Liver Disease in ARPKD

Benjamin L. Shneider, MD
Professor of Pediatrics
Chief, Division of Pediatric Hepatology
Mount Sinai School of Medicine

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

Disease Types

- Portal Hypertension
- Biliary Cysts
- Renal Failure
Prevalence of Liver Disease in ARPKD

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

Issues to Discuss

- Portal Hypertension and Biliary Disease
- Surveillance and Interventions
- Approaches to Transplantation
Pathology: Ductal Plate Malformation

Pathology: Congenital Hepatic Fibrosis
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: 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
- 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 source
- ? 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