

# ARPKD Clinical Consensus Conference

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 Polycystic  
Kidney Disease  
Charity



# 1<sup>st</sup> ARPKD Clinical Consensus Conference

- Organiser: Prof Lisa Guay-Woodford, leading ARPKD expert



# 1<sup>st</sup> 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

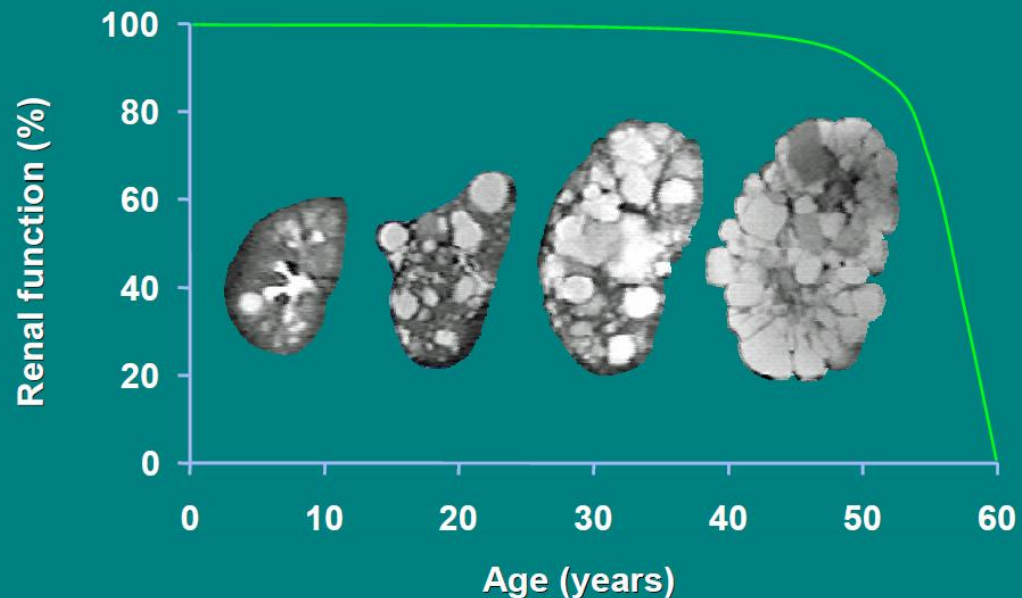
# Neurocognitive/Psychological Summary

- 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

## When to Treat and What to Measure?



# 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
- 1<sup>st</sup> of many and international collaborations to continue and to include other continents



**Any questions**

# 2<sup>nd</sup> ARPKD Family Information Day

Thank you for attending

Birmingham Children's Hospital  
NHS Foundation Trust



**pkd** Polycystic  
Kidney Disease  
Charity



# 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*