ARPKD Clinical Consensus Conference
May 2013 Washington

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1\textsuperscript{st} ARPKD Clinical Consensus Conference

• Organiser: Prof Lisa Guay-Woodford, leading ARPKD expert
1st ARPKD Clinical Consensus Conference - Objectives

• International experts from nephrology, hepatology, genetics, perinatology and behavioural pediatrics to:
  – Update diagnostic criteria for ARPKD
  – Recommend best practices for diagnosis, monitoring and management of ARPKD
  – Discuss biomarkers and the prerequisites for developing clinical trial protocols in ARPKD
Steering Committee

- Lisa Guay-Woodford (Chair)
- John Bissler (Cincinnati): Nephrology
- Ben Shneider (Pittsburgh): Hepatology
- Marshall Summar (CNMC): Genetics
- Eric Eichenwald (UT Houston): Perinatal/neonatal issues (primarily the lung)
- Kristy Hardy (CNMC): Neurocognitive/behavioral issues
- Ron Perrone (Tufts): FDA/EMA liaison
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- **Work Groups:** 3-5 experts
- **Pre-meeting:**
  - Reviewed and summarised all literature 2000-2012
  - **Topics:**
    - Post-natal management
    - General Management
    - Dialysis
    - Renal Transplantation
    - Liver Transplantation
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• At meeting:
  – Each Group reviewed and discussed summaries
  – Developed consensus guidelines
  – Presented summaries to assembled experts with group discussion
General & Renal Summary

• Good evidence (from RCTs) lacking
• 2 large series and rest case reports so need for international joint approach
• Number of patients affected ??
• Renal recommendations:
  – postnatal, dialysis, nephrectomy, transplantation
• General management:
  – Nutrition, UTIs, HT, growth, intracranial aneurysm, low sodium
Perinatal Summary

• Prenatal management once ARPKD is suspected, differentials – what else can it be?
  – Genetic testing: who should be tested and costs
  – No indication to deliver early if low amniotic fluid alone
  – 10% pts on Database have chronic lung disease
  – Pts should be referred to unit with kidney, heart, lung support facilities for delivery

• Postnatal:
  – Once delivered, ventilation: when to use ECMO
  – If not needing ventilatory support, should have nephrology and genetics consult
Hepatic Summary

- Liver: screening for liver problems with blood tests and ultrasound
- Antibiotic prophylaxis for cholangitis
- Surgical intervention guidance for cholangitis and/or portal HT
- Portosystemic shunting
- Liver Tx: decompensated liver disease doesn’t tend to happen so
- Indications: recurrent cholangitis and portal HT
- Discussion about types of antihypertensive medications for both systemic and portal HT
Neurocognitive/Psychological Summary

• Neurocognitive outcomes:
  – No specific studies but 24% problems with attention and Tx doesn’t correct this
  – 18% hearing loss
  – Memory and learning improve after Tx

• Psychosocial functioning:
  – Depression and anxiety

• Issues of obesity
Summary

• Also discussed TEMPO study and what markers we can use in ARPKD for designing trials
• Discussion with FDA
What happens next?

• Post-meeting
  – Summary of meeting with recommendations for future directions that incorporates discussion with FDA/EMA
  – Each Group to prepare clinical consensus manuscript for publication
  – Ideas for research
  – 1st of many and international collaborations to continue and to include other continents
Any questions
2nd ARPKD Family Information Day

Thank you for attending
My infinite gratitude to

Tess Harris
Kate Paget
Melanie Dillon
Jane Cotterill
Volunteers: ward 1
BCH Volunteers
BCH Play Centre: Dave Baker and his staff
Speakers: Raine Family, Carsten Bergman, Paul Winyard, Detlef Bockenhauer, Jo Jarvis, Melanie Dillon, Khalid Shariff
ARPKD RDG Members
Lisa Guay-Woodford
Sea Life Centre
PKD Charity
Arran Brown Rainbow Foundation
Renal Association
Kidney Research UK
BCH
And all the patients and families with ARPKD

For a better future together