Liver Disease in ARPKD

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Medicine, especially in complex and rare diseases, is often more art than science.

Confusing Terminologies

Congenital Hepatic Fibrosis

Caroli's Syndrome

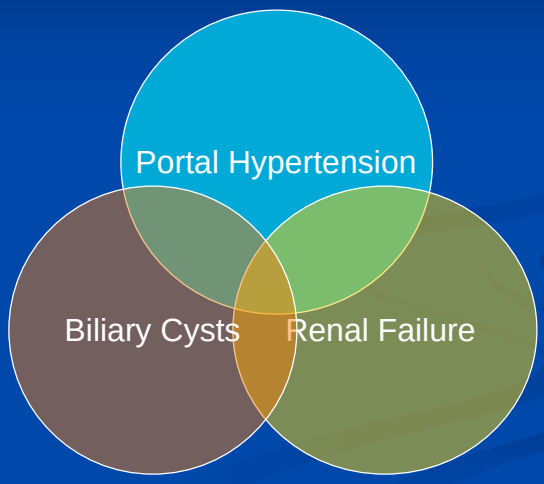
Bile Duct Ectasia

Caroli's Disease

Choledochal cyst

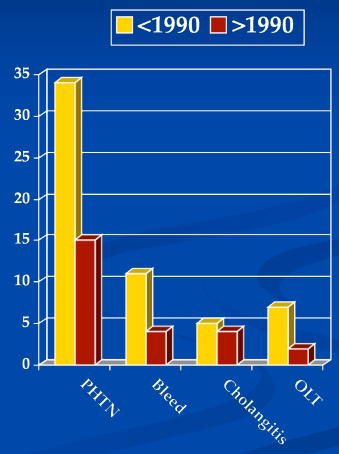
Ductal Plate Malformation

Disease Types



Prevalence of Liver Disease in ARPKD

- Liver disease significant issue in many children with ARPKD
- Risk of mortality may be higher
- Management complex

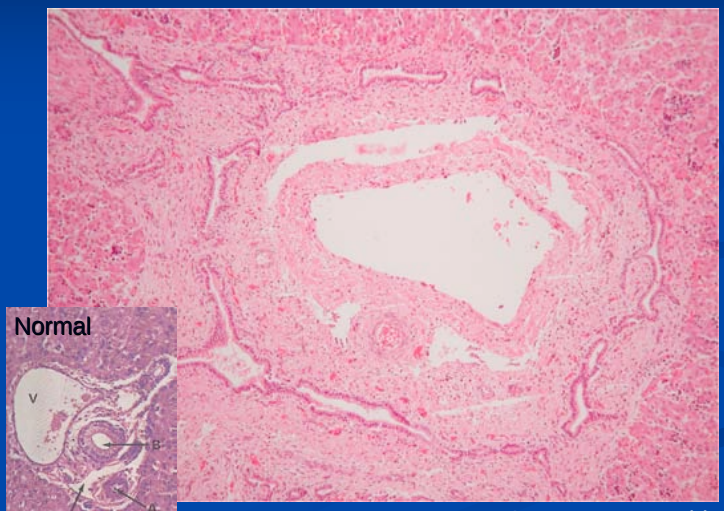


Guay-Woodford *Pediatrics* 2003;111:1072

Issues to Discuss

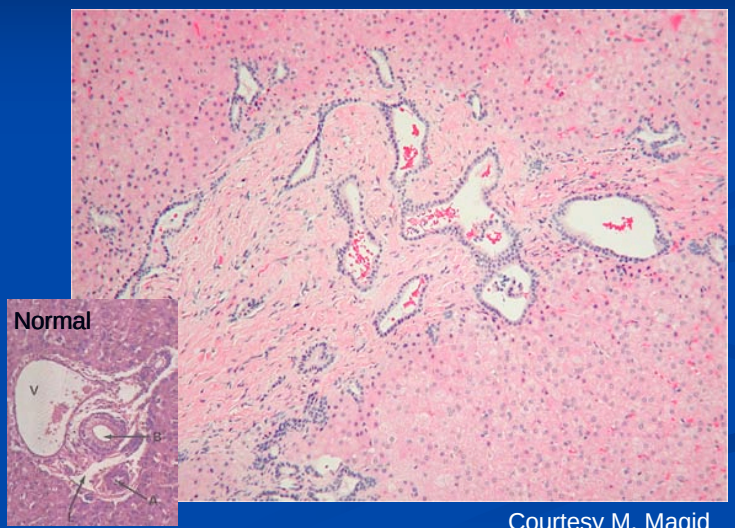
- Portal Hypertension and Biliary Disease
- Surveillance and Interventions
- Approaches to Transplantation

Pathology: Ductal Plate Malformation



Courtesy M. Magid

Pathology: Congenital Hepatic Fibrosis



Courtesy M. Magid

Portal Hypertension: Issues

- Spleen size risk of injury
- WBC and platelet counts
  - Infection
  - Bleeding
- Variceal bleeding

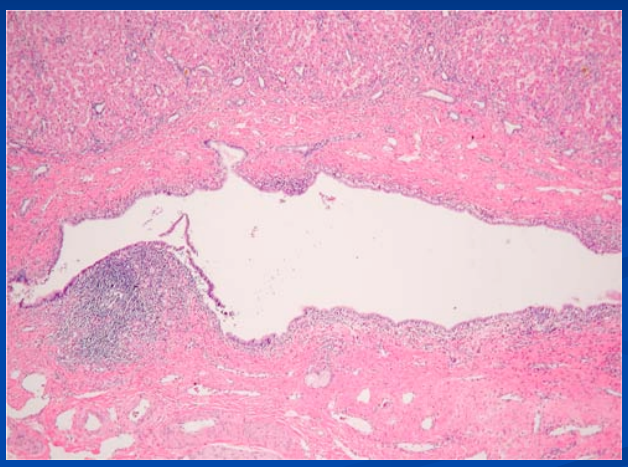
Portal Hypertension: Surveillance

- Spleen size on physical examination
- WBC and platelet counts
- Ultrasonography
- Endoscopy
- Upright oxygen saturation

Portal Hypertension: Interventions

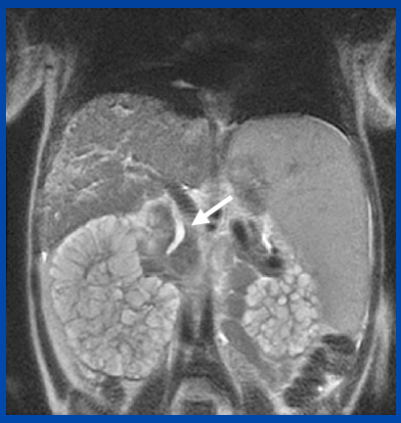
- Activity restrictions
- ß-blockade: propranalol
- Endoscopy
- Surgical shunt
- Immunization issues
  - Pneumococcal, meningococcal

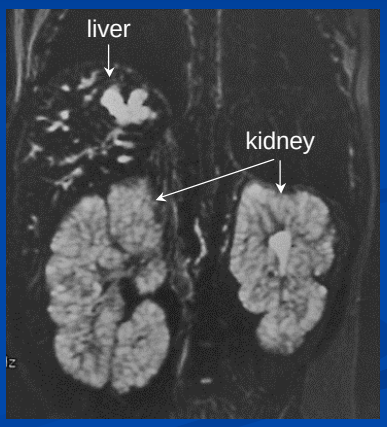
Pathology: Intrahepatic Biliary Cyst



Courtesy M. Magid

APRKD Biliary Cystic Disease



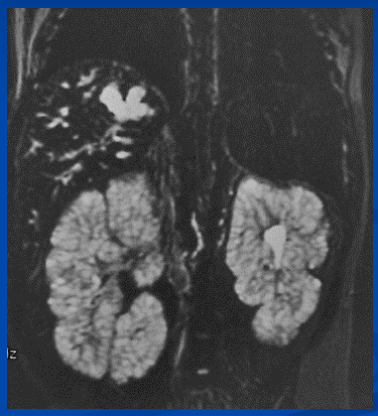


Biliary Disease: Issues

- Cholangitis/sepsis
- Cholelithiasis (stones)
- Pancreatitis
- Cholangiocarcinoma

Biliary Disease: Surveillance

- Importance
  - 64% of post transplant deaths attributed to sepsis
    - Davis *Pediatr. Transplant.* 7:364-369, 2003
- Ultrasonography
- MR Cholangiogram
- High index of suspicion



Biliary Disease: Interventions

- High index of suspicion
- Prophylactic antibiotics ?
- Ursodeoxycholic acid ?

Disclosure: Speaker's Bureau for Axcan-Scandipharm

Special Issues: Transplantation

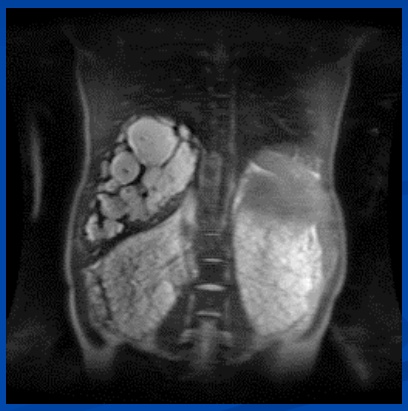
- Indications for portosystemic shunting
- Indications for combined liver and kidney transplantation
- Approaches to combined liver and kidney transplantation

Porto-systemic Shunting

- Intrinsic liver disease minimal - relatively intact function
- Refractory variceal hemorrhage
- Pre-emptive for renal transplant in severe portal hypertension

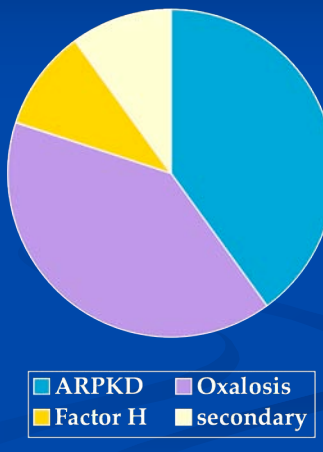
Indications for Combined Liver-Kidney Transplantation

- Recurrent biliary sepsis
- ? Sepsis - indeterminant dsource
- ? Marked biliary abnormalities
- Refractory hemorrhage
- Hepatopulmonary syndrome



Approaches: Combined Liver-Kidney Transplant

- 10 out of 315 ARPKD children at Mt. Sinai received combined transplant
- Joint management
- 9/10 long-term survival (2 mo to 7 years)
- Approaches
  - LRD x 3
  - LRD/Cad x 1
  - Sequential x 4
  - Simultaneous x 6



Summary

- Liver disease related to ARPKD is of varying clinical significance
- Portal hypertension and biliary tract infection are the key clinical issues
- Collaborative surveillance and interventional approaches optimal
- Special considerations for transplantation