growth and development. Renal teens may start puberty and its associated growth spurt much later than their peers which can contribute to their anxiety. It is hard to think of yourself as "normal" when your peers are so much bigger and better developed. Does your child have scars? If a child showers after gym class, the scars cannot be hidden. This is a time when the world revolves around self so the adolescent is convinced that everyone is always looking at him or her. And all those medications? No "normal" kids take all those pills! What is the adolescent's favorite food? Big Macs, pizza, tacos, fried chicken, french fries — everyone else eats them, why can't the renal teen? And football — I have had children on peritoneal dialysis tell me that if they can't play football, their life is over (this is a very dramatic stage of life). Then there is the mortality issue. The average teenager feels immortal which is in direct conflict with the renal teen's chronic illness. Is your child worried about dying? Does this worry translate into noncompliance with medications and diet to prove they are normal? Is there risk-taking behavior involving sexual activity or substance abuse in an effort to be like everyone else?

Here are some suggested approaches: First, always tell your child the truth. Don't "protect" your child by speaking privately with the doctor as this may foster a spirit of distrust. Encourage your child to become involved in his care by asking questions of the medical team. This is a good time to slip out and let the physical exam be done in private so that "embarrassing" questions can be asked and reassurance given.

The renal teen needs to be treated as normally as possible. "Normally" means chores at home, expectations of daily school attendance and academic performance to the level of his or her ability. Renal disease should not be an excuse for poor performance. Allow your child to experience the satisfaction that comes from hard work and work well done. Adolescents on dialysis are perfectly capable of doing dishes and cleaning their room. Mastery of tasks increases self esteem and everyone wants to feel competent. If you never learn anything useful, how can you take care of yourself?

Always emphasize the positive, concentrate on what the child can do, not what he or she can't do. Encourage exercise, swimming, baseball, soccer, bike riding, tennis, etc. There are very few restricted activities, but check with your doctor for specific recommendations. Look into summer camp. Although regular summer camp is often desirable, there are special camps across the country for children and adolescents with renal disease. In this environment your child will be "normal" and may find that he or she is healthier or stronger than the average renal kid. There are often junior counselor opportunities for the renal teen who feels too old for camp. Your social worker can help you find an appropriate camp and scholarships are often available. Physical activity helps keep bones and hearts healthy and you don't have to be tall to play baseball or soccer.

Lastly, don't forget to tell your child how much he or she means to you. Acknowledge the difficulty you are having with letting go. Let the child know about your concerns and ask for his or her input. Finally, counseling should not be a last resort, it should be a first response. Your facility has many services available to you; use them early and often. With a lot of hard work, flexibility, and a good sense of humor you can successfully master the challenges of raising a responsible young adult.***—Deborah Miller, RN, CNN is a clinical nurse specialist in pediatric nephrology at Fairfax Hospital in Falls Church, Va. She is Chairman of the Pediatric Special Interest Group of the American Nephrology Nurses Association.***

—from Special Edition—AAKP ***RENALIFE,*** **a publication of The American Association of Kidney Patients. Reprinted by permission of The American Association of Kidney Patients.**

ABOUT SPLIT LIVER TRANSPLANTS

By Jeffrey D. Punch, M.D.

The liver is made up of multiple "lobes." The two major lobes, called the right and left, are further divided into segments. The left lobe is divided into the 'left lateral' and 'left medial' segments. Each segment has separate and individual blood supplies that come from main blood vessels in the center of the liver. These segments are like individual units; almost like sections of an orange. Just as the sections of an orange can be separated without getting into the juicy part, it is possible to separate the segments of a liver without having very much bleeding.

For split liver transplants, the left lateral segment is separated and transplanted to a child. This leaves the right lobe together with the left medial segment to be transplanted to an adult.

"In-situ" liver splitting means that the liver is divided while it is still in the donor's body with the blood flowing to it. As the liver is slowly cut in two, it is possible for the surgeon to see the bleeding spots and stop the bleeding with sutures or electrocautery (a small electrified wand that burns the tissue it touches).

"Ex-vivo" means that the liver is divided after it has been removed from the donor's body. There is no blood flowing to the liver, which makes it more difficult to take care of all the potential bleeding spots. Thus, liver grafts split ex-vivo tend to bleed more after ***they*** are implanted in the recipients.

The disadvantage with the in-situ technique is that it takes a long time at the donor hospital and ties up their operating room. Other organ procurement teams must wait until the splitting has been performed before they can remove the heart, lungs, kidneys, and other

organs.
Ex-vivo splitting is done while the liver sits in a sterile bowl of ice. This can be done after the liver is transported back to the transplant center while the recipients are being readied for transplant.
— from *Class Notes,* Fall, 1997; a publication of the Children's Liver Association for Support Services.

—Dr. Jeffrey D. Punch is Assistant Professor of Surgery and Transplant Surgeon at the University of Michigan. He is a member of the Scientific Advisory Board of CLASS.

LETTERS FROM READERS

Lorenzo was born on the 19th of July, 1993. My husband and I were expecting a beautiful, healthy baby just like our first child. But it wasn't to be. I remember promising myself at the beginning of labor that I would do everything right—push, breathe, etc.—and the baby and I would be better for it.

Soon after our baby boy was born it was obvious he was struggling to breathe. He was bluish in color and foaming a little from the mouth. We were quite alarmed as the doctor wasn't around—he had left immediately after cutting the umbilical cord. He was soon called back and our baby was placed in a humidity crib with oxygen. The doctor explained that it was most likely that Lorenzo, being born a large baby (9.2 Lbs.) had struggled when taking his first breath and swallowed some amniotic fluid.

X-rays in the morning confirmed a pneumothorax[1] and arrangements were made for our baby to be flown to the Mercy Hospital in Melbourne. It was a frightening and uncontrollable time. Our baby was fighting for his life and we were totally helpless.

Robert and I drove 350km to the hospital completely numb and shocked. By the morning, prayers had been answered. Lorenzo's pneumothorax had healed itself and he was going to be taken off the oxygen. We spent ten days in the hospital. For the first five he was tube fed, as he wasn't feeding, and administered antibiotics. It was a great joy to take our baby home.

At four months Lorenzo received his triple antigen vaccine. He developed a fever soon after and I believed it was a reaction to the vaccine. But a couple of days ***later*** routine specimens revealed a urine infection. Doctors thought this unusual for a baby boy of this age. A special X-ray revealed that it wasn't a kink in his waterworks causing urine to go back up to the kidneys as believed. So then an ultrasound was performed and it revealed two very large kidneys. We still did not know what we were facing and it was easier to deny anything serious. We were then sent to the Royal Children's Hospital for biopsies of the kidneys and liver. At this stage, we were still hopeful that it would be something that could easily be rectified. After all, we had no kidney disease on either side of our family. They confirmed our darkest fear.

I felt so angry and cheated. It was impossible to believe, even with the confirmation. How could this happen? Our child's pediatric nephrologist tried to answer our questions. We knew it was a genetic disease. When we returned home, I started to arm myself with as much information as possible. I couldn't believe just how little was known and how little information was available to parents. The best source we had was a family that had all three children with ARPKD. Our pediatric nephrologist had contacted them and they kindly agreed to be contacted. They answered many of our questions.

I also contacted the Kidney Foundation in Sydney for any information they had. We became members and look forward together to the future with hope. Our son is now four. Though he had a difficult start in life, you would never imagine he had a kidney disease. He is a bubbly, happy, full of life, little boy. Lorenzo visits his pediatric nephrologist yearly and a pediatrician every six months. We are due to go to the Royal Children's Hospital in March, 1998.

At this stage, we understand that it is important we monitor our son's blood pressure, as it will most likely rise in the near future and need medication. Every time we take his blood pressure we get very anxious. I feel like this inevitable monster is just waiting to pounce[2]. I do thank God that he is four and that it is not a problem at this stage—as I am well aware, some children have faced hypertension and other serious complications at a very early age. I would just like to finally share a question that my husband and I both had. We have often wondered if either of us were compatible to give a kidney to our son when the time came. We wanted some peace of mind, so the last time we visited our pediatric nephrologist we asked. He had records of Lorenzo's blood group and I quickly got out my blood group card from my bag. I was heartbroken—I was not compatible. My husband had a blood test and he was compatible, so that was some consolation. I feel immense relief knowing my son doesn't have to wait for a donor kidney.

Regards,
Maria DiPalma, Australia

Editors Notes:

[1] Pneumothorax is an accumulation of air in the lining of the lung walls, resulting in collapse of the lung on the affected side.

[2] 60% of ARPKD children develop hypertension by 15 years of age.

FALL/WINTER 1998 ISSUE NO. 6

LETTER FROM THE EDITOR

Dear Friends,

Have you ever felt an obligation or need to teach your ARPKD child about their chronic condition and in a sense prepare them for the future? How much information do you either keep or give him/her regarding their ARPKD/CHF situation? Do you try to protect your child from the disorder as much as possible, or do you attempt to seize opportunities to educate them whenever you can? Have you ever wrestled with how much to tell your child about their medical condition?

Certainly, what may be the best approach for one child may be wrong for another. The age, maturity, personality, and health of the child are all factors when making a decision on the extent of what is communicated. For some parents there is a thin line between protecting their child's innocence and being honest about reality. Here are some professional recommendations I have found:

- Answer their questions simply and honestly.
- Secrets cause confusion, and lies create mistrust. Even young children can sense a lie and the truth will present itself eventually, especially with a progressive disorder.

Often children will take in as much as they can, the rest may be forgotten or sit in memory until they are ready to process it. (Even if your child is not anywhere near kidney failure, you may find some guidance to this topic in the article "*An age-appropriate guide to helping your child with a transplant*" on page 4).

As you surely know, we, as parents, strongly influence our child's destiny. Our children may "own" their medical condition, but we contribute to the outcome. The beliefs, values and attitudes we aspire to, color our children's. You are what you think and often you get what you expect. We have the potential to foster negative/positive behaviors and thoughts. Even the medical/home community is influenced by the attitudes we hold. As best as you can, normalizing your child's life experiences, (school, extracurricular activities, social events, etc.), makes good sense. In addition, if you are responsible for past blunders, fix what you can and move on. Do not waste further time and energy on it. Instead, commit yourself in creating an unparalleled safe, positive, and nurturing environment for your child. I challenge you to do your best and be the best you can, for your child's sake.

SINCERELY, COLLEEN ZAK

YOUNG TRANSPLANT RECIPIENTS—an age-appropriate guide to helping your child with a transplant

BY PAMELA BOONE, RN, MSN SUSAN M. KELLY, RN, BSN KATHERINE OSWALD, RN, BSN

Imagine being awakened in the middle of the night, **taken** from your warm, cozy bed, **driven** to a place you have **never seen** before and **separated** from your family.

IT WOULD BE VERY SCARY **FOR AN ADULT,** IMAGINE **HOW SCARY THAT WOULD BE FOR** A CHILD**.**

Unfortunately, this scenario happens all too frequently. Health-care professionals strive to educate the **family** for a transplant hospitalization so the parents can then prepare their child and other family members.

The process of transplantation for a child of any age has a profound impact on the entire family. Often children do not understand why they are sick or how this happened to them. Children can blame themselves for their illness and may believe it is a result of their behavior.

Being hospitalized, however, does not always have to be thought of as a negative experience. The proper education, including age-appropriate activities such as play therapy, tours of the hospital plus talking with other parents and kids prior to hospitalization, can turn this experience into a positive one.

In an effort to help you help your child, here are some guidelines to prepare your child for the experience of transplantation. When your child is sedated, it is important to talk to and comfort your child through touch, as even sedated patients respond to sound and tactile stimulation. Once your child is off the respirator and on the general transplant unit, he or she will become more active. Because of the incision, the IV tubes and drains etc., your child will find it harder to move around. Using toys, books, videos and music to distract them is very helpful.

Infants and Toddlers (birth to two years old)

Infants and young toddlers are too young to benefit from pre-hospitalization teaching. Older toddlers can be told about the upcoming hospitalization and transplant depending on their development level. They will not understand what a transplant is all about or why they need one, but these issues can be dealt with when they are older.

During the hospitalization, infants and toddlers will benefit from having family members with them as much as possible. Having familiar toys, tapes, or a favorite blanket can help the child feel more at home. Remaining positive and non-stressed, as difficult as this may sound, can help diminish your child's anxiety as even infants and toddlers can pick up on these cues. Your child might find it relaxing to sit in your lap or go for walks in a stroller.
It is not possible to prepare an infant for a medical procedure. Older toddlers, **however**, can understand simple explanations of the procedure. Again, a familiar person being with the child and distractions like singing, watching videos, stroking the face or arms, etc. are very beneficial.

Preschool (three to five years old)

Preschool children will benefit from pre-hospitalization teaching. Children of this age do not have a good concept of time, which makes it difficult to know then to start your preparation. Compounding this is the fact that the timing of a cadaver transplant is unknown. Play therapy is especially helpful with this age group. Since children this age are very dependent on their parents, separation from parents threatens their security. Parental involvement is strongly encouraged throughout the hospitalization. Preschoolers are very aware of their parent's feelings so it is important to stay positive. They may experience some regression while they are hospitalized (i.e., using a bottle, bed-wetting), but this is usually temporary.To explain medical procedures to preschoolers, use non-threatening vocabulary and simple concrete explanations. You may use play kits and/or books to help explain the procedure to them. They are very protective of their body's intactness, so Band-Aids and bandages should be used to cover any incision. After the procedure, you may use diversionary games to help them cope.

School Age (six to 12 year olds)

School-age children can benefit greatly from pre-hospitalization teaching. After the age of seven, children are able to understand time concepts so you can begin teaching about the disease and transplant earlier. Taking tours of the clinic, hospital room and ICU, and having the child meet and speak to personnel from these areas can help to alleviate fears of the unknown. School-age children have very active imaginations and sometimes can create scenarios which are worse than reality. While you the parent may view the transplant as a "chance for life," your child will probably have a very different opinion regarding the upcoming surgery. In many instances the child may not believe that they are very ill and in need of a transplant, especially if they have a chronic illness and have adjusted to the way they feel. Honesty is of the utmost importance so that your child will continue to trust you and the medical team. To "protect" your child by not informing them only serves to complicate the situation. The child should be told about the types of tubes that will be used, what the incision will look like, how long they can expect to be hospitalized, what the typical hospital stay is like and what types of medication they will take after transplantation.

Adolescent (13 to 18 years old)

The information that is provided in the school-age section of this article is also helpful for adolescents.
In addition, as the parent of an adolescent, you are already faced with helping your child deal with the emotional and psychosocial stress associated with the transition from childhood to adulthood. Your child is dealing with developing their own sense of identity, adjustment to major physical changes, establishing meaningful relationships with their friends, peer pressure and mood swings. Add to that the stress of dealing with an illness plus the upcoming transplant surgery and you could have a very volatile situation on your hands.
The importance of supportive communication and careful preparation cannot be stressed enough. Encourage your child to talk about his or her feelings. Quietly listen when your child is talking and acknowledge his or her feelings as being real.

In summary, the more prepared you and your child are, the better your experience will be. If you or your child do not understand some of the complicated medical jargon used, ask the doctors and nurses to simplify their explanations and perhaps draw pictures. You will want to enlist the aid of the child life specialist at your transplant center who can often engage your child in conversation that they may not share with you. Encourage your child to tell their friends about the up coming surgery so they can be a source of support. During the hospitalization, encourage friends, classmates and family members to visit or write to maintain communication.—

ABOUT THE AUTHORS — Pamela Boone, RN, MSN, Susan Kelly, RN, BSN, and Katherine Oswald, RN, BSN are pediatric liver transplant nurses at the University of Chicago Children's Hospital. All have experience taking care of children of all ages. —
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A noted physician finds tangible proof . . .WHEN SCIENCE INVESTIGATES PRAYER

by Larry Dossey, M.D., Santa Fe, New Mexico

I was doing my residency at Parkland Memorial Hospital in Dallas, Texas, when I had my first patient with a terminal case of cancer. It had spread throughout both lungs. I advised him what therapy was available and what little I thought it would do. Rightly enough he opted for no treatment.

And yet whenever I stopped by his hospital bedside he was surrounded by visitors from his church, singing and praying. *Good thing,* I thought when I heard them, *because soon they'll be singing and praying at his funeral.*

A year later, when I was working elsewhere, a colleague at Parkland called to ask if I wanted to see my old patient. *See him?* I couldn't believe he was still alive. At the hospital I studied his chest X rays. I was stunned. The man's lungs were completely clear. There was no sign of cancer.

"His therapy has been remarkable," said the radiologist, looking over my shoulder.

Therapy? I thought. *There wasn't any—unless you consider prayer.*

I told two of my medical school professors what had happened. Neither of them were willing to acknowledge the man's miraculous healing. "That was the natural course of the disease," one said. The other professor shrugged. "We see this," he said.

I had long given up the faith of my childhood. Now I believed in the power of modern medicine. Prayer seemed an arbitrary frill. So I put the incident out of my mind.

The years passed and I became chief of staff at a large urban hospital. I was aware that many of my patients used prayer, but I put little trust in it. Then one day in the late '80s I came across a study done by Randolph Byrd, a cardiologist at San Francisco General Hospital. Half of a group of cardiac patients were prayed for and half were not. Those who were did better in a significant number of ways.

I could not ignore the evidence. The study was designed according to rigid criteria. It was a randomized, double-blind experiment—neither the patients, nurses nor doctors knew which group the patients were in. If the technique being studied had been a new drug or a surgical procedure instead of prayer, it would have been heralded as some sort of breakthrough.

This study inspired me to look for others. To my amazement I found an enormous body of evidence: more than 100 experiments exhibiting the criteria of good science. Many were conducted under stringent laboratory conditions; about half showed that prayer brings about significant changes in a variety of living beings. Scientists, including physicians, can have blind spots. The power of prayer seemed to be one of them.

I have since given up practicing medicine to devote myself to writing and research about prayer and how it affects our health. Here is some of what I have found:

1. The power of prayer does not diminish with distance. Prayer is as effective from the other side of the world as it is from next door or at the bedside. As researcher William G. Braud has pointed out, "The operating characteristics of the remote influence are not a function of spatial distance or time, and it is not influenced importantly by physical barriers or shields."

2. Prayer can be continuous. As a child I was puzzled by the advice "pray without ceasing." I would fight sleep as I said my prayers in bed. Eventually, sleep would overtake me and I felt as though I had failed. Only recently have I seen how prayers might continue in my subconscious.

In the fourteenth century, St. Peregrine, still a young priest, was scheduled for amputation of his leg because of cancer. The night before the surgery, he prayed fervently and dreamed he was cured. On awakening, his dream had become reality. He lived to be 80, dying in 1345 without any further evidence of cancer. An attitude of prayerfulness can exist even during sleep. As Isaac the Syrian stated, "When the Spirit has come to reside in someone, that person cannot stop praying; for the Spirit prays without ceasing in him."

3. There is no one right way to address God. I once attended a seminar given by a well-known authority on prayer, and when a man in the audience boldly asked, "Doctor, how should I pray?" the expert replied, "Ask God." It seems that there is more than one "best" way to pray.

For instance, in the coronary care experiment that so impressed me, both Protestants and Catholics were simply told to pray, not *how* to pray. Or when Herbert Benson of Harvard Medical School studied the health benefits of prayer and meditation, he found there was no difference in the effectiveness of Catholics using "Hail Mary, full of grace" or Jews using the peace greeting "Shalom" or Protestants who said the first line of the Lord's Prayer. The only contrast that could be made was that those who meditated on simple phrases instead of prayers with personal meaning to them eventually gave up.

4. Relinquishing prayers work best. In our prayers it's tempting to dictate to the Almighty, but sometimes we simply do not know what to pray for. Suppose we want to control our physiology in a way that promotes healing of a particular health problem. Should we pray for an increase or decrease of blood flow to a specific organ? For an increase or decrease in a specific type of blood cell? Or what is the best in the long run for everyone involved? These questions can be bewildering. Fortunately, research suggests that nonspecific prayer, the "Thy will be done" approach, works as well or better than when we specify the outcome.

5. Love added to prayer increases its power. One survey of ten thousand men with heart disease found a fifty-percent reduction in frequency of chest pain (angina) in married men who perceived their wives as supportive and loving. As the faith healer Agnes Sanford wrote, "When we pray in accordance with the law of love, we pray in accordance with the will of God."

Empathy, compassion and love seem to form a literal bond between living things. For example, a young boy found a wounded pigeon in his backyard. He nursed the bird back to health and gave it an identification tag. The next winter the boy suddenly became ill, and was rushed to a hospital two hundred miles away. While he was recovering from surgery, he heard tapping at the window. The boy summoned a nurse and asked her to open it. In flew the same bird. Pigeons are known for their homing ability, but this bird was traveling to a place it had never been before. Love had drawn it there.

6. Prayer is outside of time. A man diagnosed as having colon cancer asked his minister to pray for his recovery. He was not a religious man and never prayed for himself. A very private person, he told no one else about his diagnosis. When he returned to his physician later the same week, follow-up studies showed complete disappearance of the cancer. When the dates of the diagnosis, the initial prayer request, the minister's prayer and the disappearance of the cancer were compared, it was apparent that the cancer had

disappeared before the minister had actually prayed for the man.

Can a prayer be answered before it is made? It certainly seems possible. As the Almighty says, "And it shall come to pass, that before they call, I will answer" (Isaiah 65:24).

7. Prayer is a reminder that we are not alone. A patient of mine was dying from lung cancer. The day before his death, I sat at his bedside with his wife and children. He knew he had little time left and he chose his words carefully, speaking in a hoarse whisper. Although not normally a praying person, he revealed to us that recently he had begun to pray frequently.

"What do you pray for?" I asked.

"I don't pray for anything," he responded. "How would I know what to ask for?" This was surprising. Surely this dying man could think of *some* request.

"If prayer is not for asking, what is it *for*?" I pushed him.

"It isn't 'for' anything," he said. "It mainly reminds me I am not alone."

Prayer is like that. It is a reminder of our unbounded nature, of that part of us that is infinite in space and time. It is the universe's affirmation that we are not alone.

You may wish to join other ARPKD families in daily prayers according to your own faith or use the following prayer. . .
. . .*God in Heaven, we pray you will bless our children with ARPKD, including those in heaven. Protect, comfort and strengthen them and our families. We pray for the continuance of improved clinical treatment and a PKD cure in the near future****. . . .***

SIBLINGS—The Children Who Sometimes Get Lost in the Raindrops

by JUDY COLVILLE, MSW—STADTLANDERS SOCIAL WORKER

The needs of children with chronic conditions can be as plentiful and as diverse as water drops falling during a summer rain. In the middle of this rainfall of needs, though, it can be easy to lose sight of the other youngsters in the family.

The family itself is often in turmoil and may be unable to offer enough attention and support when the well child needs it most. There can be increasing financial difficulties. The parents are often absent from the home to be with the sick child. Other care-givers may not be equipped to offer the needed parental understanding. Because of the intense focus on the sick child, a sibling may feel that he's lost his parents' attention.

And this child may be grieving over the loss or lack of a "normal" family live. These feelings related to loss and grief are often difficult for siblings to recognize and understand.

The parents are not able to make the sick child well, but they can try to smooth the course for the family. I know parents who, realizing vacations would be difficult with their disabled daughter, bought a boat so that the family could still get away together.

It is important for the parents to be in tune with their own feelings as well as those of the siblings and to be creative in finding ways to address the needs of the entire family during these stressful times. There are no magic solutions, but here are some suggestions for parents:

Discuss the illness with the siblings and give them as much information as they are able to understand or want to know.

Acknowledge the challenge of the situation for the patient, parents and siblings.

Let them know that your door is open to them for future questions or concerns — including the negative feelings.

Include siblings in the care of the sick child, being careful to assess the ability of the child to accomplish the task.

Applaud efforts of siblings as meaningful and helpful.

Set limits for both the sick child and well siblings; it is important to equalize the "family rules".

Recognize the need for adolescents, regardless of their health, to pursue and gain appropriate independence.

Make a special effort to give time and attention to each sibling. These private moments can be very meaningful.

Take advantage of groups, summer camps or caretakers to provide a respite for the child and family living with an illness.

Remember that children often model their coping skills on their parents' behavior. If you are having a difficult time dealing with the illness of your child, talk to a counselor, social worker, minister or friend to address your own struggles. The better you are able to cope with the situation, the better your children will, too.

Hug your children — all of them.

Ask them for a hug when you need one.

While living with the chronic disease of a child in the family may be difficult, there are often surprising benefits. The experience may broaden, and sensitize the family tremendously. There is great comfort for the well siblings to have the assurance that when someone in their family needs care, like the sick child, it is there. They witness the strength of the family overcoming obstacles — and can feel they have played an important part in the victory. The unwelcome and uninvited rain often brings unexpected growth and beauty!

Stadtlanders social worker Judy Colville, MSW, is dedicated to serving the emotional and financial needs of Stadtlanders patients.

Author's note: The term "sick child" does not necessarily mean that the child looks or acts sick. "Sick child" is merely a term used in this article to define and designate that family member with a chronic diagnosis which impacts and affects the family in a significant way.

HELPFUL HINTS

— If your child develops hysteria from needle injections, it may help to know that at least one ARPKD child receives growth hormone injections while in a deep sleep without awakening and with great success.
— Medication elixirs sometimes need refrigeration. If your child is on a prepared formulation, check with your pharmacist as the expiration date may differ with and without refrigeration.

SPRING/SUMMER 1999 ISSUE NO. 7

LETTER FROM THE EDITOR

Dear Friends,

Several months ago the shaping of the first ever Autosomal Recessive Polycystic Kidney Disease/Congenital Hepatic Fibrosis conference was taking place **by the ARPKD Friends Group**. Since then an exciting development has occurred. At the next Polycystic Kidney Research Foundation's (PKRF) annual conference, additional topics and speakers specific to ARPKD educational needs will be provided. With this recognition of the uniqueness of ARPKD/CHF at the PKR Foundation conference, June 11-13, 1999, in Scottsdale, Arizona, there is not a need for an October 1999 conference.

The PKR Foundation's three-day conference begins Friday afternoon with Dr. Lisa Guay-Woodford, pediatric nephrologist speaking at General Session I on "The Basics of PKD", followed by a "Welcome Reception" that includes special entertainment and a silent auction fund-raiser for PKD research. A continental breakfast is provided Saturday morning before General Sessions II, III, and IV. On Saturday there is also a luncheon with table group discussions and award presentations to honor individuals who have made a positive impact on PKD. ARPKD Session I "Genetics, Diagnosis and Development of Clinical Features", by pediatric nephrologist Dr. Ellis Avner, is scheduled for Saturday afternoon. This is Dr. Avner's last year on the PKR Foundation's Scientific Advisory Committee, and possibly the last time to hear him speak on PKD issues and concerns at the annual conference. I have heard his presentations several times and they are always EXCELLENT. Both Drs. Guay-Woodford and Avner are currently doing ARPKD research. A two hour special children's program, "A Lesson on Native American Sign Language", will be offered during the Saturday afternoon ARPKD session at no additional charge.

Then for the first time ever, a "2nd Track", designed for ARPKD families will be hosted Sunday morning after the continental breakfast (breakfast incorporates special interest group table discussions) and the optional worship service. Dr. Aileen Sedman will present ARPKD Session II "ARPKD: Clinical Features." This "2nd Track" also includes a presentation by Dr. Mounif El Youssef, a gastroenterologist from the Mayo Clinic, who will focus on the cause, manifestations and treatment options of CHF, followed by a "Question and Answer" session with a physician panel. Dr. Dennis Drotar, a clinical psychologist, will address "Behavioral Development in Children with Chronic Disease" during a special luncheon following the Q & A session. Please see pages 9 and 10 for more details and registration information.

I hope to see you there!

Colleen Zak, Editor

A CHILLY PEAK AT THE NEAR FUTURE

The following article appeared in *GENEWATCH*, July 1998

GATTACA

Written and directed by Andrew Niccol; starring Ethan Hawke, Uma Thurman, Alan Arkin, Jude Law, Loren Dean, Gore Vidal, Ernest Borgnine; rated PG-13; Columbia Pictures, 1997

In video stores the movie *Gattaca* is labeled “science fiction” — for now.

At the headquarters of the Gattaca Corporation, a space exploration conglomerate is the film’s genetically enhanced future, fact-to-face encounters are so bloodless they might as well be e-mail. Members of the ruling elite, called “Valids,” start out as engineered embryos. “In-valids,” conceived the old-fashioned way, do the grunt work.

But Vincent (Ethan Hawke), a determined In-valid with a genetic predisposition for heart trouble (the delivery-room DNA printout predicted an early death) won’t settle for the janitor’s life to which his genes have consigned him. To realize his dream of space travel, he illegally assumes the identity of Jerome (Jude Law), a Valid whose legs were paralyzed in an accident.

For cash and a roof over his head, Jerome keeps Vincent supplied with the DNA samples he needs to beat the system. Because bodily fluids are tested at every turn, Vincent must keep sachets of Jerome’s blood glued to his fingertips and a pouch of Jerome’s urine strapped to his leg. He also has to meticulously scrub off the outer layer of his skin every morning, so that no dead cells will fall where they could give him away. He is metaphorically scraping off his humanity to pass as a Valid professional.

This is a carefully made, stylish film. First-time director Andrew Niccol, working from his own original screenplay, shows us the new eugenics in the flesh, and it is chilly flesh indeed. Valid clothing looks like 1940s Sunday best. The Gattaca corporate headquarters building is really Frank Lloyd Wright’s Marin County Civic Center. The plot, a slow-moving murder investigation that threatens to blow Vincent’s cover, is little more than a device to show us what the young spaceship navigator is up against.

There is no evil dictator in the film, no rebel band working to overthrow what appears to be consensual corporate totalitarianism under the scepter of DNA. (After all, who can argue with genes?) The illusion that people are free in their off hours is still enough to keep society stable. Is this our future? Maybe there will be Extra Strength Prozac.

Ironically, the movie may reinforce the ominously popular view that genes are everything. In the world of *Gattaca*, the nature-versus-nurture debate is moot. **When genetic determinism has become the conventional wisdom and people want only the “best” for their children, taking a chance on a natural pregnancy is seen as irresponsible.**

That future will not come overnight, but it certainly can sneak up on us. Genetic engineering progresses daily, and is becoming more systematized. More prenatal genetic tests are coming on line every year. Pre-implantation embryo screening for genetic diseases has already begun. **How much longer before difference becomes defect, and any child who doesn’t measure up to some arbitrary standard of health, behavior or physique is seen as flawed?** Niccol wisely takes us only a few steps beyond the present.

The movie’s main problem is an unwarranted optimism that can be summed up by its pretentious subtitle — “There Is No Gene for the Human Spirit.” The audience apparently is expected to cheer for Vincent as a self-taught, up-by-the-bootstraps hero. Outwitting the authorities against *overwhelming* odds, with some lucky breaks but at great personal sacrifice, he anxiously awaits a flight to Titan, a moon of Saturn. However, Vincent’s extraordinary discipline and tenacity are literally incredible. (How likely is it that someone would go as far as to surgically lengthen his legs to reach the height of the person he wants to impersonate?) Even if one person could muster such willpower, what difference would it make to anyone else in such a society?

Besides, how can we cheer for a character who is not much more than a cardboard cutout? Likewise, how can we empathize with the self-loathing Jerome when the movie doesn’t fully examine the new society’s mega-bias toward the disabled. (A scene in which he desperately pulls himself up a spiral staircase symbolic of the double helix is a nice stylistic touch, though.) Vincent, the wary impostor, has a good excuse for acting uptight, but none of the characters really comes alive for the viewer as a believable, multifaceted personality. The result is numbing.

Perhaps to expect more character development in this film is to miss the point. In the absence of genuine community cohesion, regimentation is chic. Without pluralism, the alienated and the dysfunctional are the only individuals left. A society dedicated to eugenics has no use for ambiguity.

Whether in film or in real life, ambiguity is worth keeping. Throughout history, for example, a rising full moon was a powerful source of wonder, until the moon walk. Nowadays, we think we know the moon as just another lonely place. It’s a high price to pay for the fruit of knowledge. The movie’s hero is excited about exploring the outer solar system, which will bleed those new worlds of their mystery as well. In this sense, he contributes to the pro-technology mindset that would keep him a janitor if his real identity were known.

Gattaca intrigues, but fails to provide the only thing that could move the public to action — a sense of outrage. It’s probably too late anyway. — Reprinted with permission from *GeneWatch.*

WHAT IS POLYCYSTIC KIDNEY DISEASE?

By Colleen Zak

Polycystic Kidney Disease (PKD) is a chronic and progressive kidney disorder. It is an inherited disease which affects both kidneys with structural renal changes. Fluid-filled cysts form, crowding out healthy tissue which often leads to end-stage renal failure. It is the most common of all life-threatening genetic diseases in the United States. It affects people of all ages, races, ethnic, social groups and sexes. There are more persons with polycystic kidney disease than there are with the following diseases combined: cystic fibrosis, muscular dystrophy, hemophilia, Down's syndrome and sickle cell anemia.

There are two types of polycystic kidney disease. One is very common, while the other is rare. The first, autosomal dominant polycystic kidney disease (ADPKD), affects 1 out of 400 persons to 1 out of 1,000 *persons* in the general population, mainly adults. The second, autosomal recessive polycystic kidney disease effects 1 to 2 per 10,000 persons, mostly children.

The age of symptom onset differs between the two types of PKD, and severity of symptoms varies greatly with both types of PKD. Though the more cystic the kidneys are and the earlier PKD is discovered, the more rapid the disease progression often is. Kidney impairment may be profound, resulting in the accumulation of waste products in the blood (uremia) and kidney failure. There is not a cure for PKD, though continuous research studies focus on locating all the genes responsible for the disease, improved clinical treatments and a PKD cure.

AUTOSOMAL DOMINANT POLYCYSTIC KIDNEY DISEASE—(ADPKD)

ADPKD is often still referred to as the "Adult" form of PKD, because symptoms typically begin in the 3rd or 4th decade of life. ADPKD diagnosis may occur prenatal to very late in life, sometimes not until the time of an autopsy. ADPKD is considered a systemic disease with potential problems in the liver, pancreas, heart, blood vessels, and intestine. Approximately 30% will develop liver cysts and the need for treatment. Eventually more than 60% of the individuals with ADPKD will develop kidney failure and the need for dialysis or transplantation.

Frequent urinary tract infections, high blood pressure, and constant or intermittent pain in the back or abdomen are common first signs of a problem. There may be cysts in the ovary, pancreas, spleen, central nervous system, and liver. Other related problems are fatigue, frequent urination, kidney stones, diverticuli of the colon, inguinal and abdominal hernias, brain and abdominal aneurysms (a weakened wall), and cardiac valvular abnormalities. Symptomalogy may vary greatly between family members.

There is often a long history of PKD in the family, as ADPKD is inherited 50% of the time from the parent who has the disease. There are at least three genetic mutations that cause ADPKD: PKD-1, PKD-2, and PKD-3.

AUTOSOMAL RECESSIVE POLYCYSTIC KIDNEY DISEASE—(ARPKD)

ARPKD has been called "Prenatal PKD", "Infantile PKD", "Childhood PKD", "Adolescent PKD", and "Juvenile PKD", depending on the child's age at diagnosis. For example, if a child was discovered to have ARPKD early, such as after birth, then that child was considered to have "Infantile PKD". If that same child did not have symptoms till later in life, say during his/her adolescent, then the label "Adolescent PKD" was given. This age-related labeling still exists, but should be obsolete, as it is confusing and antiquated. An individual either has ADPKD or ARPKD.

In contrast to ADPKD, there is almost never a family history of PKD, and it is less common than ADPKD. Carriers of the defected gene, the mother and father, may unknowingly pass the gene to their offspring. The chances of a child inheriting ARPKD is 25% for each pregnancy when both parents are carriers. The potential for each offspring to inherit the defective gene from carrier parents and become a carrier is 50%.

ARPKD frequently causes death in the newborn period. These infants are often born with profoundly underdeveloped lungs, resulting from low amniotic fluid, due to little or no fetal urine, may result in "Potter faces" or "Potter syndrome". The manifestations of this are deformities of the face (broad, flattened nose, abnormally small jaw bones and large, low-set ears) and limbs from compression in the uterus.

If an infant survives the newborn period, chances of survival are good. The child who survives the first year of life has a probability of renal survival of 67% at 15 years of age. Progression and severity of symptoms and outcome are highly variable within the ARPKD population, which results from the dysfunction of the kidneys/liver. However, 60% do develop hypertension by 15 years of age, and one-third will need dialysis or kidney transplantation by 10 years of age. High blood pressure left uncontrolled over a period of time may lead to heart failure. (Thirty-plus years ago this was often the cause of death for the ARPKD child.)

All individuals with ARPKD have some degree of Congenital Hepatic Fibrosis, which is scarring and malformation of the bile ducts. Despite this, the scarring may be only microscopic. (There seems to be different opinions in the long term survival of the ARPKD liver. Some believe that clinical treatment may never be needed, others believe that CHF is progressive, therefore, eventually it will create clinical problems.)

The spleen may massively enlarge due to portal hypertension; associated problems include bleeding esophageal varices, hemorrhoids, anemia, leukopenia and thrombocytopenia. If symptoms become severe and cannot be controlled by re-routing of the displaced blood with a shunt or such procedures as the "TIPS", then liver transplantation may be needed.

DIAGNOSIS OF PKD

If PKD is suspected an ultrasound is often sensitive enough to detect it. The most accurate tests available are a CT scan or an MRI. It is recommended that children from ADPKD families are tested only if they have symptoms of polycystic kidney disease, as the risks of diagnosis may outweigh the benefits in the children who have no symptoms. Potential detrimental effects include the "labeling" of the child and jeopardizing insurance coverage. However, each child should be considered at risk for AKPKD and his/her physician notified.

Specific gene-based diagnosis is not available at the present time for ADPKD, though linkage-based analysis is. This can be done if at least one affected family member as well as the individual at risk are studied.

Prenatal diagnosis for ARPKD may begin to be made at 20 weeks gestation with a Level II ultrasound. In addition, prenatal genetic testing is possible through Dr. Lisa Guay-Woodford, M.D. at the University of Alabama in Birmingham, Alabama. It is available to those families who have had at least one child diagnosed with ARPKD. Participation involves sending a blood sample from each immediate family member.

TREATMENTS AND INTERVENTIONS OF PKD

Treatment is aimed at preserving kidney function. It is important to find physicians who specialize in the care of PKD patients (a nephrologist or pediatric nephrologist), and with whom a patient is comfortable with. Control of high blood pressure is of paramount importance. This is to slow the progression of cystic development (and kidney deterioration) and prevent rupture of aneurysms.

Caffeine-containing products, i.e. coffee, tea, and chocolate should be avoided because caffeine may cause enlargement of cysts. A sodium restricted diet may also reduce cyst formation and enlargement, and help control hypertension. All non-steroidal anti-inflammatory agents must be avoided because they may result in kidney failure. For those with grossly enlarged kidneys or enlarged liver and spleens, it is wise not to engage in body-contact sports. Blunt abdominal trauma may rupture cysts, kidneys, liver, or spleen.

When and if end-stage renal failure is reached, treatment with peritoneal dialysis, hemodialysis, and transplantation is available. Chances of long-term survival continually improve with technical advances and newer anti-rejection drugs.

WHEN WILL YOUR CHILD NEED A LIVER TRANSPLANT?

BY JEFFREY PUNCH, MD, ASSISTANT PROFESSOR OF SURGERY, UNIVERSITY OF MICHIGAN

Timing for transplantation is very important, but unfortunately there are no absolutes to use as guides. Like the TV show "The Price is Right" where contestants try to guess as close as possible to the price without going over, one would like to wait as long as possible before doing a liver transplant, without waiting too long.

One must keep in mind that a liver transplant involves removing the child's own liver. From that moment on the child will be completely dependent on the donor liver. It's too late to decide that child was actually better off with their original liver. Unlike kidney failure and heart failure, there is no artificial means of support if the new liver doesn't do its job. While scientists are trying to develop an artificial liver, to date there is no system that performs the complex functions of a human liver reliably.

In general, if a child can live much longer (a year or more) without a transplant and still have as good a chance at survival, then a transplant would be too early. On the other hand, one waited too long if something happens which decreases the chance of survival after the transplant. Unfortunately, there is no accurate way to predict when such events will take place. As a child's liver disease gets worse and worse it becomes easier to predict what will happen, but there is still no way to be accurate.

Some children will have good quality of life for many years with "compensated" liver disease, while others will not. For example in the case of biliary atresia, some children will need a transplant within the first year of life, some not until elementary school age, and some not until adolescence or even into adulthood.

Compensated liver disease is when the disease exists but there are either few symptoms, or the symptoms are mild and stable. Decompensation is when a new and dire set of symptoms appears, especially if these new symptoms cannot be effectively treated. For example, a child with liver disease may develop ascites which can be effectively treated with a diuretic (water pill). The child is then said to have compensated liver disease. If after months to years the ascites suddenly will not go away even if diuretics are used, this would be decompensation. Patients are referred for transplant when they begin to show signs of decompensation.

Not every physician will agree on who is compensated and who isn't. Like the terms "mild" and "severe," the terms compensated or decompensated are not the same in everyone's eyes. Your physician should answer this question individually for your child, based upon his or her own experience and medical judgment.

Certain signs portend a bad prognosis, meaning that death from liver failure is likely within a year. These signs include encephalopathy, spontaneous bacterial peritonitis (SBP), and profound coagulopathy.

Encephalopathy is when the liver can no longer clear the wastes in the blood. These wastes build up and cause first a sort of

sleepiness, then more profound lethargy and eventually coma.

SBP is when fluid called "ascites" collects in the abdomen, and this fluid gets spontaneously infected. It is thought that the infection may come through the walls of the bowels. The symptoms usually include abdominal pain and fever, but not always. Sometimes SBP will cause encephalopathy and this will be the only symptom.

Coagulopathy is when the blood fails to clot normally. The liver synthesizes the proteins in blood which cause blood to clot. When the liver isn't making enough of the protein "factors," the patient has coagulopathy. Coagulopathy is measured by the pro-time (PT). When the PT is longer than normal (usually about 10 seconds is normal), the patient has coagulopathy.

In general, if the child's over health is not getting any worse, it is usually better to wait. But if they are not growing or if they are having trouble with encephalopathy or ascites, and they are certain to continue to get sicker, they should be transplanted as soon as possible. It is always better to transplant before the child becomes very ill and needs an urgent graft. Urgent transplants always have worse success rates.

One final note: Some patients require transplantation even if their liver is functioning well! Examples include patients with tumors that cannot be removed without removing the entire liver, and children with certain diseases of metabolism due to inherited diseases. In these cases, the term "compensation" doesn't really apply to their problem, and their circumstance require unique considerations.

Fortunately, most children get the livers they need before they become critically ill. Living donor liver transplants (LDLT) continue to play an important role in solving the problem of timing. LDLT can be scheduled before the child becomes so sick their chances for a successful transplant are diminished. However, LDLT is not always the best option. As of 1998, the overall results for LDLT appears to be at least as good, if not better, for babies and small children compared to grafts from deceased donors (cadaveric grafts). But for larger children (over 40 pounds), the results for cadaveric grafts are at least as good if not better. For children who are nearing adult size (75+ pounds), the results for living donor grafts still looks to be inferior compared to cadaveric grafts. Different programs and doctors have different opinions regarding the issue of LDLT and the state of the art is constantly changing.

—Reprinted from *C.L.A.S.S. Notes*, Summer 1998 issue

About the liver

The liver is located behind the lower ribs, below the diaphragm on the right side of the abdomen. In a man with an average build, it is about the size of a football and weights a little more than 3 pounds.

A miniature refinery, the liver processes chemicals necessary for the body's overall functioning. The liver:

- Manufactures and exports bile that is used by the intestines during digestion.
- Modifies drugs taken to treat disease so they are used efficiently by the body.
- Cleanses the blood of toxic substances either ingested or produced by the body itself.
- Regulates the blood's ability to clot.
- Governs the transport of fat stores.
- Stores extra vitamins, minerals and sugars to prevent shortages.
- Produces quick energy as needed.
- Controls the production and excretion of cholesterol.
- Breaks down alcohol.
- Monitors and maintains the right level of numerous chemicals and drugs in the blood.
- Maintains and controls hormone balance.
- Helps the body resist infection by producing immune factors and cleansing bacteria from the blood.
- Stores iron.

—Source: "Dealing With Liver Disease" from the Physicians Desk Reference Family Guide to Prescription Drugs.

LETTER FROM A READER IN Hawaii:

2/10/99

Dear Colleen,

I have wanted to call and talk to you personally, but since you are in Pennsylvania and I live in Hawaii, time zones seem to get in the way.

My 6 1/2 year old daughter, Megan, was diagnosed with ARPKD in Oct. 1998. This was quite a shock for us, since she has always seemed healthy. In fact, the diagnosis came from a routine school physical in which the pediatrician noticed that Megans' spleen and liver are enlarged. She then sent us to an oncologist for further tests. After many procedures we were told about ARPKD. Megan seems to be mostly affected in her liver, though she does have cysts on her kidneys. She was hospitalized for two weeks after a liver

biopsy which required two emergency surgeries. Megans' 8 1/2 year old sister, Caitlin, has had an ultrasound and appears normal. My husband is in the Army and we had just moved to Hawaii two weeks before our medical journey began. I am very interested in connecting with other parents of ARPKD kids. Since my family is in California and I am just building a network of friends here I am feeling isolated and confused. Our doctors can only answer some of my questions and fears. I would love to receive the ARPKD newsletter. I'm especially interested in talking to parents whose kids are older and may have more problems with their livers.
Thank you for giving me and others like me a chance to connect to others who understand our fears and concerns.

Sincerely, *Sam Mulvihill*

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IDEAS FOR SELF-CARE

- Remind yourself to slow down.
- Learn to do one thing at a time.
- Count your blessings.
- Unplug your phone.
- Take a bubble bath.
- Write notes to yourself.
- Do something you enjoy every day.
- Take up hobbies you enjoy.
- Get a "Not Now" sign: Use it when you don't want to be interrupted.
- Relax your standards: Accept the fact that you can't do everything.
- Allow time every day for privacy, quiet, and introspection.
- Learn to laugh: It releases endorphins into your system which are the body's natural painkillers.
- Talk out your worries: Sometimes another person can help you see a new side to your problem.

—From *Home Care Family Newsletter*, Children's Hospital of Philadelphia, Philadelphia, PA.

FALL/WINTER 1999 ISSUE NO. 8

1999 Annual Conference-COMMENTS FROM ATTENDEES

—This year we attended the 1999 Annual Conference. As a result of Colleen Zak's hard work, the parents of ARPKD children were exposed to the cutting edge information related to PKD from the most noted researchers.

I will admit I was a bit skeptical about how much attention was going to be placed on ARPKD vs. ADPKD. My fears were for the most part never realized. To our family it is a given that the ARPKD tract on Sunday was without a doubt very beneficial. Listening and speaking to other ARPKD parents was an invaluable experience.

As for the general seminars, just as in any classroom experience, "You get what you put into it". For example, here is a list of some topics and what we learned:

1) Basics of PKD by Lisa M. Guay-Woodford, M.D.; Dr. Woodford talked for about 2 hours and spent only 15 minutes on ARPKD. However, during her description of ARPKD's mechanisms of hypertension she described how captopril is an angiotensin converting enzyme inhibitor (ACE inhibitor). Our son is on captopril and now we understand how and why it works.

2) Research Update by Arlene Chapman, M.D.; it was during this seminar that we learned how the PKD2 gene in ADPKD is like ARPKD at the tissue level. Therefore, it is important for us as parents of ARPKD children to follow the advances in ADPKD research.

3) Extrarenal Manifestations of PKD by Robert Schier, M.D.; During this seminar we learned of erythropoietin (EPO) which is released by the kidneys and is related to red blood cell production. In speaking with another ARPKD, parent their child had injections

of EPO administered when the child's hemoglobin level was at 8. Our son does not need the injection; however, we are watching his levels carefully because he is slightly anemic. Dr. Schrier also helped to clarify the role of angiotensin. As you may know, Captopril is an angiotensin converting enzyme inhibitor. Angiotensin constricts blood vessels and regulates aldosterone release, which regulates salt retention by the kidneys. Since ARPKD children have difficulty concentrating their urine, it is important to understand that excessive consumption of foods high in sodium can worsen an existing problem.

My wife and I can think of many other things we would rather do than learn the vocabulary and definitions related to ARPKD, but we have no choice. I am sure many others feel the same. Our hope is that a cure will be found for all those children approaching renal or liver failure, anti-rejection drugs will improve so that those transplanted children will never require further surgeries, and the ARPKD gene will be isolated and treated prenatally.
Let's all educate ourselves to help this process along. «

—Brian & Kris Kopan,
New Holland, PA

Lori Spier writes:

As the mother of a toddler, who was diagnosed with ARPKD at 36 weeks gestation, among many other emotions, I initially felt powerless to help my child. However, after becoming involved with the ARPKD Support Group I have armed myself with knowledge. As this disease is commonly misunderstood by many, including doctors who don't specialize in this area of expertise, it is imperative that parents learn all they can about the progression of the disease, the problems associated with the disease process, and the treatments available, as well as how to raise funds for research.

The conference began on Friday with a very basic overall discussion of PKD by Dr. Lisa Guay-Woodford. On Friday evening, a silent auction and reception was next on the schedule. Items up for auction ranged from homemade crafts to beanie babies to sports memorabilia. The reception was a great way to renew old acquaintances for some, and for others to begin new friendships.

There were approximately 420 participants from over 40 states, including a few people who came from Canada and Peru! As this is such a rare disease, it was a great opportunity to speak with other families who are going through the same or similar circumstances.

This year was the first year that the conference organizers included a separate track just for ARPKD families. Although there were other General Sessions that were offered, I personally found that the ARPKD Sessions addressed the specific concerns of ARPKD, while the General Sessions were directed more towards the dominant PKD.

The ARPKD lectures were given by Dr. Aileen Sedman from the University of Michigan, Dr. Mounif El-Youssef, from the Mayo Clinic in Minneapolis, MN, and Dr. Dennis Drotar, from Rainbow Babies and Children's Hospital in Cleveland, Ohio. All of the lectures were very understandable and the doctors answered any and all questions after the lectures. Not only did the doctors discuss the various aspects of the disease that affect the kidneys and the liver, but they discussed practical ways through proper nutrition that you as a parent can do to help prolong the good health of your child.

There were approximately 40 families with ARPKD who were represented at this conference and hopefully more families will join us next year in the "Windy City".«

—Lori Spier

My name is Art Margolis, and my son Jake who is five has ARPKD.

I wanted to take a few minutes and tell you about some of our experiences at the 1999 Polycystic Kidney Research Foundation Conference in Scottsdale, AZ. This was the 10th Annual Conference on Polycystic Kidney Disease and this year's theme was Taking Control of PKD.

My wife, Susan Light, and I went to the conference with our children, Jake and Zoe. For most of the meeting I was glad that we had come with the children. We have almost always been honest with Jake about his disease, he knows that his kidneys are different than other children's, and that he needs to take medication that other children don't and have his blood pressure tested at home. He has been in the hospital, and goes for regular blood and urine tests.

During lunch on Saturday Dan Larson, President of the Polycystic Kidney Research Foundation, was introducing their new campaign called "END PKD" and showed a new video that they have put together to be used to promote the campaign nationwide. The video is very effective at getting people's attention by making them aware of PKD, and I was crying while watching it. The video focused on ADPKD, but did include one family with ARPKD. The bad news is that Jake was also paying attention to the video, and he got more information than he needed to know. He became very upset and had to leave the luncheon. The question that this brings up to our family is should he have been there to be exposed to this much information? The answer is that we need to be a little more careful the next time, I would bring him next time, but Susan has concerns.

I do need to tell you that the Foundation had a special class for the children who attended the meeting that was wonderful. They had an American Indian who taught them Indian sign language and how to make and use native tools and musical instruments. Both of our children had a wonderful time at this class.

In closing regarding this point, I hope that more families bring their children next year, so we can have them interact together as normal children and have fun!

One of the things that made me very happy were all of the "ARPKD Fast Track Meetings". The meetings were informative and worthwhile, but hearing and meeting Dr. Aileen Sedman and Dr. Lisa Guay-Woodford was the highlight for me. I am glad to have them on our side!

The big news is that the Foundation has already reached 50% of their $5 Million Dollar goal to set up four centers specially designed for research into ending PKD. We all need to search our hearts as well as our wallets to find the needed funds!

The Silent Auction was a real blast and raised $8,400 while we had a great time.

The meetings were expertly planned and coordinated by Tim Mastin, the Program Manager for the PKR Foundation. The hotel was beautiful, the food was good, and the weather was hot.

This was our first time at one of the national meetings, and it was great to interact and network with other families with children with ARPKD. We made some new friends as well as seeing some old ones.

We did take the opportunity to check out the local parks and found the Paradise and Pacific Railroad where they had ¼ scale steam trains to ride on, went swimming, and drove into the desert. We all enjoyed our extracurricular activities!

We are looking forward to seeing more of you at the meeting in Chicago, June 9-11, 2000.

One last note: We all owe *Colleen Zak* a great deal for pushing ARPKD into the forefront at the PKR Foundation, and with the special ARPKD "fast track" meetings held at the conference. «

—Art Margolis

CONGENITAL HEPATIC FIBROSIS—THE LIVER LESION OF ARPKD

Mounif El-Youssef MD
Mayo Clinic and Foundation
Rochester, MN

Introduction

The kidney lesion of ARPKD is the more serious part of this condition. The liver is affected in patients with ARPKD to a variable degree. However, all patients with ARPKD have liver abnormalities.

The liver develops in the embryo after close contact between the future intestine and the muscle of the heart. Close touching of the cells is required otherwise the liver does not develop normally. The liver is made up of cells that control metabolism of sugars, fats, and proteins. The liver cells also eliminate toxins produced by the germs that live in the large bowel, and they eliminate the drugs that we may take for a variety of ailments.

The liver cells also produce bile. The bile is essential to help digest the foods we eat and is a vehicle of elimination of many unwanted substances. The bile is collected around the liver cells by small pipe like structures called the bile ductules. These canals are supported by fibrous tissue.

ARPKD and CHF

Autosomal recessive polycystic kidney disease (ARPKD) is one of the most common congenital renal lesions in pediatrics. The condition is often associated with early renal failure depending on the severity of the lesion. Congenital Hepatic Fibrosis (CHF) is present in the livers of patients with ARPKD. **With marked improvement in prenatal diagnosis and in therapy of chronic renal failure, life expectancy has improved dramatically for these children. As such the manifestation of the slowly progressive fibrosis and portal hypertension become evident.** The liver in CHF is hard and grossly shows areas of fibrosis. Under the microscope, the liver has a lot of fibrosis and the bile canals are in disarray. The fibrosis surrounds normal appearing liver tissue. The patients with CHF have normal liver function although some may have significant elevation of the biliary enzymes. Because of the excess fibrous scar tissue, blood flow to the liver is impaired. The blood being a liquid has to go elsewhere and it goes back to engorge the spleen and to engorge the veins of the esophagus. The result is an overactive spleen and the potential risk that the veins of the esophagus may bleed. The association of ARPKD and CHF is almost universal. A few isolated cases of CHF without renal involvement have been described. More importantly, the liver lesion is present even when the patient has not developed any signs of portal hypertension. The slowly progressive fibrosis with subsequent development of portal hypertension in the absence of cirrhosis or deterioration in liver function is unique to CHF.

Diagnosis

The diagnosis of CHF is clinical. The infant and child is first diagnosed with renal disease and examination of the abdomen reveals the large and hard liver. More subtle, is the diagnosis of spleen engorgement. A hint of an overactive spleen comes from the fact that it clears old cells in the blood. When it is overactive it also clears healthy young ones and the number of cells becomes lower with time. Ultrasound may detect the engorgement before any dysfunction of the spleen occurs. Likewise, it may detect subtle changes in the flow of blood and point to increased pressure in the portal vein supplying the liver.

Definite diagnosis of the extent of bile canals disarray can be obtained from a special Magnetic Resonance Imaging of the bile ducts called MRCP. It may help the physician have an estimate of the degree of disarray.

Treatment

There is no specific treatment for the hepatic fibrosis. The process of fibrosis has only recently been investigated at a cellular and molecular level. However, the understanding of the peculiar form of fibrosis unique to ARPKD is unknown. The relationship between the fibrosis and the disarray of the bile canals is also unknown. How to tie the liver and the kidney lesion together is also under intense study. Identification of the gene will help, and as usual more questions will arise. In the fall, at the national meeting for liver disease, a group of scientists including myself will meet to form a interest group in the developmental diseases of the biliary system and in antifibrotic therapy. «

GROWTH HORMONE THERAPY

BY

Bradley Warady, MD is Director, Dialysis and Transplantation and Chief, Section of Pediatric Nephrology

for Children's Mercy Hospital in Kansas City, MO.

Progressive growth retardation is an all too frequent characteristic of children on dialysis and children who receive a kidney transplant. The result is a substantial percentage of patients (as many as 60 percent in one study) who develop end-stage renal disease (ESRD) during childhood, develop marked short stature and attain a final adult height at or below the third percentile on a standard growth chart. Although a variety of factors play a role in the varying degrees of growth impairment seen in children with renal disease, the patient's age at the onset of renal insufficiency can have a significant impact. It is well recognized the younger the age of the child at the onset of renal disease, the more severe the growth impairment tends to be. While complications such as malnutrition, renal osteodystrophy (bone disease) and acidosis may also contribute to the poor growth rates and need to be treated aggressively in all patients, abnormalities related to the function of growth hormone and related substances exert their influence during mid-childhood (age three to puberty) and have the most profound influence on growth delay in this population.

Growth hormone (GH) is synthesized in the pituitary gland and indirectly promotes the growth of a child by stimulating the production of insulin-like growth factor 1 (IGF-1). IGF-1, which directly promotes cell growth, is produced in the liver, growth cartilage and many other tissues. Its production is in part dependent on an adequate dietary protein and calorie intake in addition to the presence of GH. Paradoxically, many children with renal disease and growth retardation have normal or elevated levels of GH in their blood. In contrast, the levels of IGF-1 that are needed to stimulate growth are low. The low levels appear to be due to the presence of proteins in the blood that are normally removed from the body in urine, but accumulate in kidney failure. These proteins bind to and reduce the function of IGF-1 which results in the suboptimal height velocity characteristic of children with impaired kidney function.

The initial trials using man-made or recombinant human growth hormone (r-HuGH) therapy to increase the growth rate of children with renal insufficiency occurred just 10 years ago. Subsequent studies conducted over the past five years have provided indisputable evidence that infants and children with renal disease and growth retardation who receive daily r-HuGH, typically by subcutaneous (under the skin) injection, grow faster than children with similar kidney function who do not receive r-HuGH therapy. In most studies and in clinical practice, the indication for starting r-HuGH therapy has been growth retardation as defined by a height less than the third percentile, although a very poor growth rate has been the stimulus for starting treatment in some centers. Recombinant human growth hormone appears to work by increasing the availability of IGF-1 to directly stimulate growth and is only administered to children with "growth potential" as evidenced by open bone growth plates on X-ray evaluation. The use of this therapy has allowed some recipients to experience a faster rate of growth than their peers, or so-called "catch up growth". Because of the growth inhibiting effects of uremia, in association with the need for dialysis and steroids following transplantation, (see paragraph to follow), **the provision of recombinant human growth hormone (r-HuGH) during the pre-ESRD period is likely the optimal approach to take when the goal is to prevent or correct the growth retardation that occurs in these children.**

Whereas r-HuGH has been used by a substantial number of children with chronic renal failure (CRF), the use of this therapy in the pediatric dialysis and transplant population has been more limited. As in the case of patients with CRF, the use of r-HuGH by children on dialysis has resulted in enhanced growth rates during the first year of treatment, although at a lower rate than seen in association with pre-ESRD therapy. Whether or not an increased dose of r-HuGH might further enhance the growth rate of these patients without the development of treatment related complications is currently being studied. Adequate nutrition and control of renal osteodystrophy are prerequisites for a successful response and all dialysis patients who receive r-HuGH should have their bone disease monitored closely with repeated physical examinations and assessments of their intact parathyroid hormone (PTH) level.

In children who receive a kidney transplant at more than six years of age, spontaneous "catch-up growth" is unlikely, even with a properly functioning transplant. Here too, preliminary studies have provided evidence that r-HuGH usage can increase the growth rates of these patients. However, the possibility does exist that r-HuGH might also increase the risk of transplant rejection. This issue is of concern to some transplant physicians and has been a major reason there is hesitancy to use the therapy in this setting. A multi-center study addressing the safety and efficacy of r-HuGH in the transplant population will be completed within the next one to two years and clinicians anxiously await the results of this important investigation.

To date, many children who are growth retarded and who have kidney failure do not receive r-HuGH because of an apparent lack of substantial patient, parent and physician concern about the short stature when the patient is young. Unfortunately, many of these same children later desire the therapy when there is little time available for growth. Accordingly, strategies designed to prevent this life-long complication of renal insufficiency must include educational programs designed for patients and parents that address the following: 1) the natural history of growth retardation associated with CRF and ESRD, 2) the limited "window of opportunity" children have for growth and 3) the results of *studies* and clinical experiences with r-HuGH therapy in children with renal insufficiency. Only in this manner is it likely that a substantial percentage of the children deserving of this effective therapy will benefit from its use. **«**

"Note: The original title of this article is *Growth Hormone Therapy for Children with Kidney Failure*. Bradley Warady, MD is Director, Dialysis and Transplantation and Chief, Section of Pediatric Nephrology for Children's Mercy Hospital in Kansas City, MO."

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ANYTHING BUT ROUTINE

The majority of kidneys used in transplantation are recovered from cadaver donors – people who have died in a way that is compatible with the recovery of organs and whose families have consented to donating the organs. The other source of kidneys is a living donor. The decision to donate a kidney can only be made by you, the individual considering donation. This decision must be completely voluntary, and it helps to know as much as possible about the potential medical risks before you proceed with the medical evaluation. There may be financial considerations as well.

Once a prospective donor discusses donation with his/her family members and the potential recipient, the next step is to call a transplant coordinator. The coordinator will explain the living donation process and schedule an appointment for you with the transplant team.

A medical evaluation or workup is done on all potential donors to make sure the donor is in good health and that he/she has adequate kidney function. In most cases, the workup is completed on an outpatient basis and hospitalization is not required.

Ideally, the donor should be between 21 and 55 years old. Medical conditions preventing donation include kidney disease, either in the donor or in the donor's immediate family; cancer; diabetes; coronary artery disease; hypertension; obesity; malnutrition; psychiatric illness; a history of autoimmune disease (such as lupus); or substance abuse, including tobacco.

The first test of the medical evaluation is your blood type, done prior to your appointment with the medical team. The donor and recipient must have the same or compatible blood types. Every human being has one of four basic blood group types: A, B, AB, and O. The chart below explains which groups are compatible.

AB blood group, called the universal recipient, is the rarest type, but is the easiest to match because it accepts any blood group. Blood group O, called the universal donor, is the most common – it can donate to all groups – but will not accept any other blood group.

This test is done from a small sample of your blood. It is easy and relatively inexpensive to perform. If the blood type is incompatible, there is no point in proceeding with the remainder of the evaluation.

If you are blood type compatible with the recipient, you are then evaluated by the transplant team. If you live out-of-state, the transplant coordinator can arrange for this evaluation to occur at a transplant center or with a nephrologist, a kidney specialist, near you.

Otherwise, you would be scheduled to come to the transplant clinic to meet the transplant team. A nurse coordinator will get your medical history and explain the entire process of donating a kidney. You also will meet with the financial coordinator who will review your insurance coverage for the transplant. Most often, the recipient's insurance, not yours, covers the cost of testing and surgery. A dietitian will assess your nutritional status and ideal body weight. The social worker will discuss the emotional impact of donating an organ to someone, and make sure your decision to donate is informed and completely voluntary.

You will meet with a transplant surgeon who will do a physical examination and discuss your family medical history with you. He/she will explain the surgical process and the medical risks involved with the removal of a kidney, referred to as "donor nephrectomy". A nephrologist will also examine you, take a medical history, and discuss the long-term risks of living with one kidney.

After meeting with each transplant team member, the following laboratory assessments are done to determine compatibility between you and the recipient:

HLA (Human Leukocyte Antigen) Typing or Tissue Typing

This blood test identifies the most important genes that make you unique from an immune system perspective. Presently, we test for 6 HLA markers or antigens. Each person usually has 6 antigens, 3 that are inherited from their mother and 3 from their father. They are labeled A, B, and DR. Each gene from each parent has a number.

As you can see from the pattern of inherited antigens (Figure 1), a parent and child will almost always be a 3 out of 6 antigen (or half) match. Siblings, however, have a 25 percent chance of being a perfect 6 antigen match, a 50 percent chance of being a half match, and a 25 percent chance of being a 0 antigen match.

HLA match alone does not guarantee a successful transplant. This is evidenced by the fact that a recipient of a 6 antigen match kidney, in theory a perfect match, still requires some immunosuppression to prevent rejection.

Another important test to determine compatibility between the donor and the recipient is called crossmatch.

Crossmatch

This test is performed in the tissue-typing laboratory by mixing a small sample of blood cells from you, the potential donor, with the

serus (fluid part of blood) from the prospective recipient in a test tube, and then examining it under a microscope. If the donor's cells die, the crossmatch is termed positive. This means the recipient has antibodies to your tissue. Although we say "positive", this means that a transplant cannot be performed, because it would result in immediate rejection of that particular organ. It is possible that a recipient could have a positive crossmatch even with a 6 antigen HLA match.

A negative crossmatch means that your cells are not destroyed and the recipient has not formed antibodies to your tissue. The crossmatch must be negative for the surgery to occur.

All of these immunologic tests help us find the best possible donor for each recipient. However, these tests do not predict who will experience rejection and who will not.

Other lab tests required for the living donation workup are:

CBC (Complete Blood Count)

This test checks your blood count to rule out anemia, infection, or other abnormal blood conditions.

Chemistry Profile or SMAC.

Measures kidney and liver function, blood sugar level, and other functions of body metabolism. Any abnormality may require further testing.

PT (Protime) and PTT (Partial Thromboplastin Time).

Measures the clotting ability of your blood. This is done to ensure that you are not at risk for abnormal bleeding during the surgery.

You will be screened for several viruses and bacteria which may be passed on to the recipient by the transplanted kidney. The tests involved are:

CMV (Cytomegalovirus).

Your blood will be tested for Cytomegalovirus. Most people have been exposed to this virus but have few or no symptoms. However, when a person's immune system is suppressed, as it is in the transplant recipient, symptoms may develop. We need to know whether you have been exposed to the virus prior to donating, so the recipient can be treated to prevent the virus from becoming a problem. **«**

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GLOSSARY OF LABORATORY TESTS — A LOOK AT COMMON LAB TESTS

Below is a list of some of the common laboratory tests that can be important for kidney patients. Provided in the glossary is an explanation of what an "increased" or "decreased" reading might mean—that is, if it's either higher or lower than what is normal for you.

BLOOD TESTS:

BLOOD UREA NITROGEN (BUN)—is a waste product of protein breakdown that is removed from the blood by the kidneys. It is a very important test of kidney function.
Increase: may mean that your kidney is not functioning properly, or that your diet is too high in protein. Dehydration and some anti-rejection medicines may cause a rise in your **BUN** level.
Decrease: may be a sign of liver disease or too much water in your body.

CHOLESTEROL (Chol)—is a waxy, fat-like material carried in your blood. It helps your body make hormones and build cell walls.
Increase: can lead to narrowing or blockage of blood vessels, which can, in turn, lead to heart or circulatory system disease. Eating fatty foods up to 12 hours before the test may cause a higher level. Certain anti-rejection medications may also increase the cholesterol level.
Decrease: sometimes indicates that the liver is not working properly.

CREATININE— (pronounced kree-at-a-neen) is a protein waste substance produced by your muscles and released into the blood stream where it is removed by your kidney. The substance itself is harmless, but if your kidney isn't working well, the amount of creatinine will rise. This is the test that many kidney transplant patients watch most closely because it is an important measure of how well your kidney is working.
There are two ways of doing the test:
SERUM CREATININE LEVEL — measures the creatinine in your blood. The normal level of creatinine in your blood usually depends on how large and muscular you are, and your age. It remains fairly steady from one day to the next unless there is something wrong.
CREATININE CLEARANCE TESTS — is a more reliable measure of kidney function, since it doesn't depend on the amount of muscle you have. For this, you will have to collect your urine for 12 or 24 hours.
Increase: If your creatinine level increases, it could be a sign of kidney rejection. However, it could just be due to a temporary change, a side effect of a medication you are taking, a need for you to drink more liquids or even an error in the lab test.

Decrease: (not applicable)

HEMATOCRIT (HCT) — This test measures the percentage of oxygen-carrying red blood cells (RBCs) in your blood. It is a routine part of a complete blood cell count (CBC).
Increase: too many RBCs can thicken your blood and cause problems with unwanted clotting.
Decrease: could be a sign of anemia (too few RBCs in the blood) which can cause weakness, dizziness, fatigue and, in severe cases, breathing difficulties and heart problems.

HEMOGLOBIN (Hgb) — This is a pigment found in red blood cells that carries oxygen to the body from the lungs.
Increase: may indicate dehydration, heart disorder, or blood disorder.
Decrease: could be a sign of anemia.

PLATELETS (Plts) — (also called thrombocytes) in the blood help to stop bleeding by clumping and forming a clot around the injury.
Increase: too many platelets can make your blood thick.
Decrease: can cause you to bruise more easily and bleed a little longer. It may be a sign of kidney or liver disease, internal bleeding or anemia.

WHITE BLOOD COUNT (WBC) — This test measures how many white cells (leukocytes) are in your blood. Some of these cells are involved in fighting off infections and are also involved in kidney rejection.
Increase: may indicate that you have an infection, inflammation or dying tissues. Stress can increase the count, as can certain anti-rejection medications.
Decrease: can mean too few infection-fighting cells to protect your body. Certain medicines—like antibiotics or diuretics—can lower the WBC.

ELECTROLYTES (help balance chemical processes):

BICARBONATE (HCO_3)—This measures the acid/base balance of your blood as it is controlled by the kidneys. An acid is a strong solution (like stomach juices) that can dissolve foods and other substances. A base (also called an alkaline) is the opposite of an acid and can also be strong or burning—household ammonia is a base solution.
Increase: the blood becomes more base (alkaline), which can result from prolonged vomiting, a lung disorder or from taking too many antacids, such as sodium bicarbonate tablets.
Decrease: means there is too much acid in your blood, and it can make you feel sick or short of breath. This may be a sign of diabetes or kidney failure.

CALCIUM (Ca)—measured in your blood, is needed for blood clotting, to build strong bones and for normal muscle functioning. It also helps with heart and nerve function.
Increase: may indicate too much calcium intake (such as by the overuse of stomach antacids), bone disorders, too much vitamin D or problems with the thyroid or parathyroid glands.
Decrease: might suggest an inflammation of the pancreas, kidney failure, too little vitamin D or too much water in your body.

MAGNESIUM (Mg) — Your body needs magnesium to perform many functions, such as blood clotting, the breakdown of carbohydrates and proteins and carrying impulses along nerves.
Increase: can be a sign of kidney disease, diabetes or thyroid disorders.
Decrease: can lead to muscle weakness, sleepiness and problems with the heartbeat. Certain anti-rejection drugs and antibiotics may lower the level.

PHOSPHOROUS (PO_4) — in the blood works with calcium to build strong bones, and with other chemicals as a source of energy.
Increase: may signal kidney failure, an acid condition of the blood or too much phosphorous in the diet.
Decrease: is sometimes seen in bone disorders, too little vitamin D or as a complication of diabetes. Some antacids can also lower the level.

POTASSIUM (K) — is very important for changing carbohydrates into energy and building protein. It also helps the heart, muscles and nerves function properly. Most of the extra potassium in the body is excreted (given off as waste) by the kidneys.
Increase: can lead to heartbeat problems and too much acid in the blood. It can indicate that your kidney is not working well. Certain anti-rejection drugs may increase potassium level.
Decrease: may be due to diuretics (substances, like caffeine, that cause you to give off excess water in the urine). Too low a level can lead to heart problems.

SODIUM (Na) — This blood test indicates the balance between electrolytes and water in your body. It also helps evaluate nerve or muscle disorders, and kidney or adrenal gland problems.
Increase: may indicate excessive sodium in the diet, not enough water in the body or kidney-function difficulties.
Decrease: may indicate chronic kidney disease or inadequate sodium intake.

URINE TESTS:

PROTEIN (Proteinuria) — Proteins are basic components of all living cells and are not normally eliminated from the body in the urine.
Increase: any unexpected protein in the urine may indicate a kidney or urinary tract disorder.
Decrease: (not applicable).

URIC ACID — This test measures uric acid in the urine, which is a waste by-product of energy production by the body.

Increase: may indicate gout, liver disease or ulcerative colitis.
Decrease: may be due to kidney disease, certain drugs or large doses of vitamin C.

OTHER LABORATORY TESTS:

URINE CULTURE — This test confirms if there is bacteria causing an infection in the urinary tract. If it is **POSITIVE,** you can then be given appropriate antibiotics.

A NOTE ON TAKING LAB TESTS

Before going for any laboratory tests, it's important that you follow your doctor's instructions on what you should or shouldn't eat, and when you should take any medications. Not following instructions could cause inaccurate test results. Also, while it is wise to be concerned about any unusual test results, you should ask your doctor what those results might mean, if anything, and what you should do about them *before* you start worrying.

BLOOD PRESSURE:

Although blood pressure is not read in the laboratory setting, for the PKD person, it is very important to monitor. Blood pressure is the measure of the force of blood in your arteries. Two types of pressure are measured: systolic and diastolic. Systolic is the pressure created by the heart squeezing blood into your circulatory system. Diastolic is when the heart relaxes between beats. Blood pressure is stated as the higher reading (systolic) over the lower reading (diastolic), for example, 110/75.«

WELCOME TO HOLLAND

I am often asked to describe the experience of raising a child with a disability — to try to help people who have not shared that unique experience to understand it, to imagine how it would feel. It's like this …..

When you're going to have a baby, it's like planning a fabulous vacation trip — to Italy. You buy a bunch of guidebooks and make your wonderful plans. The Coliseum. The Michelangelo David. The gondolas in Venice. You may learn some handy phrases in Italian. It's all very exciting.

After months of eager anticipation, the day finally arrives. You pack your bags and off you go. Several hours later, the plane lands. The stewardess comes in and says, "Welcome to Holland."

"Holland?" you say, "What do you mean, Holland? I signed up for Italy! I'm supposed to be in Italy. All my life I've dreamed of going to Italy."

But there's been a change in the flight plan. They've landed in Holland, and there you must stay.

The important thing is that they haven't taken you to a horrible, disgusting, filthy place, full of pestilence, famine and disease. It's just a different place.

So you must go out and buy new guidebooks. And you must learn a whole new language. And you will meet a whole new group of people you would never have met.

It's just a different place. It's slower-paced than Italy, less flashy than Italy. But after you've been there for a while and you catch your breath, you look around … and you begin to notice that Holland has windmills … and Holland has tulips. Holland even has Rembrandts.

But everyone you know is busy coming and going from Italy … and they're all bragging about what a wonderful time they had there. And for the rest of your life, you will say, "Yes, that's where I was supposed to go. That's what I had planned."

And the pain of that will never, ever, ever, ever go away … because the loss of that dream is a very significant loss.

But … if you spend your life mourning the fact that you didn't get to Italy, you may never be free to enjoy the very special, the very lovely things about Holland.

—Anonymous

SPRING/SUMMER 2000 ISSUE NO. 9

POLYCYSTIC KIDNEY DISEASE: AN OVERVIEW AND COMMENTARY *by* *Colleen B. Zak, RN*

Colleen Zak, RN is the PKR Foundation, ARPKD FRIENDS group coordinator She also volunteers as a parent coordinator for the ARPKD Family Support Group, based out of The Children's Hospital, Philadelphia, Pennsylvania. Her active seven-year-old child with ARPKD was born without deformities and continues with normal kidney and liver function.

The very common disorder; polycystic kidney disease (PKD), presents a wide spectrum of symptomatology and severity, often resulting in end-stage renal failure. Misconceptions and antiquated nomenclature regarding PKD continue to exist in medical textbooks, at times resulting in less-than-accurate diagnoses, prognosis presentation, and treatment for those diagnosed prenatally and onward. This paper presents an explanation of what PKD is, how often it occurs, who gets it, and how it affects those with the autosomal dominant vs. autosomal recessive variant. Optimal diagnostic tools, prognostic views, and health maintenance suggestions are reviewed, along with a look at the direction of current PKD research.

Polycystic kidney disease (PKD) is the most common of all the life-threatening genetic diseases in the United States, affecting 500,000 to 600,000 Americans.[1] There are more persons in the U.S. with polycystic kidney disease than there are with all of the following diseases combined: cystic fibrosis, muscular dystrophy, hemophilia, Down's syndrome, and sickle cell anemia.

The National Institutes of Health (NIH) estimates that the genetically dominant form of PKD affects 1-in-400 to 1-in-1,000 persons in the U.S., making PKD more common than AIDS. It is twice as common as multiple sclerosis, 10 times more common than sickle cell anemia, and 20 times more common than cystic fibrosis or Huntington's disease.[2,3]

PKD affects people of all ages, races, ethnicities, and social status, men and women alike.[4]

Polycystic kidneys have an inborn defect that results in the formation of multiple cysts. The formation and filling of these cysts causes crowding of tissue and a reduction of function, and often failure, of the kidneys. While the normal kidney is about the size of a clenched human fist, a polycystic kidney may grow as large as a football.[5] It is the third leading cause of renal failure in the United States, costing taxpayers more than $1.5 billion every year in Medicare and Medicaid fees for dialysis, transplantation, and related treatments.[3]

There are two types of polycystic kidney disease: autosomal dominant polycystic kidney disease (ADPKD) and autosomal recessive polycystic kidney disease (ARPKD). Both are progressive and always affect both kidneys. They result from structural renal tubular changes in which fluid-filled cysts develop and crowd out healthy kidney tissue. The kidneys may enlarge massively and fill the abdominal cavity. Both types of polycystic kidney disorders can cause severe hypertension, both can affect the liver (in different ways), and both lead to renal failure in the majority of cases.

Autosomal Dominant Polycystic Kidney Disease

The NIH estimates that 1-in-400 to 1-in-1,000 persons in the U.S. have ADPKD, the dominantly inherited form of the disease. This is often called the "adult" form of PKD because its symptoms most often do not appear until the third of fourth decade of life. For the sake of accurate nomenclature, the term ADPKD or the "dominant form of PKD" is considered to be the more correct wording, since children may also have symptoms.

A person with ADPKD can remain relatively symptomless throughout life, with the diagnosis being made only at autopsy; it can, however, also be diagnosed prenatally using genetic linkage testing and/or ultrasound.[5]

Symptomatology can vary greatly among individuals and even within the same family.[6,7] One family member, for instance, might develop renal failure at an early age and another at a late age. There is as much variation of symptom manifestation and onset possibilities within families as there is between families.[7] Nonetheless, ultrasound signs of cyst development are present in most individuals by 18 years of age.[8]

There is a 50% chance of each child inheriting the PKD gene from an affected parent.[9] A new mutation can account for spontaneous PKD presentation in families with no history of the disease, but more often than not there is a long family history of kidney problems among extended family members as the gene is passed along from generation to generation.[10]

PKD is also a systemic disease that can potentially lead to problems of the liver, pancreas, heart, blood vessels, and intestines. Eventually, more than one-half of those individuals with ADPKD will develop kidney failure and will require dialysis and/or renal transplantation.[5,6]

The first symptom of ADPKD is often hypertension.[10] Other early symptoms that frequently occur are urinary tract infections and hematuria. There may also be discomfort or pain in the back, flank area, or abdomen due to increasing cyst size and the resultant pressure; a single cyst can grow as large as a grapefruit.[5] Other related problems can include proteinuria, kidney infections and stones, fatigue, diverticula of the colon, inguinal and abdominal hernias, brain and abdominal aneurysms, and cardiac valvular abnormalities.

In addition to the kidney, persons with PKD can also develop cysts in the ovary, testes, pancreas, spleen, central nervous system, and liver.[3] In women with severe cyst involvement, the enlarged abdomen can cause them to have the appearance of pregnancy.[11]

Severe polycystic liver disease (PLD) occurs most often in women and in individuals who are on dialysis or who have received a

kidney transplant. These individuals may present with intracystic bleeding, compression of the bile ducts, abnormal liver enzymes, and ascites on rare occasions.[11] Liver failure is very uncommon. Most individuals with PLD are actually asymptomatic, have normal liver function, and need no treatment.

There are at least three genetic mutations - known as PKD-1, PKD-2, and PKD-3 - that are responsible for polycystic kidney disease,[12] and the first two have been fully sequenced.

In 1994, researchers identified the first of the PKD genes, PKD-1, and a year later a "blueprint" was created to help determine how it functions.[10] The mutation for PKD-1 is located on chromosome 16p; that of PKD-2 is on chromosome 4q. It is now known that the PKD-1 gene accounts for 80% of the ADPKD cases and is associated with an earlier onset than is the PKD-2 gene.[13] In PKD-1 there is liver involvement 60% of the time, and cerebral aneurysms 10-15% of the time.[14] Clinical features associated with PKD-2 are colonic diverticula, aortic aneurysm, and mitral valve prolapse.[15]

Autosomal Recessive Polycystic Kidney Disease

Case Study: *A 33-year-old white female (the author), gravida I, para 0, presented for an obstetric ultrasound evaluation at 32 weeks gestation. This was to confirm the expected date of delivery (EDD) and fetal well-being because the fundus height appeared clinically small.*

Ultrasound revealed a low amniotic fluid volume. Several hours of ultrasound examination and consultation by technicians, perinatologists, and radiologists yielded a diagnosis of infantile polycystic kidney disease.

The prognosis was extremely grim. The parents were told that "these babies only live a few months to no more than one year after birth," and that "the infant would never be capable of living outside the hospital environment."

The mother obtained a second opinion, and the diagnosis of infantile polycystic kidney disease was confirmed. No hope for the infant's long-term survival was offered by the physician who did the antenatal evaluation. Due to the significant renal dysplasia, it was suggested that "the infant would not even survive until the EDD."

Soon afterward, the distraught parents spent hours inside a medial library researching the disease, looking for answers and hope. They found descriptions of Potter's facies and Potter's syndrome (facial deformities and postural deformities of the extremities), which can accompany the disease. They also learned that babies born with this disease can also develop severe pulmonary hypoplasia and/or kidney failure and death. Eventually, they came to accept the dire prognosis. They went home to await the birth of their child and planned for the baby's funeral.

The clinical presentation and diagnosis of autosomal recessive polycystic kidney disease (ARPKD) typically occurs early in life and has been called "prenatal PKD," "neonatal PKD," "infantile PKD," "childhood PKD," "adolescent PKD," and "juvenile PKD," depending on the age of diagnosis.

Early nomenclature was confusing. If the affected individual was an infant, then the label "infantile PKD" was given, but if a child was not diagnosed until he or she was an adolescent, then the diagnosis of "adolescent PKD" was given. This age-related, antiquated classification is still used in some reviews but should now be considered obsolete.[16]

In contrast to autosomal dominant PKD, there is almost never a family history of autosomal recessive PKD among the parents and grandparents. It is much less common than ADPKD, affecting only 1-in-10,000 of the general population.[17] Parents of ARPKD children never have the disease, yet each is a carrier of the defective gene. The probability of each offspring inheriting a defective gene and becoming a carrier is 50%.[17] ARPKD occurs when a child inherits a defective gene from each parent. If both parents are carriers, the probability of a fetus inheriting the disease is 25% for each pregnancy.

ARPKD frequently causes death in the newborn period. Oligohydramnios (decreased amniotic fluid) due to little or no urine output by the fetus' kidneys may result in Potter's facies or Potter's syndrome, the manifestations of the latter being deformities of the face and limbs due to compression in the uterus. ARPKD individuals are often born with pulmonary hypoplasia (small lungs) resulting from a low amniotic fluid level. As a result, 30–50% die at birth or soon after–not from kidney failure, but from complications of underdeveloped lungs.[18]

If the infant survives the newborn period, the chances of survival are good. For the child who survives the first year of life, the probability of not reaching end-stage renal failure is 67% at 15 years of age.[19]

The progression and severity of symptoms are highly variable within the ARPKD population. Nonetheless, 60% do develop hypertension by 15 years of age, which is often severe.[19] All individuals with ARPKD have some degree of congenital hepatic fibrosis, with scarring of the liver and malformation of the bile ducts.[20]

While the congenital hepatic fibrosis is always present, at least microscopically, it is possible, at least theoretically, that in some patients this may not cause clinically significant problems.

Characteristically, patients diagnosed early with ARPKD tend to follow a course of mild liver disease with significant kidney involvement, while those diagnosed later in childhood tend to have mild renal involvement with significant liver disease.[21] Symptomatology generally results from either the kidneys or liver; however, some individuals have significant symptoms from both organs.

For those in whom the disease chiefly involves the kidney, renal cysts continue to develop in the collecting tubules until normal functioning of the nephrons is greatly compromised. With enlarging kidneys, the collecting tubules are unable to concentrate water and eliminate body wastes. As a result, large volumes of urine are produced, but the body retains more waste products.

Of those who survive the newborn period, approximately one-third will need dialysis or transplantation by 10 years of age.[17] While end-stage renal failure can occur during the first years of life, it might not occur until the person is into his or her early adulthood.

Most serious congenital hepatic fibrosis involvement causes hepatomegaly and bile duct dilatation. Cholangitis can also occur.

Secondary organ involvement caused by the resistance of the free circulation of blood through the liver results in "backup pressure" into other parts of the abdomen. This is called portal hypertension and is most often manifested as splenomegaly, bleeding esophageal varices, and hemorrhoids. Massive splenomegaly is called hypersplenism, which can cause anemia, leukopenia, and thrombocytopenia. Further, the child's abdomen may enlarge grossly. For severe cases with portal hypertension, a liver transplantation may be needed.

In many medical facilities, treatment of kidney and liver complications is driven by the locally available technology/expertise. Hence, in some centers, children with end-stage renal failure and significant portal hypertension have been managed with combined kidney and liver transplantation.

Diagnosing PKD

Currently, in most centers, ultrasound is sensitive enough to detect PKD, but may not be sensitive enough to completely exclude the diagnosis.[8] A CT scan using contrast media and a MRI are the most accurate tests available. An IVP is the least sensitive test and is rarely done.[8]

It is recommended that children from autosomal dominant PKD families be tested only if they have symptoms or signs of polycystic kidneys, as the risks of diagnosis may outweigh the benefits in asymptomatic individuals. Potential detrimental effects include the "labeling" of the child and jeopardizing insurance coverage.

A specific gene-based diagnosis is not available at the present time for ADPKD. Linkage-based analysis is the best that can be offered for PKD-1 and PKD-2. This can only be done if at least one affected family member, as well as the individual at risk, are studied.[18]

A prenatal diagnosis for autosomal recessive PKD can begin to be made at 20 weeks gestation with a level II ultrasound. In addition, prenatal genetic testing can be performed through Lisa Guay-Woodford, MD, at the University of Alabama in Birmingham. (Testing centers abroad might also exist.) It is available to those families who have had at least one child diagnosed with ARPKD. Participation involves sending a blood sample from each immediate family member.

Health Maintenance for PKD:Interventions and Treatment

There is no cure for any form of polycystic kidney disease. Care is aimed at slowing down cyst enlargement and multiplication. Caffeine-containing products (e.g., coffee, tea, and chocolate) should be avoided, as caffeine has been associated with cyst enlargement. All non-steroidal anti-inflammatory agents must also be avoided because their intake can result in kidney failure. It is not wise for an individual with PKD to engage in body-contact sports because blunt abdominal trauma might rupture cysts and even the kidneys themselves.

Control of hypertension is of paramount importance. This needs to be done in order to slow down the deterioration of kidney function and to reduce the risk of intracranial aneurysm rupture (if present). Salt should be avoided in most cases; however, some patients with polycystic kidneys actually excrete larger than normal amounts of sodium in their urine, and in such individuals salt supplementation might be indicated. Hypertension is best treated with angiotensin-converting enzyme inhibitors.

Not all antibiotics can penetrate into infected kidney or liver cysts. The correct antibiotic, therefore, must be chosen carefully by a physician who must take into account the sensitivity of the organism as well as the drug's ability to enter a cyst and/or the biliary tree. The antibiotic ciprofloxacin is found biologically active in cystic kidneys, effectively reduces infections, hospital stays, and mortality.[22] With polycystic liver disease flare-ups, percutaneous drainage, alcohol sclerosis, or surgical interventions might be needed.[11]

It is important to find physicians who are specialists in the care of adults and children with PKD in order to best help manage these patients. Such physicians should be knowledgeable not only about kidney diseases, but also about the diagnosis, treatment, and prognosis of PKD. They should be supportive and not alarmist.

As PKD awareness and education increase among the medical community, ignorance and misconceptions will fade and will be replaced by improved PKD clinical treatment and understanding.

Insurance

Obtaining or maintaining health coverage oftentimes has been difficult or impossible for those who have polycystic kidney disease. PKD is considered an "uninsurable disease" due to the foreseeable medical problems individuals with PKD encounter because of their pre-existing condition. There are many accounts of people who were ultimately diagnosed with PKD putting off the needed medical examination–along with the possible diagnosis–to prevent being rejected by insurance companies. "Job-lock" and maintenance of secrecy regarding a medical history of PKD are not uncommon due to fear of not being afforded insurance coverage.

PKD Research and the Outlook for the Future

Polycystic kidney disease was once viewed pessimistically and thought of as hopelessly incurable. Now there is great optimism and anticipation for not only improved clinical treatment, but also for a PKD cure. As scientists learn more about the exact nature of the biochemical and genetic defects of PKD, treatment that interferes with the disease process will follow.

A cure–perhaps even gene replacement therapy–is foreseeable in the future. Dr. Francis Collins, the director of the U.S. Human Genome Project, discussed the impact of the PKD-1 and PKD-2 genetic discoveries during an interview conducted in 1994. He stressed that the identification of these genes are important first steps toward developing therapies or cures for PKD.[23]

Current research includes continued genetic research, development of medications to help retard cyst growth, dietary strategies, a PKD registry of families in order to analyze common family traits, and clinical research to identify better methods of treating PKD

and its complication.

Despite these strides, much ignorance and a relative lack of widespread public awareness still exist. PKD research continues to be under-funded compared to less prevalent diseases. Although PKD is 10 times more common than sickle cell anemia and 20 times more common than cystic fibrosis, annual federal research funding is $44 million and $60 million more for those diseases, respectively, than for PKD.[3] A treatment and eventual cure would save taxpayers billions of dollars in Medicare fees by eliminating the dialysis and renal transplantation now needed by this segment of the renal patient population.

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FOUR PKD MAGNET CENTERS of EXCELLENCE ESTABLISHED

The National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) of the National Institutes of Health (NIH), in collaboration with the PKR Foundation, has awarded grants totaling $20 million over the next five years for research centers on polycystic kidney disease (PKD). The PKR Foundation will supplement these grants through its END PKD campaign, while continuing to fund its ongoing PKD research initiatives.

"For the past 10 years, we've lobbied Congress and the NIDDK to establish PKD centers and to increase their overall funding of PKD research," Dan Larson, PKR Foundation president and CEO said. "We knew from observing how other diseases have made major research strides forward that establishing PKD Magnet Centers of Excellence would be critical to finding a treatment and an

eventual cure for the millions of children and adults living with PKD worldwide."

"We are pleased to award these grants to some of the most innovative and cutting-edge researchers in the field," said Dr. Josephine Briggs, director of NIDDK's Division of Kidney, Urologic and Hematologic Diseases. "Polycystic kidney disease is the hottest topic in renal science at the present time. This research is critical to developing treatments and hopefully a cure for this devastating disease."

Researchers at Yale University School of Medicine, Johns Hopkins University School of Medicine, Rainbow Babies & Children's Hospital at University Hospitals of Cleveland and Case Western Reserve University School of Medicine, and the University of Kansas Medical Center received a PKD center award. Each center will take a different approach in their efforts to develop treatments and a cure for PKD. Three will focus on the dominant form of the disease (ADPKD) and one will specialize in recessive PKD (ARPKD). However, research discoveries in one form of PKD often have overlapping and positive effects on research advances in the other form.

YALE UNIVERSITY SCHOOL OF MEDICINE

The NIDDK awarded the Yale University School of Medicine in New Haven, Connecticut, $1.15 million for the first year of research. The goal of the Yale Center for Polycystic Kidney Disease Research is to elucidate the mechanisms by which defects in the polycystin genes cause autosomal dominant PKD and to understand the factors that modify the expression of this disease in affected individuals. Stefan Somlo, MD, is the principal investigator, working with Peter Igarashi, MD; Michael Caplan, MD, PhD; Peter Aronson, MD; Barbara Ehrlich, PhD; Guanqing Wu, MD, PhD; and Michael Kashgarian, MD.

THE JOHNS HOPKINS UNIVERSITY SCHOOL OF MEDICINE

The NIDDK also awarded $844,396 for the first year of the PKD center at The Johns Hopkins University School of Medicine in Blatimore, Maryland. This grant will focus on the two genes linked to ADPKD, PKD1 and PKD2, to determine their genetic pathways. Gregory G. Germino, MD, will provide the lead on this research.

RAINBOW BABIES & CHILDREN'S HOSPITAL

Rainbow Babies & Children's Hospital at University Hospitals of Cleveland and Case Western Reserve University School of Medicine received a first-year award of $775,000 for its PKD center. **Research for this collaborative effort will focus on preventives and cures for autosomal recessive polycystic kidney disease (ARPKD), looking at pharmacological and genetic therapy of ARPKD.** Ellis D. Avner, MD, is the primary investigator for this research.

UNIVERSITY OF KANSAS MEDICAL CENTER

The NIDDK awarded the University of Kansas Medical Center in Kansas City, Kansas, $774,999 for its first year. This grant will be used to help researchers understand the functions of polycystin and to develop novel treatments to slow or arrest the progression of polycystic kidney disorders. Jared J. Grantham, MD, is the leading investigator for this research.

More than 840 disease-specific research centers exist around the United States. Prior to these center awards, none of the 840 disease-specific research centers focused on PKD. "With the establishment of these centers, the best scientific minds now believe we are within a five-to-10 year window of developing a treatment for PKD," Larson said.

"It's a relief to know this disease is finally getting recognition, and we're moving more quickly toward finding a cure. It's very exciting," said Kevin Gilbride, offensive coordinator for the Pittsburgh Steelers of the National Football League (NFL). Gilbride's father died from PKD, and three siblings and many relatives have the disease.

"Establishing these centers will take research to a much higher level," said Drew Netter, PKR Foundation vice chairman. "We now have an opportunity to focus research efforts, and this should speed the process of finding a treatment and ultimately a cure." Netter also has a personal investment in the centers. Several members of his family have PKD, including himself.

The PKR Foundation has been the catalyst in drawing attention and financial support to PKD. NIDDK's public support for PKD research increased more than 550 percent during the past 10 years: $1.8 million in 1989 to $11.7 million in 1999. The PKR Foundation's private research support over the 10-year period from 1988 to 1998 grew by 1,233 percent; from less than $75,000 in 1988 to more than $1 million in 1998. Despite these advances, research funding for PKD lags far behind other less prevalent genetic diseases such as cystic fibrosis. «

—Reprinted from the PKR Progress; Polycystic Kidney Research Foundation newsletter; Winter 1999-2000; Volume 14, Issue 5

POST-TRANSPLANT ISSUES: Missing a Dose: Does It Matter?

Did you take your medicine today?

That question makes many people cringe. Still, it's an important one to consider.
The research community is beginning to pay a lot of attention to how well the general public follows instructions. In scientific terms the problem is labeled one of "compliance."
U.S. health care professionals conservatively estimate that half of the 1.8 billion prescriptions prepared each year are not taken correctly. As for transplant patients, one study conducted at the Hartford Transplant Center in Connecticut found that 15 percent of the hospital's organ recipients were not taking their immunosuppressive medication as directed or keeping office and laboratory appointments.

WHAT ARE THE RISKS?

The danger in noncompliance is that it can result in rejection episodes, organ loss, or even death.
In a study conducted at the University of Texas Medical School in Houston, noncompliance was the second most common cause of organ loss after two years, ranking close behind chronic rejection.
"There's always the temptation to play around with medications, especially when you're feeling good," says Kathleen Lake, transplant program director for Abbott Northwestern Hospital and the Minneapolis Heart Institute Foundation.

WHY AND WHO?

Besides the disappearance of symptoms, other reasons patients don't take their medication correctly include forgetfulness, unclear directions, avoiding side effects, saving money, and lack of concern for consequences.
Women tend to follow doctors' orders more carefully than men. And young people tend to be the most negligent about taking their medications, often because of their sense of invincibility. "They have the most hope for a cure," says Joyce Cramer, a frequent author and lecturer on patient compliance. "It takes a little gray hair to know that you really have to buckle up your seatbelt and button up your coat."
Young people also have a difficult time handling the side effects common with transplant medications, Lake points out. Acne, hirsutism (excess hair growth), and weight gain can be devastating to a teenager. "The key is, before you do anything on your own, talk with your transplant team," Lake says.
As life becomes more routine after transplantation, Cramer points out it's easier to forget to take your medication as directed. This is especially true *because* you can miss a dose without immediate problems or rejection. But the danger of noncompliance is real. In the Hartford study, 30 percent of the noncompliant patients experienced organ rejection or died.

PREDICTING RISKS

Doctors and researchers are looking for ways to predict who will be noncompliant. It doesn't seem to be related to intelligence, economic level, or personality, according to Cramer. However, compulsive patients tend to be the most compliant because they are the most organized. Cramer calls it the "clean closet, clean desk theory."
Absentmindedness and memory loss are also problems for some people taking transplant medications. Lake says the best advice is to make a commitment to be as faithful as you can to your treatment plan. And if you accidentally skip a dose, call your transplant coordinator. She or he can help you get back on the best schedule.*«*

Sources: Kathleen Lake, Pharm. D., is the transplant program director for the Minneapolis Heart Institute Foundation and Abbott Northwestern Hospital in Minneapolis.

Joyce Cramer is project director for Health Services Research at the Veterans Administration Medical Center in West Haven, Connecticut, and an associate in research at the Yale University School of Medicine in New Haven, Connecticut.

—Reprinted with permission from Encore, a quarterly magazine for individuals who have undergone transplantation; Summer 1993 Issue. For information, please write to: Chronimed Pharmacy, 13911 Ridgedale Drive, Minnetonka, Minnesota, 55305

AN INTERVIEW WITH ELLIS D. AVNER, M.D.…

By Colleen Zak

ELLIS D. AVNER, M.D. is Pediatric Nephrologist, Chairman & Professor of Pediatrics at Case Western Reserve University and Director of Pediatrics at Rainbow Babies and Children's Hospital at University Hospitals of Cleveland, Ohio. Dr. Avner holds many honors and awards and serves on numerous elected national and international offices. He has authored over 145 professional articles

since 1988 and is the principal investigator at the **PKD Magnet Center of Excellence**, at Rainbow Babies & Children's Hospital at University Hospitals of Cleveland and Case Western Reserve University School of Medicine.

CZ: First, let me thank you for agreeing to do this interview Dr. Avner. ARPKD families and the PKD community as a whole are very enthusiastic and excited about your work with the promising drug, novel tyrosine kinase inhibitor (EKI-785) on the BPK mouse, a murine model of ARPKD. It provides much hope for a PKD treatment.

CZ: What kind of results have you had with EKI-785 on the BPK mice? Who may benefit from the drug?

Dr. Avner: In previous studies, we identified specific abnormalities of a growth factor receptor in cystic kidneys. We then demonstrated, in the laboratory dish, that reversing these abnormalities with a newly developed drug (EKI-785; Wyeth Ayerst) was possible. Our most recent studies were designed to determine whether treating animals with ARPKD with this drug would decrease cyst formation, improve kidney function, improve survival, and have an effect on liver abnormalities. Our results were exciting — EKI-785 dramatically reduced cyst formation, preserved kidney function, prolonged survival, and prevented progression of liver abnormalities without significant side effects. We are now working with our colleagues at Wyeth Ayerst to do an exhaustive toxicology profile in animals of all ages to determine the overall safety profile of EKI-785 and newly developed 2nd generation compounds which will be effective when given orally. This new approach, once we have determined safety profiles, has potential for all patients with ARPKD. For those with enlarged kidneys who have not had significant deterioration in kidney function, it might prevent disease progression. For those with advanced kidney disease, it may stabilize declining renal function and delay the need for end stage renal disease therapy. For all patients, it may decrease the progression of liver abnormalities and fibrosis and delay or prevent portal hypertension and its complications. We also have some preliminary information to suggest that a similar approach may have therapeutic benefits for children (and adults) with ADPKD.

CZ: What are the long-term and short-term goals of the PKD Magnet Center of Excellence at Rainbow Babies & Children's Hospital at University of Cleveland and Case Western Reserve University School of Medicine? What to you hope to accomplish there?

Dr. Avner: We are proud of our designation as a PKD Center of Excellence. Our Rainbow Center for Childhood PKD has both basic science and clinical components. Our basic science program, which includes scientific leaders from our rich academic program at the University Hospital Research Institute and Case Western Reserve University, is focused on identifying basic abnormalities at the molecular and cellular level which make cystic tissue function differently than normal tissue. Each of these abnormalities represents a potential target for the development of specific therapies to treat PKD. In addition to the studies noted above, we have many new approaches to treatment under evaluation in experimental models. Our clinical program, which includes a multidisciplinary team of physicians (pediatric nephrologists, urologists, gastro-enterologists, geneticists, radiologists), nurse specialists, psychologists, and nutritionists is focused on the comprehensive evaluation of children with PKD, and the development of clinical management plans for families and referral physicians. This will also be the coordinating arm of our future clinical studies. Our PKD Center is supported by the National Institutes of Health, the PKR Foundation, Wyeth-Ayerst, and University Hospitals of Cleveland.

CZ: What time frame are we looking at with the clinical drug studies, from start to finish? When can we expect clinical trials to begin, how long will they last, and if the results are successful, when could they be available and on the market? Where will these clinical drug trials/studies take place and how will patient selection for participation in the clinical study be made? What criteria for participation will be looked at, for example, will the patient's creatinine level or clinical signs of portal hypertension have any bearing on participation?

Dr. Avner: We are collaborating with Wyeth-Ayerst in the development of future clinical trials. Drugs like EKI-785 are being predominantly developed as anti-cancer agents. This is because some forms of cancer demonstrate growth factor receptor abnormalities similar to those we and others have described in PKD. The timing of clinical trials will depend entirely on the results of the toxicology studies currently in progress, and preliminary safety information derived from the treatment of adult cancer patients with these agents. We hope to organize a pilot study for patients with PKD which would start in 12 to 18 months. The key is determination of drug safety. We believe that we have an approach which will be effective--we must be sure that it will be safe. This is of particular concern in considering therapy for children. If a pilot study demonstates safety and efficacy, large-scale clinical trials could begin within 24-36 months. Pilot clinical studies will be coordinated and largely take place at the Rainbow Center in Cleveland. Large scale clinical studies will be coordinated through the Rainbow Center and involve national recruitment and participation of at least 5-10 major academic pediatric medical centers. It is too early to determine the exact eligibility criteria for future clinical studies at this time. This is a topic currently under active discussion and evaluation.

CZ: For the 1997 Fall issue of the "PKR Progress" newsletter you wrote an article titled, "Of Mice and Men", discussing your earlier research work. What have you learned since that time, what do we now know about ARPKD and Congenital Hepatic Fibrosis that we didn't understand then?

Dr. Avner: A lot!! This has been reviewed in my presentation at the 1998 PKR Foundation Annual Conference, Dr. Chapman's presentation at the 1999 PKR Foundation Annual Conference, and the regular research updates provided by many PKD scientists in the PKR Foundation newsletter and on the website. It is really impossible to give a snapshot or overview in the concise format of this interview.

CZ: The ARPKD gene is located on Chromosome 6, but is not fully sequenced (mapped out) at this time. How much longer do you believe this will take, who is doing this work and where?

Dr. Avner: The human ARPKD gene has been mapped to an increasingly small region on human chromosome 6, but has not yet been identified. I am hopeful (but far from certain) that the gene will be identified in the next 6-12 months. This work is predominantly being done by an International Consortium which includes scientific teams headed by Dr. Gregory Germino at John's Hopkins University; Dr. Lisa Guay-Woodford at University of Alabama, Birmingham; Dr. Stefan Somlo at Yale University; Dr.Klaus Zerres at the University of Aachen, Germany; and my team at Rainbow Babies & Children's Hospital at University Hospitals of Cleveland and Case Western Reserve University.

CZ: The media has given a lot of attention recently to the registering of gene patents, ownership lasting 17 years. Who will own the ARPKD gene once it is fully sequenced, if anyone?

Dr. Avner: One of the reasons we developed the International Consortium to identify the ARPKD gene was to ensure maximal scientific cooperation and dissemination of scientific information as rapidly as possible. I do not believe anyone will, or should, hold a "patent" on any disease gene, including the ARPKD gene.

CZ: On a personal note, could you explain how you became interested in doing PKD research?

Dr. Avner: I trained in pediatrics, and then pediatric nephrology at the Boston Children's Hospital. During my fellowship in pediatric nephrology from 1978-1980, I cared for a number of patients with ARPKD. Working with these patients and their families had an immediate impact on my career--I decided to focus my scientific training and career on identifying the basic abnormalities underlying cyst formation and progressive enlargement in PKD with the hope of someday identifying specific targets for therapeutic intervention. Now, some 20 years later, we may actually be on the threshold of bringing this to fruition.

CZ: An ARPKD treatment seems possible, what is the likelihood of an ARPKD cure?

Dr. Avner: Successful disease treatments really blend into "cures"—for ARPKD, successful treatment will be measured by normalization of life with maximal rehabilitation and minimal complications. Ultimately, cure for any genetic disease like ARPKD will involve advanced genetic engineering and gene therapy. We do not yet have the appropriate knowledge or technology to accomplish this goal now or in the immediate future. However, given the continued explosive developments in molecular genetics and functional genomics, the promise of "cures" is moving farther from science fiction and closer to science. «

FUND-RAISING FOR ARPKD/CHF….

FAMILIES MAKE A DIFFERENCE...

All across the country, families are reaching out to one another for information and moral support in battling Autosomal Recessive Polycystic Kidney Disease. Out of love for their affected children, the following families have worked diligently to focus public attention on the disease and the need to raise funds for research into a treatment and cure for ARPKD.

FOR THE LOVE OF LUCY . . . *The Gombas'*

The Iron Man Triathlon is one of the most grueling tests of human endurance ever devised. Competitors first swim 2.4 miles, then bike 112 miles, and finally run a 26.2 mile marathon. Some do it for the challenge, some for the competition, still others for the glory, but Jim Gombas did it for his 2-1/2 year old daughter Lucy! After training for more than a year, Jim ran the race on August 15,1999 at Lake Placid, New York, as a platform to raise money and awareness for ARPKD. It was his hope that by competing in the Iron Man event he would bring attention, and much-needed funds, to the little known disease that affects his daughter and so many other children. Some day, when Lucy is older, her parents know she will be proud of her dad for making such a commitment because of his incredible love for her. Jim finished the grueling race in 14 hours and 40 minutes and raised approximately $22,000 for ARPKD research.

ELIZABETH'S FUN-RIDE . . . *The Adams*

Elizabeth Wingfield Adams was born on May 30, 1991, and diagnosed with ARPKD two years later after falling out of bed and bursting cysts in her kidneys. Ultrasounds revealed her kidneys were twice the size of normal kidneys in a child her age, due to numerous cysts.

The initial diagnosis was heart-breaking to her parents, Julia and Rick, and to her older sister Berkeley. Despair was replaced by empowerment after they read an article in the "PKR Progress" written by Colleen Zak. In the article she advocated "....starting a support group, organize a fund-raiser or educate others...get involved". So they did.

Three years ago they started "The Friends of Elizabeth Fun-Ride". They organized a fund-raiser where they invited friends, co-workers and family to participate in a bike ride. The 16-mile ride along the beautiful American River in their hometown of Fair Oaks, California, included refreshments at the nine-mile point and a party at their residence after the completion of the ride. Each year approximately 100 riders participate and another 50 join the festivities. The youngest rider was 5 years old and the oldest riders were over 60 years old. To date, the bike rides have raised approximately $15,000. The Adams distributed flyers educating people about ARPKD and their daughter Elizabeth at every ride.

"In addition to generating funds, the bike rides are a fun community-building activity that has brought Elizabeth and our entire family much joy and support," said Rick Adams. Currently, Elizabeth's kidney function is unaffected and she enjoys excellent health. She is actively involved in soccer, horseback riding, roller blading and third grade.

"TWO STATES, TWO EVENTS, ONE LITTLE BOY'S FAMILY". . .*The Kopan's & The Simpson's:*

Two-year-old Drew Kopan's family has been busy raising money for ARPKD research. Only one month apart, his parents and grandparents both organized a fund-raising event that was a variation on the same theme.

"Ride For Research". . . On October 2, 1999, Kris and Brian Kopan hosted the "Ride For Research" at Spring Gulch Resort Campground in New Holland, Pennsylvania. The setting of their family business provided a biker's delight with rolling hills, beautiful farmland and perfect cycling weather. After the bike ride, participants were invited to an outdoor picnic that was stocked by donations from many local businesses. The Kopan's raised $7,000 for ARPKD research and express many thanks to the thirty-four sponsors from Central Pennsylvania that made the event such a success.

"Run For Research". . . One month later, Manhasset, New York, was the site for the "Run For Research" which was organized by Joy and Bill Simpson, Drew's grandparents. The event was a 5-mile run/walk that brought together more than 300 participants from the New York area. Prizes such as a TV, mountain bike, and gift certificates to local restaurants were raffled off at the end of the race and Drew's mother and uncle each placed 3rd in their prospective age groups. It was a great family event and more than $30,000 was raised for ARPKD research! Many thanks to the Simpson's for all their hard work! «

FUND-RAISING NO-SHOW DINNER!!!

The ARPKD Friends Group would like to do a fund-raising event called a **"No-Show"** dinner.

A **"No-Show"** dinner works like this….. Invitations are mailed to family and friends. Though instead of actually attending a fund-raising dinner, this is a faux dinner, making it easy for those invited (no baby-sitters or preparation is needed). Those invited are asked to send in their fee (a donation). This is followed by a drawing, via the ticket number which is sent in with the donation for the prize drawings. "No-Show" dinners are very popular among the PKD Friends Groups as they have been successful fund-raising events. We are certain this can be successful with appropriate prizes. All proceeds will benefit ARPKD.

We are in need of prizes for the drawing. Suggestions have been a vacation destination or travel arrangements, plane fare(s), a new appliance or electrical equipment such as a T.V., computer, free Internet access, ect. Please keep in mind that our group is geographically spread out, so we are looking for donations/prizes shippable, transferable, or interesting enough to support travel.

Please, if anyone can help or has any suggestions for prizes, please contact me at **Btlhall@aol.com.**

For those wishing to donate directly to the PKR Foundation's ARPKD Friends Account, please make checks payable to ARPKD Friends-PKR Foundation and send to the ARPKD Friends Group Treasurers, Brian & Kris Kopan, 487 Lynch Rd., New Holland, P.A., 17557. Funds in this account are used to cover expenses our group incurs, such as newsletter postage and fund-raising expenses. At the end of the year, money left over is transferred to the Restricted ARPKD Research Account, which is used for ARPKD/CHF research. Any corporate matching programs would also be greatly appreciated. Research can not occur without funding!

Thanks for your assistance,

Beth Parmington Hall at Btlhall@aol.com «

"Study to block rejection of transplants seen effective"

Associated Press June 1, 1999

A temporary treatment to block organ rejection has remained effective for up to a year so far in monkeys that got transplanted kidneys, researchers report. Scientist hope the experimental treatment will one day free some transplant patients from having to take anti-rejection drugs for the rest of their lives. The standard drugs suppress the immune system and leave patients vulnerable to infections and tumors. Researchers are now planning studies for the experimental treatment in people, said Dr. Allan D. Kirk of the Naval Medical Research Center in Bethesda, MD. He and co-authors describe the monkey study in the June issue of the journal Nature Medicine.

Two years ago, Kirk and colleagues reported on a similar treatment that staved off rejection for more than nine months in monkeys. The new treatment includes only one of the two substances administered in the prior work. The goal is to teach the immune system to accept the transplanted tissue rather than attack it. To do that, researchers injected the monkeys with a protein to prevent certain blood cells from delivering a danger signal to other cells, an initial event in rejection. The protein is called hu5C8. The researchers gave it to nine monkeys that morning of the kidney transplant, just after the surgery, about once a week for four weeks after that, and finally once a month for five months. Eight of the nine treated monkeys remain alive and well with no organ rejection. Two have lived about a year so far since the end of treatment, and another more than six months. The ninth monkey died from an unrelated cause. In a commentary accompanying the article, immune-system expert Polly Matzinger of the National Institutes of Health said a 1996 study had shown the approach works in mice. But transplant researchers largely overlooked that report, she said. "Well", she wrote, "it is time to pay attention!".«

QUESTIONS/ANSWERS

Fall /Winter 1996; issue 2

Q. After having given birth to an ARPKD child, how do I handle society's reaction to future pregnancies and my own quilt feelings? L.S., MA

A. The question can be read in two ways. What is a parent to say when someone wonders why they would try having another child with a terrible disease. Or, what is a parent to say when someone asks why they would have a child that might require a great deal of society's resources in terms of health care costs and social services.

I think the answer to the first question is for the person to say that no one wants to have a child with a devastating disease. But, anyone who considers having a child, faces some risk of having a child with medical problems and disabilities. It is true that some couples will know more about the risks they face then will others because of what is now known about the genetic bases of some diseases. But knowing a risk does not in itself mean that the risk cannot reasonably be pursued. This is especially so when it comes to having a child. When the motive for having the child is to love, cherish and support the child, when a couple understands that it will do what must be done to cope with problems, risks and even life-threatening ailments that a child might have, then there is no sound reason for impugning or disparaging the decision to try to have a second child when one has already been born with a medical problem that has a genetic component.

The other question admits of a simpler and shorter answer. In our society we do not ask anyone to reproduce or parent with an eye on the bottom line. Older parents or those who might die from a disease still have the right to parent.

Responsible parents should do what they can to insure their child's health and social needs will be met. A humane and moral society must be willing to do the rest.[2]

Q. What are some of the important indicators and laboratory values that the Pediatric Nephrologist is looking at when treating ARPKD/CHF and what do they mean?

A. Weight and height: The doctor follows a growth chart to see if your child is growing at a normal rate or needs help.

Blood pressure: The pressure of blood as it flows through the arteries. Important to keep in normal range for age of child to help maintain kidney function.

Creatinine, BUN, and Uric Acid: A measure of waste products in the blood from protein intake and tissue breakdown. A higher number means less kidney function.

Sodium, Potassium, Chloride, and CO_2 (electrolytes): Can cause acidosis affecting bones, blood formation and breathing. Must be kept normal to allow normal growth.

Calcium and Phosphorus: Affects bones. Low calcium and high phosphorus means bones are becoming weak (demineralization).

Cholesterol: Related to heredity and fat intake and can be high in children.

Protein and Albumin: Reflects general state of nutrition. Low albumin is abnormal.

Platelets: Help with the clotting of the blood.

White Blood Count (WBC): Blood cells that destroy bacteria and other foreign matter. High numbers can mean infection in the body.
Red Blood Count, Hematocrit and Hemoglobin (RBC,HCT, and HGB): Indicate anemia when numbers are low.[3]
(Watch for more Important Indicators and Laboratory Values explanations in the next Newsletter.)

Q. A lot of children with ARPKD have blood sodium levels which chronically run low. Some are placed on Na Chloride and some are not. What are the pro's and con's of supplementation and which children fare better? Also, with children who are not supplemented with sodium, should their diet include sodium or have sodium restrictions because of their hypertension?
A. Unfortunately, the serum sodium level can not be used as a simple guide to determine which children may or may not require sodium supplementation. The serum sodium level may be low if there is an actual sodium deficit, or, more commonly, if there is retention of free water. Sodium supplementation therapy is indeed appropriate for children who have true sodium deficits. However, such therapy can be dangerous in children who have relative water retention, and may worsen or cause hypertension in this setting. The amount of sodium which should be present in the diet of children with ARPKD depends on their total salt balance, as well as, the presence or absence of hypertension. Sodium restriction is an important part of antihypertensive therapy in ARPKD and other hypertensive kidney diseases.[1]

Q. My son is thirsty all the time and drinks a lot of fluids. Is this a result of ARPKD?
A. This is quite common in children with ARPKD, because their kidney tubules cannot normally conserve water and concentrate their urine. This is a potential problem during periods of dehydration (like vomiting or diarrhea), when fluid intake is very important. Children with ARPKD who are unable to concentrate their urine are at a relatively greater risk of dehydration if they do not get adequate fluids during these types of stresses.[1]

Q. Is there anything that can be done to slow the progression of ARPKD?
A. There is no known dietary manipulation which will decrease cyst growth or slow disease progression. Control of hypertension and avoidance of secondary insults to the kidney (for example, medications which can cause kidney damage) are the two key therapies which should be utilized. Experimental studies offer the hope that certain "cyst reducing" drugs may be available for initial clinical trials within the next few
years. This is an exciting and promising area of current research.[1]

[1]Ellis D. Avner, M.D., Professor of Pediatrics
[2]Editor's note: Credit and thanks to Dr. Arthur Caplan, Director, Center for Bioethics at University of Pennsylvania Health System for answering this difficult question.
[3]Excerpted from "Pediatric Worksheet for Kidney Clinic", a publication developed by Renal Consumers in cooperation with the National Kidney Foundation of Michigan.

Spring/Summer 1997 Issue No. 3

Q. What are the lab values you look at when considering treatment for congenital hepatic fibrosis?
A. Congenital hepatic fibrosis rarely causes synthetic abnormalities in the liver. The major problem is portal hypertension. However, we do follow liver enzymes such a AST, ALT and LDH along with bilirubin and albumin. The two treatments available at this time for congenital hepatic fibrosis include a portal systemic shunt which means rerouting the blood vessels around the liver so there is not relative obstruction to venous flow to the liver. However, this surgery does involve significant potential problems including deterioration of the liver function and therefore is rarely done until sequestration of platelets and red cells is significant. That is, if platelet counts fall consistently below 20,000 or white blood cell count falls lower than an ANC or absolute neutrophil count of 1000. People do consider liver transplantation as a possible therapy for congenital hepatic fibrosis, especially if there is significant life threatening bleeding from varices. There have been a number of medications to inhibit fibrosis used in animals, but none of these are directly applicable to humans at this time.

Q. Can you provide numbers of autosomal recessive PKD children affected by congenital hepatic fibrosis and the severity, treatment and success rates of treatment?
A.Theoretically, all patients with autosomal recessive PKD have some degree of congenital hepatic fibrosis. As was stated in the previous answer, the only two therapies at this point that are considered are portal systemic shunting or liver transplant. Since the shunts have changed numerous times over the last year as far as their anatomic rearrangement, each particular shunt would have to be dealt with separately and also it would be best to discuss this with the surgeons that do the shunts in order to get absolute statistics. As far as liver transplant is concerned, there is approximately a 10-15% mortality associated with liver transplant because if it is rejected or has technical failure, there is no back-up. As of now, the general literature quotes an 80% five year survival for liver transplant.

Q. What lab indicators do you use and at what levels do you consider dialysis and transplant?
A. We utilize electrolytes, creatinine and blood urea nitrogen to look at the lab test. In general, children need dialysis when their

creatinine clearance reaches approximately 10 cc per minute with normal being 120 cc per minute. However, we use other parameters to decide about transplant including the growth and development of the child. There are some children that have creatinine clearances calculated at somewhere between 40 and 60 cc per minute who maintain that renal function for many, many years.

Q. Why is the treatment of acidosis so varied from patient to patient and what are the considerations for different treatments?
A. A number of children who have kidney disease have decreased ability to generate bicarbonate in their blood by secreting acid in their kidneys. Because of that, extra buffer or bicarbonate must be added to their intake. This can be done a number of different ways, the most common being given sodium citrate as Bicitra or sodium bicarbonate either tablets or liquid. You can also use calcium carbonate which ads buffer via the carbonate. There are also a number of drinks that have some carbonate or citrate in them that can be converted to bicarbonate in the blood. In general, children that have acidosis need 3 to 4 mEq of bicarbonate equivalent per kilogram of body weight to adjust the bicarbonate. Basically we utilize all these different forms of buffer supplementation in order to help compliance.

Q. How low can white blood cell count and hematocrit go before we intervene?
A. In general a white blood cell count of 3000 to 4000 with an absolute neutrophil count of 1000 is considered adequate. A platelet count above 20,000 is considered to be adequate for clotting unless there is significant bleeding going on. Nowadays, we usually try to keep the hematocrit above 30 with the use of iron supplementation and epoetin.

Q. What is ferritin?
AFerritin is a protein and iron complex. It is the form in which iron is stored in the blood and its level is an indicator of iron stores. However, it also can be elevated in acute inflammation as it is an acute phase reactant.

Q. What is red cell morphology and specifically what was rouleaux formation?
A. Red cell morphology is a measurement of the shape of the cells. The rouleaux formation means that the red cells are stacked in a coin like distribution. It is a very nonspecific finding and can be a normal variant or could indicate increased protein in the blood.

Q. Could bicycling raise the CO_2 level in the blood?
A. Bicycling should not increase CO_2.

Answers to questions provided by: Aileen B. Sedman, M.D. Kelch Professor of Pediatric Nephrology, University of Michigan, Ann Arbor, Michigan

Fall/Winter 1997, issue 4

Transplant Q & A

Jeff Punch, M.D.

Cancer risks post-transplant
Q. Our son has been on FK506 for eight months now and is doing fine. However, recently we have met several families whose children have been diagnosed with different forms of cancer apparently related to the immunosuppressants. One child was six years post transplant, another only 1 ½ years post transplant. What are the statistics on developing cancer after transplant and are there early warning signs parents should watch for?
A. There are certain forms of cancer that seem to occur more commonly in transplant patients than in the rest of the population. These cancers are rare in normal people but are merely uncommon in transplant recipients.
It is believed that the immune system normally prevents the development of cancers by identifying cells that are growing abnormally and killing them, thus "nipping the cancer in the bud." Immunosuppression can affect the body's ability to perform this function. This is felt to be the reason why we see an increased incidence of cancers in transplant patients.
Overall, the incidence of cancer in all transplant patients is about 6%. Dr. Israel Penn at the University of Cincinnati, Ohio, keeps a registry of all transplant recipients who get cancer. His data shows that there is no increased risk of lung, prostate, colon, breast, or cervical cancer in transplant recipients. However, there is an increased risk of some tumors, the most important ones are skin cancers and lymphoma.
Skin cancer is the most common of these tumors and is often completely curable. Skin cancer is associated with sun exposure, therefore patients are cautioned against excess sun exposure. Lotions that protect against ultraviolet radiation are strongly recommended (at least SPF30). Any unusual spot or growth on the skin should be brought to the attention of either the transplant team

or a dermatologist. Children should be examined regularly for the early warning sign of skin cancer: a new mole or a mole that suddenly changes color, size, or begins to bleed. These tumors can usually be cured by simple excision.
Some types of skin cancer may spread more easily to the rest of the body. These forms (squamous cell cancer and melanoma) can be deadly.
Lymphoma is a cancer of the immune cells called lymphocytes. The type of lymphoma associated with immune suppression is called B cell lymphoma because it is made up of the B cells (cells that ordinarily produce antibodies). These cells divide uncontrollably in lymphoma.
It is believed that Epstein-Barr viral infection may cause the uncontrolled proliferation of these cells since it is almost always present in transplant patients with lymphoma. Epstein-Barr is a virus from the herpes family that is also called mononucleosis. It causes a cold-like illness with fever, lethargy, and swollen lymph glands. Severe Epstein-Barr infection can cause "LPD" or lympho-proliferative disease. This disease is thought to be the precursor to lymphoma.
Unusual swelling in the neck, groin or arm pits is a possible sign of LPD and should be brought to the attention of the transplant team if noticed. Persistent, unexplained fever is another cause for concern.
The treatment for LPD is a combination of antiviral medications like acyclovir and ganciclovir, plus reduction of immunosuppression.

Dr. Punch is a transplant surgeon and assistant professor of surgery at the University of Michigan. He is a member of the Scientific Advisory Board of *CLASS*

Fall /Winter 1997, issue 4

Q. Does Inderol seem to have any effect on reducing portal hypertension?
A. Yes. Inderol does decrease portal hypertension. The disadvantages are from the other "beta blocker" effects it has. It slows the heart rate down and may make an individual feel sluggish. It should not be used if a bleed has already occurred, for the "beta blocker effects" would interfere with the body's compensatory mechanism of increasing the heart rate should another bleed occur. This interference with the body's defense against shock (increased heart rate) could have catastrophic effects.

Q. Are there any advantages to periodically doing an endoscopy to check the status of esophageal varices or do you feel that the process of scooping can aggravate the varices?
A. Absolutely not. A person is only scoped if there was a bleed and we need to know why. If scoped only to inspect and mid-range varices are present, sclerotherapy may be performed and complications from the procedure may occur. It is not worth checking on varices that may not rupture for another 10 years or never.

Q. Do you feel that sclerosis of varices is beneficial? What about banding?
A. Absolutely. Sclerotherapy is effective if varix (single varice) have started to bleed and this can be done through an endoscope. Rubber banding around a varix clots off the blood supply and allows the varice to fall off. It seems effective, though long-term effects are not certain.

Q. At what stage would you consider putting in a shunt? What are the advantages and disadvantages of shunting?
A. When there has been esophageal bleeding that can't be controlled with therapy. If shunting works, it may work forever, though plaement involves major surgery. If a shunt does not work properly, another shunt placement may be attempted. One of the disadvantages of shunting, though it is very rare, is a person may be neurologially impaired from decreased mental alertness due to decreased blood flow to the liver, allowing chemicals like amonia to get to the brain.

Q. Does portal hypertension in any way increase the risk of cerebral aneurysm?
A. No, only with systemic hypertension.

Q. Once portal hypertension exists, does the liver function deteriorate quite quickly?
A. Absolutely not. Function is fine with excretion, production, and regulatory control.

Q. Does Congenital Hepatic Fibrosis usually end up resulting in a liver transplant?
A. Almost never.

Q. Is there any treatment for the enlargement of the spleen besides resorting to its removal?
A. If removed, the portal hypertension is unchanged except that now the pressure will go elsewhere. It is best to get the pressure off the spleen, perhaps with a shunt. For those with big, enlarged spleens a "spleen protector" may be worn which is like a bullet proof shield. It is not prove, but works well with active sports.

Q. What research is being done on Congenital Hepatic Fibrosis?
A. Not very much.

Spring/Summer 1998, issue 5

Q. Why does my child urinate so much?
A. The cysts in the kidneys of children with ARPKD are in the collecting ducts. That portion of the kidney nephron (the functioning unit of the kidney) is responsible for the fine regulation of urine volume. If a normal person does not drink, the body recognizes that water should be conserved. It sends a message to the pituitary gland in the head to make and secrete "antidiuretic hormone", otherwise known as "vasopressin". That hormone goes through the blood to the collecting duct and "tells" the collecting duct to hang on to fluid. So the urine becomes very concentrated and smaller in volume. If on the other hand, one has cysts in those collecting ducts, no matter how much antidiuretic hormone is made, the cysts cannot respond to the hormone and the urine volume stays high. Because of that, a child with ARPKD can get dehydrated much more easily than a normal child.

Q. Why am I having a terrible time trying to POTTY TRAIN my child with ARPKD?
A. This is another complication of not being able to concentrate the urine. There is so much urine volume that the bladder just cannot hold all that is made. So potty training, especially nighttime dryness, may be quite delayed. Be aware that this is to be expected and is not your child's fault.

Q. Can I use medications to help with bed-wetting?
No, they won't help. The kidneys can't respond to them. You need not do anything at all until your child is older than 6 years. Then you may find that the wet alarm systems may be helpful. They require full cooperation from family members for several months so don't get discouraged.

Fall/Winter 1998; issue 6

Q. There is Autosomal Dominant Polycystic Kidney Disease (ADPKD) and there is Autosomal Recessive Polycystic Kidney Disease (ARPKD). What does ***Autosomal*** mean?
A. Humans have 23 pairs of chromosomes. One pair is the sex chromosomes (XX for females, XY for males). Twenty-two pairs are ***autosomes***. They are numbered 1 to 22. Each has a short arm called p and a long arm called q. The two chromosomes in each pair are joined more-or-less in the middle to each other at the centremere.
In ARPKD the mutation or change in the gene is on chromosome 6p21-p12 region. In ADPKD the mutations are on chromosome 16p13.3 in 85% of cases and in most of the remainder the disease maps to the PKD2 locus, on chromosome 4q21-q23.

Q**.** Does environmental factors have any effect on the course of ARPKD.
A**.** No environmental factors that affect ARPKD have been identified.

Q. Does diet play any role in the progression of PKD? Would early protein restriction in the diet help to slow loss of kidney function?
A.There are experimental studies in rats that indicate that severe protein restriction may delay progression to end stage renal failure in cystic kidneys. I do not advocate this because children need protein to grow well and the restriction imposes a major burden without proven benefits.

Q. Should a child who is on a calcium channel blocker for high blood pressure monitor their calcium intake or have their blood calcium monitored closely? If calcium remains on the high end of normal, does this cause an increase risk for heart problems?
A.Calcium channel blockers do not affect the blood calcium levels and these should not be altered by diet. Children need calcium for good bone growth.

Questions answered* by *Dr. Bernard Kaplan, Director, Division of Nephrology and Chief of Medical Staff at The Children's Hospital of Philadelphia in Philadelphia, PA

Spring/Summer 1999; Issue 7

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Questions answered* by *Dr. Bernard Kaplan, Director, Division of Nephrology and Chief of Medical Staff at The Children's Hospital of Philadelphia in Philadelphia, PA

Fall/Winter 1999; Issue 8

Q. I know that yearly flu shots are recommended but I hate to subject my **post-transplant child** to yet another needle stick. Is it really necessary for her to have a flu shot every year?
A. Yes, it is necessary. The "flu" is caused by an influenza virus. This virus has the ability to evolve quickly by changing the structure of its outer "coat". This coat is how our immune system recognizes the virus. When the virus "changes coats," the immunity developed is no longer effective. So last year's immunization will not protect her from the flu this year. The immunity is still present, but the virus is different (on the outside anyway) each year.

Dr. Jeff Punch is a transplant surgeon and assistant professor of surgery at the University of Michigan. He is a member of the Scientific Advisory Board of CLASS

Q. My 8-year-old daughter has portal hypertension and enlarged liver and spleen. Although she is eager to keep up with her classmates, I don't allow her to participate in soccer or other vigorous sports because I'm afraid she might injure her spleen. But what about gymnastics, playing on the monkey bars or other playground equipment?
A. The spleen is like a bag filled with blood. This is because the spleen's purpose is to filter the blood supply of bacteria and remove old red blood cells. Normally, the spleen is safely tucked up under the ribs; but when the spleen is enlarged due to portal hypertension it can be felt below the ribs.

Whenever the spleen is felt below the ribs it is not protected from injury. Abdominal trauma such as a kick or direct blow with a blunt object, such as an elbow, stick, or bicycle handlebars may cause the spleen to rupture and leak blood into itself or into the abdominal cavity. This may occur rapidly and a child could die suddenly.

Vigorous exercise, including gymnastics, sit-ups, push-ups, and playground equipment are generally safe. A child with an enlarged spleen should not participate in contact sports, such as kick boxing, boxing, karate, hockey, and football. [**Editor's note:** Wrestling and Lacrosse may also not be considered safe with portal hypertension.] On the other hand, sports like soccer, baseball, volleyball, and basketball are generally safe (although elbowing can be dangerous). In some cases, an abdominal binder can be fashioned to help protect the spleen from injury.

Bicycle riding is actually one of the more dangerous activities for children with enlarged spleens. Spills are common and children can fall forward onto the handle- bars.

Accidental injury to the liver with significant internal bleeding is possible, but it is much less life-threatening. The liver has many blood vessels but not to the degree the spleen has. In fact, the liver might instead become quite hard and filled with scar tissue (cirrhosis). **«**

Dr. Berquist is Chief, Gastroenterology and Liver Transplantation at Lucile Salter Packard Children's Hospital at Stanford University and Chairman of the CLASS

Scientific Advisory Board.

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LETTERS FROM READERS

BROOKE & ARPKD

Brooke was first diagnosed as having ARPKD at birth after experiencing severe respiratory problems and the presence of grossly enlarged kidneys. A kidney biopsy was performed which confirmed the diagnosis. Once the respiratory problem was resolved during her first three weeks of life in neo-natal intensive care, the main concern was controlling her blood pressure and hydration. The disease causes the kidneys to produce large amounts of fluid and it has to be replenished with unusually large amounts of fluid intake.

The result of a liver biopsy when she was nine months old confirmed Congenital Hepatic Fibrosis had already begun its progression, portal pressure was elevated and her liver becoming rigid. We were informed that within two or three years the disease would progress to organ failure and the necessity for both a liver and a kidney transplant.

The first four years were very difficult for Brooke and trying for her father and I. She was susceptible to every little bug that came along and was hospitalized for pneumonia, an unidentifiable virus that almost took her life, and vomiting episodes requiring I.V. hydration. Brooke never had a problem with ARPKD effecting her development and remained at average weight and height throughout the progression of the disease. When she started kindergarten the illnesses seemed to level out and, except for less energy, she was just a normal elementary school kid.

By fifth grade, she was becoming increasingly tired and abdominal pain became an everyday occasion. Her peers at school were more critical and teased her unmercifully about her protruding abdomen and the fact that she was absent from school so much of the time. By then she had been diagnosed with esophageal varices and the teachers and staff had to be aware of a life-threatening "bleed" at any time. Completing schoolwork assignments and solving math equations was becoming harder. (I wrote about cognitive decrease as creatinine levels increase in a previous newsletter.) Feeling ignored by her teachers and peers at school and the resultant isolation was overwhelming for Brooke; she did not complete the fifth grade in the classroom.

A teacher came into our home during the school year and this allowed for the completion of sixth grade, because Brooke could not keep up the pace of mainstream schooling. She tried to go to school the first half of seventh grade but in April of 1997, she had a life-threatening bleed from two sites in her stomach. It was at that time that the TIPS was placed in her liver to shunt the blood flow and relieve portal hypertension pressure away from other organs, having been hospitalized for three weeks and time recuperating at home, the school year had ended. Brooke was placed on the list for a double transplant on September 10, 1997.

Hematocrit and platelet counts decreased and iron stores were at a critical low level so her Nephrologist prescribed iron infusion and subsequently Epoetin ALFA injections. The injections of Epoetin raised and kept the hematocrit and platelet count at a safe level and produced an energy euphoria that increased Brooke's quality of life; she was able to function <u>almost</u> normally. She tried going to school the second half of the eighth grade but had already missed so much that it turned out to be social exposure more than a proper educational experience.

Moving on to the High School as a Freshman was exciting and motivating, however, by mid-November she was tired all the time and in pain every day, she slept 16 to 18 hours a day. She dropped out of school, and on December 27, 1998, she developed bleeding colitis and was hospitalized. A lab technician and I watched the television in Brooke's hospital room as the festivities took place in New York City on New Year's Eve. Five minutes after mid-night the technician woke our sleeping beauty to take her first blood sample of the New Year, little did we know at the time, this would be the most eventful year of our lives so far.

Her ammonia level continued elevated, hematocrit low, CO^2 low, creatinine and BUN elevated. I believe the Lord said "Enough". January 16, 1999, the hospital transplant coordinator called with the news that organs had become available for Brooke.

For over a year, we waited with hopeful anticipation, the telephone call that would bring life to our child, but when it finally came, we were in total shock. Up to this point, we talked about organ transplant like a trip to the clinic and envisioned life after transplant as wonderful and trouble free, we could not have been more wrong! Please understand, however, the trials and frustrations of pre-transplant and post-transplant are worth the effort when viewing the result.

Anxiety sets in when all the cogs have been put into motion and it is too late to back out, you know transplant is the only answer but somehow want to postpone the finality of it to a later date. As a teenager, Brooke had second thoughts too; she was terrified and ill prepared for the transplant process emotionally. Backing out was impossible both physically and ethically, it was time for us to step aside and trust the integrity and skill of the surgeons and medical staff in whose hands she was now entrusted.

Twelve wrenching hours later the transplants were complete and the new organs working, it would be another two hours before we could see Brooke in the intensive care unit. The only information we were told as to the donor was that she was a young lady in her 20's, privacy is utmost and any original letter has to go through the hospital Social Worker. Eleven days post-transplant Brooke was released to the Hotel unit of the hospital, where we spent another three days before returning home.

Just six months post-transplant on July 16, Brooke is full of vigorous energy and enjoying life as never before, she has whipped all the devils and is rushing towards the new millennium full speed ahead. Foremost and most urgent on her list of "Things To Do", ride as many roller coasters this summer as she can find (that frightens her doctors) and go shopping for new clothes to fit her shrinking figure! (That frightens her parents)

It is not appropriate to go into details at this time as to complications and immediate aftereffects, but if anyone would like to e-mail me, I will answer any questions you might have concerning Brooke's experiences. **«**

My e-mail address is:
drobbinn@worldnet.att.net
or telephone number: 517-641-4231

ALISON MARCHESSAULT

Alison Marchessault has ARPKD/CHF. She is featured on the back cover of the "2nd edition of Your Child, Your Family, and Autosomal Recessive Polycystic Kidney Disease" booklet, published by the Polycystic Kidney Research Foundation. **AT THIS WRITING,** Alison is in the Intensive Care Unit at Jackson Memorial Hospital in Miami. On July 23, 1999, she received a kidney, and liver transplant. Since that time her kidney followed by her liver have failed resulting in two additional transplant surgeries. Since her arrival, she has battled numerous complications and infections, some requiring surgeries to correct.

Hello Everyone,

Well, the time has come to finally send some Great News! Everyone's prayers worked! We are going home! ALISON, BOB AND I hope to be home by the end of April!

July 21, 1999, ... seems like it was forever and a day that we got that call to stop our lives and fly to Florida. Eight months later, 15 times in an operating room, uncountable times in a procedure room, 3 livers and 3 kidney's later our little Alison is doing GREAT! When Alison was discharged on December 3rd she was unable to even sit up without help and needed to be moved from her wheelchair to her bed. After not walking for 5 months she needed to learn once again. Due to an IV line that Doctors were unable to remove at an extremely critical time she had no use of her right arm from the shoulder to the tips of her fingers. NOW, Alison is walking, using her wheelchair only for long distances, and her arm has movement down to her fingers, sensation isn't completely there yet but little by little it is moving down the arm and she is determined to use her right hand again. Alison literally works up a sweat during her occupational and physical therapy sessions that she receives 3 times a week. She also has a teacher that comes to do her homebound schooling 2 times a week and he feels she is right at her age level. Our little fighter has never lost her spirit and determination to live. She is so very happy. When she complains, we know something is truly very wrong.

Late in December it was determined that Alison had a blockage in her liver and a catheter was placed in it to help the bile flow through it. She had 4 procedures for this called a Cholangiogram which puts dye into the liver and helps the Doctors dilate the areas that are blocked with a small balloon.

The last of these procedures took place on March 9 and to everyone's delight there were no blockages found and the catheter was able to be removed. (I asked Alison in the recovery room if I could jump up and down and yell YIPPEE when we found out, but she said "MOM NOT NOW" so we all did when we got home!) The test has now been in the blood work...the last two weeks lab results show Alison Liver function improving steadily! By the way, her kidney has been functioning wonderfully, Thank God.

So the time has finally arrived for us to start shipping our belongings back to Massachusetts, but before UPS becomes our new friend we are taking Alison to Disney World. Before the transplant we had promised her that after her surgery when she was all better we were going to go to Disney World. This turned into something much more than a promise. While Alison was in intensive care we felt that keeping her motivated was somewhat of a way to help keep her alive. As we prayed we kept telling her you have to hurry and get better so we can go to Disney World. WE ARE GOING TO DISNEY March 22-March 26!! YIPPEE AGAIN! She is so very excited and so are we. One of her favorite things is a Carousel and if she wants to ride Cinderella's Carousel all day that's OK with us. Our good friend who just happens to be one of Alison's nurses from home will be going with us, it will be great to have another set of hands to help out (especially to take turns riding the Carousel)! Cheryl has been Alison's nurse and part of our family since the night Alison was born and this will be her 3rd visit to Florida since we've been here, but not since before Alison's last transplant...so this will be quite the celebration.

A much needed, long awaited celebration at that...its been a long rocky road one that we have learned much about ourselves and others in the past six years, since the night she was born when we told God that we wanted our Alison to survive when the Doctors had little hope that our 4 pound infant would live to the next day...that we would love her dearly and be the best possible parents to her. At that time we were told by a very spiritual friend that Alison was brought into this world to teach humanity and that Bob and I were her privileged Host and Hostess, a statement that has proven to be truer and truer as the years go by. The transplant was what we worked so hard to get Alison to for the last 6 years. Knowing that it still was not a cure for her kidney disease, just a new disease called post transplant. After the last eight months Bob and I are very tired, extremely grateful to have gotten Alison to this point, but of course never fathoming what the transplant would be like, or realizing that the after care for her would be much more involved than the pre-transplant care. Praying day after day at Alison's bedside that God take every ounce of our energy and give it to our Alison. Maybe Bob and I now know what a War Veteran lives like post war (with post-traumatic stress syndrome). We have many sleepless nights trying not to remember, thanking God every day that because of the medicine Alison was on during most of her hospitalization

she doesn't remember much. We are healing too. Looking hopefully to a healthy future. Bob will be going home to look for a new job, his company was understanding of the situation until December. We feel that's a small price to pay so to keep our family together. We never realized what it all was to become and we still don't know entirely what the future will hold, but the one thing we do know is that we are going home soon, all 3 of us so we can be a family of 5 once again. Due to the knowledge and care of many wonderful doctors, nurses, and all of the support of our family and friends our little girl will be celebrating her 7th birthday in a couple of months! We thank each and every one of you for your part in our Alison's journey.

Love to you all,

Bob, Estalee and Alison

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Note: If you would like to share your experience with other readers, please send your story to this newsletter in-care-of the Editor.

OUR STORY

By Christy and Dave Runner

The Runner Family, Dave, Christy, Suzanne (20), Jennifer (18), Vanessa (15), and Rebekah (13), live in Southern California.

Vanessa and Rebekah both have ARPKD, and were diagnosed in early childhood.

As most parents of children with any chronic health problems already know, our lives have been filled with visits to an assortment of doctors, specialists, countless hours of preparing daily medications, and the constant anxiety of, "Are we doing everything we can to give the best care to our kids?"

I don't want to go into every day, month, or year, but as our journey towards transplantation progressed, the anxiety levels seemed to grow. Fear of the unknown is usually much worse than the actual event. As we watched Vanessa's lab results gradually decline, her creatinine levels creeping up, we knew that the day was approaching when we would have to face the inevitable: dialysis and transplant.

During this past year, we, as a family, decided to take a more proactive approach to transplant, and presented the idea of using a family member as a live donor to our doctors. Our hope was that, not only would we not have to wait on a list, but we might even be able to by-pass dialysis for Vanessa.

After going through the usual drill of getting referrals from our HMO and making appointments, we found ourselves beginning the testing process of UCLA Medical Center. Christy was the first to be disqualified - wrong blood type. Dave was next, diagnosed with Adult, Type II Diabetes.

At this point, we had to weigh the possibility of one of our daughters being a donor. Without hesitation, Suzanne and Jennifer stepped up to volunteer, and also entered the testing process. Bottom line, Suzanne was a good match for Vanessa, and Jennifer was a perfect match for Rebekah. Although transplant for Rebekah is most likely several years away, it's very comforting to know that she and Jennifer are a match. We are very blessed as a family, that we have such as close and loving relationship. Suzanne and Jennifer may not fully realize the significance of their priceless gifts for many years, but as parents, we are so very proud of their selfless and special love.

On January 27, 2000, Vanessa was scheduled to receive Suzanne's kidney, already nicknamed "Suzy-Q." The days seemed to fly by, and at the same time seemed to drag, as THE DAY approached.

A couple of days prior to the scheduled surgery, Vanessa was admitted for some final tests, and to receive two dialysis treatments just prior to her transplant. Upon entering the UCLA medical "world," we were treated so well, treated with such genuine caring and understanding, that our fears started to fade. We knew we were in very capable and professional hands. Suzanne was admitted the afternoon before the surgery.

Very early on the 27th, Christy and I found ourselves, along with our best friends, Randy and Kim, looking down at our two daughters, each on a gurney awaiting their escort to the operating room. All of us, more than a little nervous, and doing our best to hide it, were deeply touched when Vanessa looked over at Suzanne and sang, "did I ever tell you you're my hero," a line from the song, "The Wind Beneath my Wings." All of a sudden, the fears and uncertainties seemed much smaller.

After four hours and several cups of coffee, Suzanne and Vanessa were both out of surgery. The transplant was "textbook" perfect, and both girls are doing fine. "Suzy-Q", Vanessa's new kidney, started working immediately, while still on the operating

table. There were several telephone calls to make to grandparents and friends, prayers of thanks to God for watching over our children, and a chance to finally relax just a little.

As I write this article, it is one month and two days since our experience. Both girls have recovered from their surgeries remarkably fast. Suzanne is back to her regular schedule. Vanessa is at home for several weeks due to her immune system being suppressed, and Christy and I are learning about new drugs, diets, follow-up doctor visits, and looking forward to returning to a normal routine.

We have been very blessed with excellent doctors, great medical care, four wonderful loving daughters, the endless support of our family and friends, and the watchful love and protection of our God.«

You, Me, and ARPKD

by Glenn Schellenberg

My name is Glenn Schellenberg, husband, father, and salesman. I have been invited to share my perspective on ARPKD in our family, and I am happy to do so. Being the husband, certain recollections are inherently suspect, but this is my story and I'm sticking to it.

My wife Cyndi, to whom I have been married thirteen years, and I decided to have children three years into our marriage. The pregnancy was textbook, aside from the fact that Cyndi is Rh-negative. That was a minor hiccough, except that she now carried a card that branded her as a "Known Rh-negative Woman." Oh, the humiliation!

Samantha was delivered by Cesarean Section. She was premature by six weeks, and weighed 3 lbs. 4.5 oz. Immediately upon her entering the world, I was taken into the Assessment Room to meet my little girl. I was terrified to see her. Trembling on the assessment table, she looked as horrified as I felt. Samantha would have to stay in the Neonatal ICU, until she got stronger, and the next three weeks would prove to be a tough time for us emotionally.

Once Samantha was home with us, life galloped at an alarming pace, but we managed to keep up. We went out and did things. We shopped. We ate out. As with any new baby, we learned to accept complete strangers cooing and ogling our child. And we learned to instantly hold in contempt, those that uttered the phrase "Oh, she's so tiny!", with an unnecessarily heavy emphasis on "tiny." We even felt obligated to loathe certain family members for it.

Aside from her size, Samantha was outwardly common. However, during a routine examination, at about four months, Samantha's pediatrician felt what he thought was an enlarged spleen. He ordered an ultrasound, and we waited for the results. When the results came back, our lives were changed in the blink of an eye. Ultimately, Samantha was diagnosed with Autosomal Recessive Polycystic Kidney Disease. "Excuse me?", I remember asking. "Auto-what?" Wrapping my head around the genetic explanation was one thing, but getting a grip on the emotions that immediately followed was quite another.

My very first reaction to the news was anger — directed straight at God.

"What unforgivable sin did I commit to deserve this?"
"What did I do?" "Why am I being punished like this?"

My concern was for my daughter, of course, but after all, we carried this corrupt gene. The genetics of this disease were explained to us, but there was immediately a sense of fault. What have we gotten this poor innocent child into? What kind of a rotten future have we created for her?

There were a thousand thoughts running through my head, all of the time, all of them grievous. My head was spinning. The mind goes off on its own tangent, out of control, and morbid visions are inevitable. I hated myself for that. I would lay awake at night, fighting images in my head that I didn't dare look at, for fear that if I did, it might become reality. But I couldn't stop the progression of these visions from marching through my consciousness. Many nights I did not sleep at all, because I was afraid of what I might dream.

It was some time, months in fact, before we told any family members about Samantha's ARPKD. On Cyndi's side, it was largely due to the fact we knew her parents would not likely take the news well. Having to explain the mechanics of it all seemed pointless, and I thought they would also feel the guilt. Looking back, I was hung up on the self-pity. That stuck with me for a long time. Time marched on though, and we slowly learned to deal with the disease. Samantha was quite healthy, otherwise, and many days we forgot about ARPKD.

About three and a half years later we decided to have another child. Actually, Cyndi decided, and I agreed. When she reads this, she may be surprised to learn that it was her suggestion, and I was very hesitant. We were aware of the odds, and were cautioned by our medical advisors against it. They said we had a one-in-four chance that any child would also have this disease, since we were both carriers of the gene. We looked at the odds, perhaps with simplicity, and believed there was a three-in-four chance we'd be lucky. If similar odds existed for the lottery, we'd be fools to ignore them.

Adam was born under similar circumstances. Cyndi found herself being rushed to the hospital long before her due-date. Only this time, we lived 200 miles away. She was transported by air ambulance in the middle of the night, while I got Samantha and myself packed, rested, and on our way to the city by car. Cyndi still hasn't forgiven me for not being there right behind her.

"What do you mean you went back home to sleep!?!?"

All the husbands reading this are visualizing "the look."

All initial indicators showed that Adam was clear of ARPKD. What a relief! The wind was unmercifully knocked out of us when Adam was one year old. The diagnosis came from out of nowhere. Adam suddenly showed progressed signs of the same kidney disease that Samantha had. How could this be? For all the progress we made in accepting the terms of this condition, it was all for naught, it seemed.

Once again I was plagued with sleepless nights. I remember having feelings again of pity, but not for myself. I fought off all my mind's urges to consider the mortality of this disease, and I still do. Adam's ARPKD has manifested itself a little differently than Samantha's has. His stature is also small for his age, especially when compared to the genetic giants we find surrounding our kids.

Anyone living with this disease knows transplant is the only viable remedy (at this time). We recently discovered that I am a blood and tissue match for both of our children, and Cyndi is a match for neither: Enter an entirely new batch of unwanted thoughts, and emotions. In my heart of hearts, I would give both of my kidneys - one for each of my children. "Let me go on dialysis," I would say. This of course is not possible, but I would do it in a heartbeat. I was initially appalled, as many people were, to learn that scientists had cloned a sheep. And the business of growing a human ear on a lab rat's back was over-the-top. But since we learned of my dual match, I have high hopes for further advances in cloning. Can they someday grow a third kidney inside of me? Even on a rat's butt, it doesn't matter.

About five years ago, my wife and I were one of four founding families of a parents' support group. We named it ***"Parents of Kids with Kidney Disease."*** We needed a group of like-minded people to be able to relate to. It was time to heal, and an opportunity to help others. What we quickly discovered was that we were not alone. More important, we were awakened to the fact that there are others worse off than us. By comparison, we had it very good. This was a critical step in helping me to get rid of the self-pity. I hope that I can one day say that I made a difference.

At the time of this writing, Samantha is 15-18 months away from needing transplant. So far we are doing well with this news, and actually looking forward to it. I am working to get healthier for her, losing weight and all. As the time draws nearer though, I know that I will have a lot more to handle. I now know, however, that I have a strong group of people around me to help. I look at Samantha and I see my biggest inspiration, and my greatest source of strength.

I don't know if I will ever really be able to deal with my children's disease. I don't know if I ever want to get too comfortable with it. I love my children, and I would give my life to see them healthy. Any parent would do the same. That is the love that we have for our children. In my case, it is because I know that I cannot take them for granted. ***Where I once thought I was being punished, I know now that they are a special gift from God. I am humble enough to know, that each day that I am granted to share with them is earned. And I thank God for that blessing***. «

Dear ARPKD Friends,

As we all know, ARPKD and CHF can bring friends and families a great deal of discouragement. Well, I am writing this article to give everyone a bit of encouragement. My name is Tia, I am twenty-five years old, and was diagnosed with infantile ARPKD and CHF at three weeks old. While I haven't been without my fair share of complications from these diseases, I feel I have had a fulfilling life so far considering my age and health.

At the time of my diagnosis, even less was known about these diseases and my parents were told next to nothing. Needless to say, my mother was very overprotective since she had no idea of what could happen to me. Now, as an adult, I understand why she treated me differently from my older brother who was perfectly healthy. I also understand how difficult it is for parents to decide where to draw the line for what they allow their children to participate in or not. Fortunately, for me, my parents were mostly in the dark and they didn't stop me from doing the things I enjoyed most, sports. Even more fortunately for me, I never got injured. I realize now how important it is for parents with children diagnosed with these diseases to interact with others in order for them to be informed of all health issues and be prepared should any complications arise.

I am sure many of you, especially parents, are curious about my medical history. Here is a brief synopsis of what I've experienced. Obviously, at birth I had two diseased kidneys and a diseased liver. As a result, I developed secondary portal hypertension, hyperslenism, and esophageal varices. Apparently, one kidney was much more underdeveloped which greatly stressed the other kidney. My doctors believed the stressed kidney would fail. Nothing was done and the kidney kept working under the stress and became quite enlarged. My kidneys have remained in this condition so far. As an infant I got fevers that doctors could not explain, "fevers of unknown origin," for which I took a preventative antibiotic daily for years. I also took liquid iron for my anemia until I could take a pill. My body kept functioning this way until I was ten years old when I experienced bleeding gastric varices. It wasn't until this episode that my parents were told that I should never have aspirin. At about twenty-two years old I got two urinary tract infections, then a bladder infection, then a kidney infection. I was hospitalized for the bladder and kidney infections to flush them out of my system in order to preserve my kidneys. My next encounter with the disease was about two years ago, at twenty-three years old. I had three occurrences, each three months apart, of rupturing cysts throughout the year. For each occurrence, I had severe bleeding, was hospitalized, and received two transfusions apiece. Since the last transfusion, I have been on Epogen and give myself one injection weekly. I have had two more occurrences of rupturing cyst since I have been on the Epogen and my body has handled the bleeding itself. This year a gallstone was discovered during a renal ultrasound. Other than these complications, I deal with the many side effects daily such as migraines, high blood pressure, heart palpitations, trouble with my blood sugar level, swelled ankles, and agonizing backaches. Currently, I take a beta blocker, high blood pressure medicine, a diuretic, and Epogen. My experiences may not compare to what other have already gone through but we are all in the same boat of not knowing what the future may hold.

I have since received my BS degree in Business Administration with a concentration in Finance and currently work in Connecticut as an Accountant and am going to start working on my Master's Degree in the fall. I also got married last year to a very supportive

husband. I am content knowing I've achieved two goals, which were very important to me despite the obstacles that I've encountered. But just like everyone else who feels the effects of these diseases I too get down at times and ask myself "why me?" Often I feel that people do not understand what I am truly going through. Other times I feel they do not believe me and I am exaggerating when I need time off for several doctor appointments or I am just too exhausted physically and/or mentally to do anything. It is then that I reflect upon what I have accomplished and what I deal with everyday and realize what a stronger person I am than others who are completely healthy and complain about a common cold. It makes me feel proud of myself and my accomplishments. It is important to realize that although you will always feel like you are riding a roller coaster in life, there is life out there for your children too.

If anyone would like to e-mail me my address is Bean92174@cs.com, I would be happy to hear from anyone or answer any questions I can. «

-Tia Hancock

KIDS TALK!

IRON MAN...By Andrew Gombas

My sister Lucy has a very rare disease that effects her kidneys by covering it with a tar like substance called cysts. My dad has run, biked, and swam over 100 miles all for Lucy. My dad did the Iron Man.

The Iron Man is one of the most treacherous triathlons on Earth! A triathlon is a course in which you swim, bike, and run. Its like a race only bigger. My dad was lucky he didn't collapse!

He did this to raise money for doctors to find a cure for this horrible sickness. I'm sure that when I grow up I want to be like him!«

—This reflection of the Iron Man Race, by Andrew Gombas was written at age 9, in the Fall of 1999. He is the brother of Lucy Gombas, an ARPKD child. (See related article on page 12.)

FOR ARPKD LOSS ……..

TO THE NEW ME

The holidays are coming and I'm afraid to face that fact. How will I ever get through Thanksgiving, Hanukkah, Christmas and the beginning of the new year without one who was so vital to my life? It is still several weeks before the time when everybody will expect me to be merry and happy. Yet, I dread that time as though it were tomorrow.

I know it is illogical, knowing how much I loved and cared, for me to expect there to be "fun" days in the immediate future; instead I will accept that I have been wounded and I must take the time to recover from my deep emotional wounds.

I hope I will be wise enough to recognize that what I am feeling is normal. I will try to remember that those parents, who have suffered the loss of a child earlier than my loss, have given me the hope that it will be better; that my family will survive to go on to better days. I will look forward to that time and try to be patient.

By *Mary Cleckley*

— Reprinted from the 1998, Fall Issue, "Loving Arms" newsletter published by the Pregnancy and Infant Loss Center, Inc., Wayzata, MN

COPING WITH GRIEF

Sometimes grief can get pretty hard. We may find ourselves exhausted beyond our capabilities, hurt beyond endurance and lonely beyond belief. Grief can be so isolating. No one seems to know what to say or how to behave around us. Many of us have discovered we are grieving not just the death of our loved one, but the loss of friendships, self-esteem and self-identity as well.

When our child died, we were surrounded by people, but the silence was deafening. Hardly anyone spoke. Maybe they were afraid that death was "catching" or maybe they just didn't know what to say. I didn't know what to hear either! As the months passed, it just grew darker and I began to wonder if we would ever know peace, joy or love again.

Life as we knew it and planned it and dreamed it was gone. My days were filled with emptiness rather than the activities I had anticipated. I found myself unable to concentrate long enough to read a book or watch a favorite television program. I couldn't remember ANYTHING and I began to think I had not only lost a child, but my sanity as well. There were no happy sounds in our house any more. Joy had been buried one afternoon in late fall and winter had come to reside within our walls and within our hearts.

I couldn't imagine living very long and even began to pray for some type of "relief". The pain was understandable. The silence,

however, was unbearable.

Eventually I managed to leave the house and then I began to run, to run as fast and as far as I could. I believed that if I kept "busy" the grief wouldn't overwhelm me. I thought I could run away from the hurt, the pain, the awful silence! I even tried joining the circus, but they told me that I cried too much. I couldn't even join the circus!

Life became something to be endured. The days and months began to accumulate, all jumbled together in an endless, faceless stream of time spent. Nothing mattered anymore. I didn't care about the seasons, the news, the weather, what I ate, what I wore or who I lived with or loved. Life had been reduced to BLANKS and I had nothing to fill in.

Attending a support group was the furthest thing from my mind and from my experience. We lived on a remote air force base, surrounded by trees, not people. I knew about groups for the drug addicted, the over and under weight, but a group for bereaved was beyond my imagination. WHO would go? WHO?

I didn't even know where to begin looking for such a group. In fact, there weren't any support groups or organizations where we lived. There were very few even in existence 24 years ago. But, one afternoon, I happened to be listening to a friend recount her troubles when I found myself thinking how nice it would be if someone would just listen to me! So, I began to really listen to her and for the next hour, I found myself immersed in her life, not mine. My own trials and tribulations took a back seat to her needs and as she finally left, she hugged me and thanked me for being so kind. She said she felt "so much better" and what a magic touch I had! Magic touch? Hardly! I hadn't even touched her until we hugged good-bye. All I had done was listen.

ALL I HAD DONE WAS LISTEN. That's the secret to helping someone. We are always trying to find the "right" words to say, the "right" things to do. There are no words in any language known to mankind that will make it all right that someone you love has died. There, are, however, words that can make it less lonely. And those words are so simple: "I AM HERE. I may not know exactly what to do to help, but I AM HERE and together, we'll figure out something."

It is the gift of your presence that helps sooooooooooo much! You don't have to say anything. Just BE THERE and the magic begins. You cannot take away my hurt, but you can make it less lonely.

I discovered something else that afternoon. I not only discovered how simple it is to listen; I also learned that listening to someone else helped me. As this friend thanked me for helping her, I found my own burden a little easier to bear. It was as if I had been "lifted" slightly, unburdened for a moment and I had been granted a few moments of "breathing space". My own troubles, my own grief was still with me, but I had, for a moment, returned to being the caring, concerned human being I once had been. I hadn't lost myself after all. I could still care!

Each time we reach out across our own pain, to find another hand searching in the darkness, we begin to lighten our own darkness. Each time we send out a message of love or hope or simple presence, we receive back the same message. We are NOT alone when we reach out to others.

Eventually we were transferred to another duty station and there I did find the beginnings of a support group for bereaved parents. I put my energies into helping build that support system, for myself and other grieving parents. My own healing paralleled my involvement with others. As I continued to reach out, others reached towards me and the circle of healing expanded.

So, the next time you feel the walls closing in and the silence getting too loud, REACH OUT. Reach out to someone else who needs a listening ear, a quiet hug, or a compassionate voice. You need not tell your story to be of help to someone else. You need only to listen and the healing will come, for both of you, in the giving and receiving of support.

Healing begins at the end of your own hand. Be careful, however, not to completely expect your own pain to disappear as you become involved with helping others. We each must still do our own "grief work" and work it is! But never again do we have to be alone unless we choose to be so. Be aware of your "agenda" so your caring does not become an excuse to postpone your own healing. Take good care of yourself as well. Healing begins from the "inside out" and the best care you can give is to model your own healthy growth towards wholeness.

Together we will join hands and hearts across the earth and decorate the world with hope, healing, and laughter. We are forever linked through the love of our children, parents, husbands and wives, siblings, grandparents, friends and all our loved ones who dance across the rainbows ahead of us. We are a family circle, broken by death, but mended by love. **«**

Darcie D. Sims is the author of *Why are the Casseroles Always Tuna*?: A Loving Look at the Lighter Side of Grief. The book is available for $10.00 each plus shipping and handling.

Call (612) 473-9372.

--Reprinted with permission from ***Loving Arms***, Quarterly Newsletter of the Pregnancy and Infant Loss Center, Inc., Volume 17, No. 2, *1999 Summer Issue*

NOTE: "A Path to Healing", 12th National Perinatal Bereavement Conference, September 22-24, 2000, in Cincinnati, Ohio. Call Alana Roush at 513-569-6402 for more information."

Dearest Daniel

My sweet little baby, how I wish you could have stayed,
To fulfill all the dreams and plans we had made.
To hold you and kiss your soft cheeks again would be a gift so sweet,

But I know that I must wait until Heaven for us again to meet.
I want to remember the bittersweet joy of your birth,
And forever hold onto my memories of all of your life here on Earth.

As time goes by, I still wonder why,
Your life was destined for me to say goodbye.
The void and emptiness you left behind,
Will be filled with love all in good time.
The pain sloly melts away,
As you and God shine your light on me every day.

We knew each other from the start,
But now I need to know you deep in my heart.
Help me to learn how to share,
Your wonder with the world out there.
Teach me faith and hope and love,
With God's help and care from Heaven above.
Add some strength and courage, too;
It isn't easy living here without you.

Why don't people understand
How much I want to hold your hand?
Or read you a story while you sit on my lap,
Or to snuggle up with you and take a nap.
Oh how I dreamed of all your firsts; I was ready for it all,
To teach you all the things I've learned, we were going to have a ball.

Oh, so many tears I've shed,
Lying awake each night in my bed.
Trying so hard to let go of all that was supposed to be,
And learning new ways to be close with you and God within me.
In my pain and struggles I have come to know,
How very much I love you so.
It is not what I choose, but I have to accept that this is the way we must be,
To share a special closeness only meant for you and me.
I often wonder how you are;
Are you a twinkling little star?
Or are you the wind that touches me,
Blowing across the deep blue sea?
Send your love on Heaven's wings,
Help me see the wonder in all things.
I need you to guide me to be all I can be,
To keep you close in my heart and memory.

Love,
Mommy

In Loving Memory of Daniel Robert Carlson, born February 18, 1993
(Daniel was born with ARPKD and passed away shortly after his birth.)

Dear Angel

I saw you once, it wasn't long; I won't forget that day.
Your perfect, precious little self; hello, good-bye, same day.
A tiny little angel; perfect, chaste and pure.
Although only a moment, that memory will endure.
Our time together was very short; we really didn't meet.
All my life I'll dream about the patter of your feet.
I wish you hadn't left us; I wish you could have stayed.
We would have lived and laughed and loved
as you grew and learned and played***.***
Since I can wish for anything, I wish that you were here.
I wish that I could hold you close and whisper in your ear.
Together as a family, there would be much to do.

Your Mom and I and brother Tim, we always will miss you.
This is no joy like children bring; so wonderful, so precious.
Such sorrow now in losing you,
it leaves me nearly breathless.
If sorrow serves a purpose, I wonder what it is.
It seems like empty, useless pain. I hope its more than this.
You've changed our lives in unknown ways;
more change will surely come.
But we'll never change the splendor of
your memory in our home.
I look forward to the times in life when we are all alone.
You'll be there with me in my thoughts,
my precious little one.
I wish I could have held you; instead we have to part.
I'll honor you and cherish you and hold you in my heart.
I will love you and remember you, your precious little self.
I'll dream of you forever. Good-bye, take care, stay well.

Love, Papa
Written in memory of Angela Rose Sherry
by her father, Bill Sherry — 1996

— from *Loving Arms*, Winter 1996, Issue, Volume 14, a quarterly newsleter. Reprinted by permission of Pregnancy And Infant Loss Center.

Reflections of a Mother Denied

On this, my first Mother's Day, I asked myself, "Do I have the right to celebrate Mother's Day?" Have I truly been a mother this past year? The answer is yes. Each day I have cared for my child as every mother does, except differently. In every way possible I have mothered him.

I have mothered him with every tear shed, through the agony of longing to hold him. I have rocked him in my heart if not on my arms. I have kissed his little cheeks in my mind if not with my lips. Smelled his sweetness with my hopes if not with my nose. Felt his softness with my memory if not with my hands. Tickled him with my wishes if not with my fingers.

Am I a mother? I truly am. My physical mothering has been limited to lovingly tending his grave. But I am a mother all the time.

Michelle M. Parrish, mother of Stephan Andrew

—Reprinted by permission of Compassionate Friends, TCF/Baltimore, MD.

TIPS FOR THE BEREAVED COUPLE:

By Elisa Stone
Sharing Parents

- Communication is probably the single most important tool for getting through the loss of a baby within the couples relationship.
- Remember that each person grieves differently. No way of grieving is wrong; refrain from judging each other.
- Your partner may not cry but he/she does miss the baby as you do. Grief manifests itself in many different ways. Accept your partner's differences.
- Your partner may not seem happy anymore, but, if you are supportive toward his or her current feelings, the day will come when smiles return to your lives.
- Your partner may be very angry but that is a stage of grief, expressing anger is OK as long as safety is maintained.
- Let each other know how you are doing. Create a code with each other. An example is, "I am having a SAD day." Your partner will then know where you're coming from.
- If you don't want to talk when your partner does, set up a time when it is mutually acceptable. It is alright to not want to talk, just remember the first tip — communication is very important — mutually plan a time when you can.
- Take care of each other. When you are feeling a little better, go on a date. Remember, the love between you created the child.
- Together plan a way to memorialize the baby. For example, go to a nursery and chose a tree together. Plant it in memory of your baby.

- And remember, taking care of yourself is an important part of being successful as a couple. Take some time to yourself, nurture yourself, and encourage your partner to do the same. Above all else, do what feels right for you and the rest will likely find a new balance.

-Reprinted with permission from the Pregnancy & Infant Loss Center, Inc.; featured in their newsletter, "Loving Arms", Spring 1999 Issue.

SAD VACATIONS

Vacations are great for relaxing and taking a break from the routine of life. But as bereaved parents, vacations can be one of the most difficult times of the year. We are supposed to be having fun, relaxing and revitalizing ourselves. But how can we do this when our child has died?

Our first few vacations were disasters. I felt torn between the desire to enjoy life again and the need to keep Matthew and my grief alive. There was no way to rush through the process. The pain had to be faced even more directly without the diversions of daily routine.

A few things helped me to bear vacations. Some of these were necessary only for the first few years, some are still a part of any vacation. I set aside a time each day to remember Matthew, and to try to deal with my grief. Sitting on a rock jetty in Panama City, Florida, overlooking an ocean sunset, I felt God in a way that I had not felt since Matthew's death. Setting a time each day to remember him is a way to include him in my trip.

Every new place I go, I bring something home in Matthew's memory. A shell sits on the shelf in the office. Matthew never went to the beach, but this is Matthew's shell.

Accept your feelings. You may not feel happy. That will come later. For now, accept your grief. Accept the fact that vacations mean something different than before your child's death.

Accept the fact that you need rest. Be kind to yourself. Do only the things you feel you can do. Eventually, you will smile again. Eventually, you will have fun again.

Kathy Boyette

-Reprinted with permission from the Pregnancy & Infant Loss Center, Inc.; featured in their newsletter, "Loving Arms", Summer 1999 Issue.

COPING SKILLS FOR PATIENTS: Taking Control Control of Your Illness

By Mary Beth Callahan, ACSW/LMSW ACP

Farmers Branch Dialysis
2280 Spring Lake Rd., #110
Farmers Branch, TX 75234
(972) 488-1191

Taking Control of Illness

- Know as much as possible — Read, Read, Read then
- Ask, Ask, Ask
- Be involved in decision making
- Talk to your health care team
- Keep active
- Have goals
- Know your rights and responsibilities

When & If Needed, Making Treatment Choices

- Assess your lifestyle
- Review advantages and disadvantages of each treatment type
- Talk with your family and treatment team
- Talk with people who have been on dialysis or transplanted
- Visit a dialysis center

Finding and Selecting a Transplant Center

- Locate centers (UNOS 800/24-DONOR)
- Gather information — waiting times, # transplants done/year, survival rates
- Visit the center
- Talk to staff
- Talk to patients

Talking with your Nephrologist

- Make a list
- Make sure that your complaints, expectations and treatment are clear
- Make sure your doctor understands your concerns
- Be very specific
- Describe other contacts with physicians
- Carry a list of medications and doses
- Work together

Members of the Health Care Team

- YOU

- Social worker
- Dietitian
- Nurse
- Technicians
- Nephrologist

Adjustment Issues
- Feelings/Common reactions to illness
- Role/Task changes
- Dependence/Independence issues

Coping
- Gain understanding
- Talk about your feelings
- Focus on the positive
- Keep active/Exercise
- Re-establish balance in life
- Use available support (family, friends)
- Laugh
- Draw on your inner strengths

Coping as a Caregiver
- Be aware of your own symptoms of stress
- Accept changes as they occur and be realistic
- Learn what resources are available
- Share responsibility if possible
- Stand up and be counted
- Take good care of yourself
- Focus on the good times as they occur

Common Feelings
- Loss
- Sadness
- Anxiety
- Depression
- Uncertainty
- Confusion

Draw on Your Inner Strengths
- An ability to look on the positive — Hope and Happiness
- The acceptance of change — Flexibility
- The ability to hang on — Stick-to-itiveness
- The enjoyment of the present — Pleasure
- The realization of a strength beyond ourselves — Faith and Gratitude

Susan Jaskula, ACSW, ESRD patient

Paying for Treatment
- Medicare
- Insurance
- Medicaid

Medicare Part A & B
- Part A—Hospital Insurance—has deductible
- Part B—Medical Insurance—has a smaller deductible—you pay a monthly/quarterly premium

You become Medicare eligible…
- When you need maintenance dialysis or a kidney transplant and
- You are insured or are getting monthly benefits from SSA or RRS or
- You have worked long enough in government employment or
- Have a spouse who meets these requirements or
- Are under 21 and have a parent who meets these requirements

Insurance
- Employer group health plans—Coordination of Benefits period (30 or 33 months)
- Managed Care
- Health Insurance Portability and Accountability Act

Medication Assistance
- Insurance
- Medicaid
- Medicare (only transplant)
- State Renal Program
- Home Delivery Programs

Social Security Programs
- Social Security Disability—unable to work ($700 gross/month, 7/1/99)—disability has lasted or is expected to last for at least 1 year—amount you receive is based on # quarters paid to SSA and amount of earnings during that period
- Supplemental Security Income—based on resources and monthly income
- How to apply: 800/772-1213

Employment
- Prepare your employer
- Try to maintain your employment—improves adaptation to illness—stabilizes financial status
- Retraining
- Vocational rehabilitation commission
- Americans with Disabilities Act (ADA)

Empowerment
- "a state of mind in which you authorize yourself to make educated decisions, leading to a healthier life. Empowered patients demand and receive what they know is right or best for their overall health...With empowerment comes responsibility."

Doug Strickland, ESRD patient, Family Focus, Vol. 7, No. 3

Special Resources for People with Kidney Disease

American Association of Kidney Patient(www.aakp.org)(800/749-AAKP)

National Kidney Foundation (www.kidney.org)(800/622-9010)

Polycystic Kidney Research Foundation (www.pkdcure.org)(800/PKD-CURE)

Life Options Rehabilitation Advisory Council (www.lifeoptions.org)(800/468-7777)

National Organization for State Kidney Programs (800/733-7345)

Rehabilitation Services Administration (inet.ed.gov/offices/OSERS/RSA/rsa.html)

American Kidney Fund (www.akfinc.org)

United Network for Organ Sharing (UNOS) (www.unos.org)

Social Security Administration (www.ssa.gov)

United States Renal Data System (www.med.umich.edu/usrds

Renalnet (www.renalnet.org)

National Council on Disability (www.ncd.gov)

RESOURCES

BROCHURES

United Network for Organ Sharing (UNOS) a nationwide computer registry 1-800-330-8500:

1.) "What every patient needs to know"
2.) "Questions patients should ask"
3.) "Financing transplantation"
4.) "Donor card brochure"

American Liver Foundation 1-800-223-0179:
"Facts on Liver Transplantation" brochure

Stadtlanders Pharmacy 1-800-238-7828:
"National Transplant Resource Directory" (will assist with the national resources available)

[Most of the above brochures are free.]

BOOKS TO ORDER

(from the Association for the CARE of Children's Health, 1-800-808-ACCH)

When Your Child Has A Life Threatening Illness *- A supportive booklet for parents with several different topics covered.*

Price: $5.00

What About Me? When Brothers and Sisters Get Sick *- Siblings of sick children often are confused and have many different feelings. The story of a young girl whose brother is sick.*

Price: $10.95

Becky's Story *- Another story which helps brothers and sisters of hospitalized children to look at and understand their own responses and feelings.*

Price: $6.50

"Lizzy Gets A New Liver"—written by Lizzy Poling — A children's book printed in both English and Spanish. Purchase price of **$11.95** (**order #09550)** includes shipping and handling. To order call: **1(800)524-2612**

OTHER PUBLICATIONS

"***The Transplant Educational Foundation***", is operated

∗by transplant family members. Services available include: newsletter, family publications, and an annual family conference, at 1-800-448-3354.

CLASS (CHILDREN'S LIVER ASSOCIATION FOR SUPPORT SERVICES)
Call 1-800-255-0353 to receive a free 20 page booklet called "Your Child and Prednisone" written by transplant surgeon Dr. Jeff Punch in a Q & A format. The organization also produces a quarterly newsletter.

SANDOZ, a pharmaceutical company, has support materials available that cover pre and post transplant issues encountered by kidney and liver candidates and recipients. They are free of charge by calling 1-201-515-7500.

"TRANSPLANT VIDEO JOURNAL" is a quarterly video news journal which covers transplant news and features stories. To receive free of charge, call 201-515-9888.

THE MAGIC FOUNDATION for CHILDREN'S GROWTH can be reached at 1-800-3 MAGIC 3 for information regarding issues of growth hormone deficiency. A newsletter is available.

WEB SITES:

The American Board of Medical Specialties' site:
www.certifieddoctor.org, allows you to browse for doctors by specialty and locale.

FOR TRANSPLANT SUPPORT ON THE WEB:
URL (Uniform Resource Locator); the address of a document or site that may be visited. Just type URL:TRANSWEB.ORG for transplant information.
"NEWS" has the latest information which is constantly being added to.

ORGANIZATIONS:

American Kidney Fund
—is a national voluntary health organization dedicated to improving the daily lives of people with chronic kidney disease through patient aid programs.
6110 Executive Blvd., Suite 1010, Rockville, MD 20852-9813. Telephone: 301-881-3052
Toll free hot line: 1-800-638-8299
E-mail: helpline@akfinc.org.

American Association of Kidney Patients (AAKP)
—Their purpose is to help patients and their families cope with the emotional, physical and social impact of kidney disease.
100 S. Ashley Drive, Suite 280, Tampa, Fl 33602
Telephone: 1-800-749-2257.

Polycystic Kidney Disease (PKD) Access Center— Health Q & A, Medical information, organizations, PKD support group, Listserve, Friends groups, AOL Chat room, and more at:
Http://www.nhpress.com/pkd/groups.html

Children's Liver Alliance — Their mission statement: empowering the hearts and minds of children with liver disease, their families and the medical professionals who care for them. — **3835 Richmond Ave., Box 190, Staten Island, NY 10312**
Telephone/Fax: 1-718-987-6200

National Patient Air Transport Helpline (NPATH) — The only such service of its kind in the U.S., NPATH maintains current data on all known charitable, charitably-assisted, and special discount commercial long-distance air medical transport options.
Telephone: 1-800-296-1217

COTA — Children's Organ Transplant Association — 1-800-366-COTA — Free fundraising advice and support for transplant patients, including campaign start-up kits and on-site staff assistance. COTA provides nonprofit status to local campaigns, making tax-deductible contributions possible. There is no fee for services.

American Association of Kidney Patients
(AAKP)—Their purpose is to help patients and their families cope with the emotional, physical and social impact of kidney disease.
100 S. Ashley Drive, Suite 280, Tampa, Fl 33602
Telephone: 1-800-749-2257.

Charitable Medical Air Transportation:
NO-COST, LONG-DISTANCE MEDICAL AIR TRAVEL AVAILABLE

One of America's best kept secrets is the existing charitable medical air transportation system. It's purpose is to ensure that no financially-needy patient is denied access to distant specialized evaluation, diagnosis or treatment for lack of funds to pay for long-distance travel.

During this year (2000) the system will serve in excess of 18,000 patients plus family members.

The system provides transportation either via the airlines (for longer trips) or via private aircraft flown by highly qualified FAA licensed volunteer pilots.

Many charities are involved—but the largest and only "full service" system in the country is that of **Angel Flight America**.

Access to this system is by calling the National Patient Travel **HELPLINE** at **1-800-296-1217**. Information help is available 24/7/365 —Internet assistance can be accessed at **www.PatientTravel.org.**

Patient families and medical centers need to know about this system and make use of its resources.

BEREAVEMENT:

Pregnancy and Infant Loss Support, Inc. are people caring for people: caregivers or parents who have experienced the devastation of the death of a baby, supporting others who are going through the same pain. SHARE is international with 240 chapters. **Call (314)947-6164** for the chapter closest to you.

Compassionate Friend—write—P.O. Box 3696, Oak Brook, IL 60522— **Call—(630)990-0010**

Bereavement Packet—available for grievous parents who have lost a child to ARPKD. Contact Colleen Zak at:
1-717-529-6732
Zakland@epix.net

QUOTE —

"TO ACCOMPLISH GREAT THINGS, WE MUST NOT ONLY ACT, BUT ALSO DREAM, NOT ONLY PLAN BUT ALSO BELIEVE"
—Anatole France

DISCLAIMER

Information from articles and resources in this newsletter is not intended as an endorsement, but as a way to educate and inform parents. We recommend that parents evaluate the information and resources carefully; and discuss them with other professionals and parents if there are any questio

HAVE YOU MOVED?

— Please contact the editor with any address changes so you may continue receiving the ARPKD/CHF Newsletter. Newsletters returned because of address changes will not be forwarded.

NEWSLETTER MAILING LIST

—If you have a child with ARPKD and are not on our mailing list and wish to be, please write or contact the editor. Likewise, if you wish to be taken off our mailing list, please let us know. We encourage you to share this newsletter with other ARPKD families.

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