

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

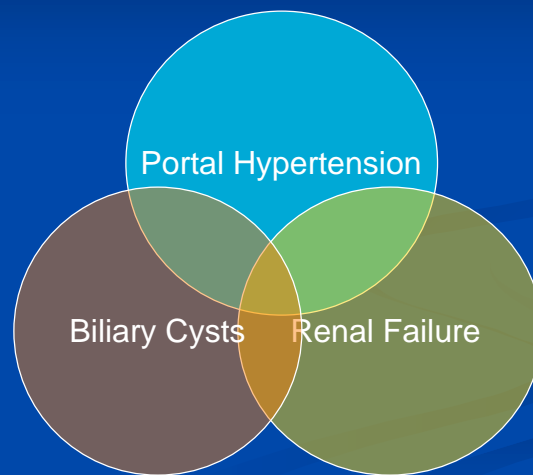
Bile Duct Ectasia

Caroli's Disease

Ductal Plate Malformation

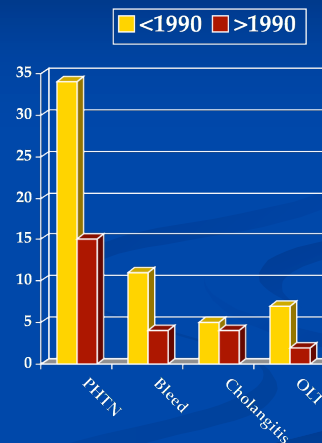
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Disease Types



Prevalence of Liver Disease in ARPKD

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

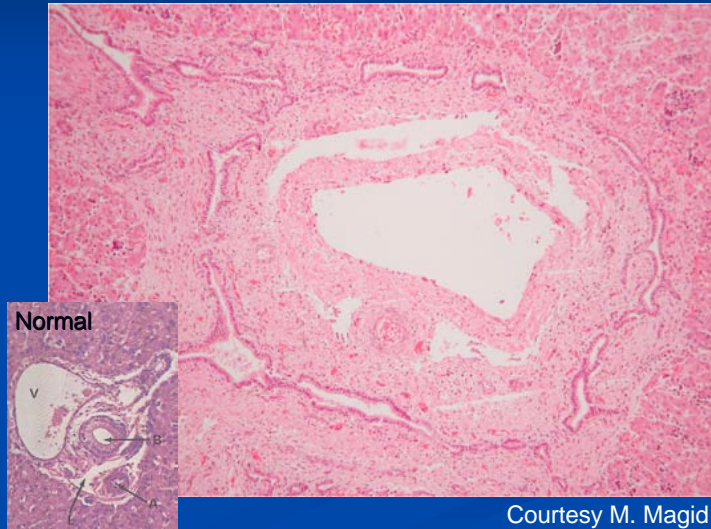


Guay-Woodford *Pediatrics* 2003;111:1072

Issues to Discuss

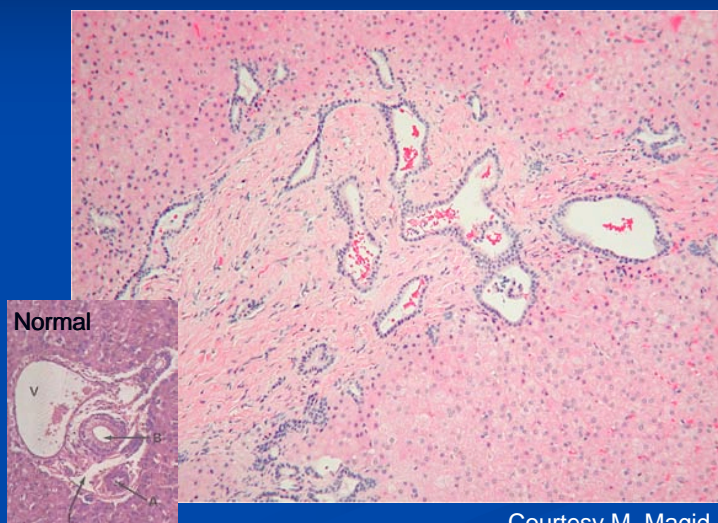
- Portal Hypertension and Biliary Disease
- Surveillance and Interventions
- Approaches to Transplantation

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

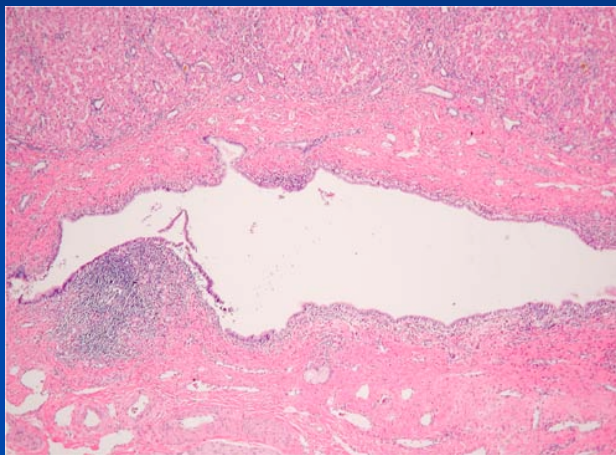
Portal Hypertension: Surveillance

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

Portal Hypertension: Interventions

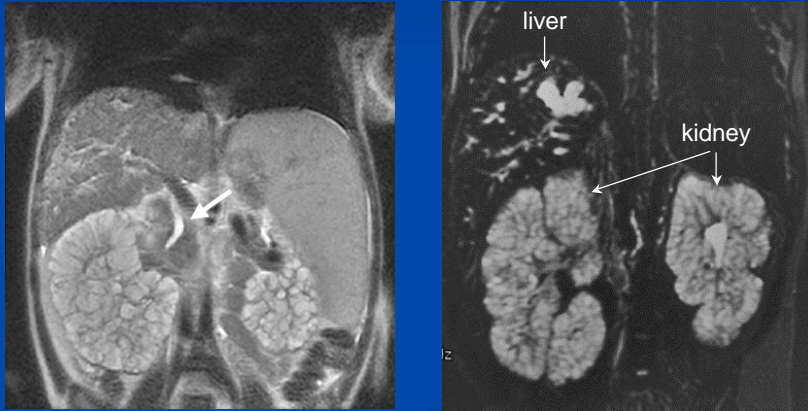
- Activity restrictions
- β -blockade: propranolol
- Endoscopy
- Surgical shunt
- Immunization issues
 - Pneumococcal, meningococcal

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

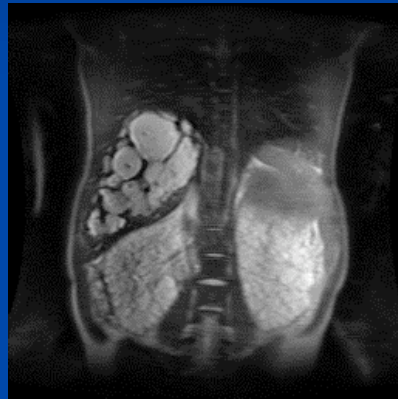
- Indications for portosystemic shunting
- Indications for combined liver and kidney transplantation
- Approaches to combined liver and kidney transplantation

Porto-systemic Shunting

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

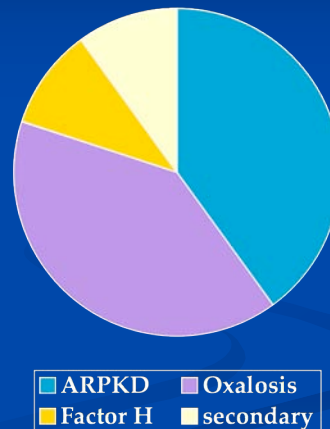
Indications for Combined Liver-Kidney Transplantation

- 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