CHILDREN'S HOSPITAL

Center of Translational Science
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CLINICAL RESEARCH STUDY

FAQ SHEET for Potential Participants

TITLE OF STUDY:	**Core A: The Hepato-Renal Fibrocystic Diseases Translational Resource**
PRINCIPAL INVESTIGATOR:	**Lisa Guay-Woodford, MD**
IRBEAR PROTOCOL:	**Pro00003209**

INTRODUCTION: We are conducting a research project as described below.

PROJECT:

We are doing research to learn more about Autosomal Recessive Polycystic Kidney Diseases (ARPKD) and other Hepato-Renal Fibrocystic Diseases (HRFD). This is brief information about our research study: "Core A: The Hepato-Renal Fibrocystic Diseases Translational Resource" and how to participate in it. Not much is known about your disease. We would like to create a registry of clinical information for your disease with the goal of trying to understand more about your disease.
This study has two parts, the clinical database (medical health information), and the educational part. In this study we want to build a registry with information about HRFD to learn more about these diseases. We want to provide information about these diseases to families, physician and genetic counselors via the study website.

What are Autosomal Recessive Polycystic Kidney Diseases (ARPKD) and Hepato-Renal Fibrocystic Diseases?

ARPKD and other HRFD are genetic diseases affecting the kidneys and liver. They occur mainly in infants and children and cause serious and sometimes deadly health problems. Some HRFD disorders include Joubert syndrome, Bardet Biedl syndrome, Meckel-Gruber syndrome, congenital hepatic fibrosis (CHF), Caroli syndrome (CS), polycystic liver disease, oro-facial-digital syndrome, nephronophthisis (NPHP), and glomerulocystic Kidney Disease.

Who can participate in this study?

Males and females up to 35 years (including fetuses) with a diagnosis of ARPKD or another Hepato-Renal Fibrocystic disease based on clinical information, imaging studies, biopsy, autopsy or genetic data.

Who cannot participate in this study?

Individuals affected with: Autosomal Dominant Polycystic Kidney Disease (ADPKD), urinary tract malformations, or other fatal and severe defect of other systems present at birth suggesting a diagnosis other than HRFD.

If you would like to enroll yourself or your child (alive or deceased) as a participant in Core A: "The Hepato-Renal Fibrocystic Diseases Translational Resource", please follow the instructions provided below.

INSTRUCTIONS:

•On the website **http://www.arpkdstudies.uab.edu/** (Patient/family information and consent box) click on Consent and download our Informed Consent Form and assent (if the participant is 12 to 18 years old). Read through the forms for more details about the study.

•After you have reviewed the Informed Consent Form (s), please click on Contact Information, complete the required fields, and submit it. The data will be sent encrypted (secure) via Internet to the Research Study Coordinator: Elena Gibson (egibson@childrensnational.org). She will receive an automatic E-mail notification alerting her to review this information. She will contact you by phone to discuss the study and consent form, and answer any questions that you may have.

•After getting all your questions answered sign the Informed Consent and/or Assent Forms and mail them to us via US mail. You will receive a copy of your consent form signed by Dr. Guay-Woodford or Ms. Gibson.

•After Ms. Gibson receives the signed Informed Consent (and assent if appropriate), the participant in the study will be assigned a unique number.

•You must also complete the form titled: "**Authorization for Release Medical Information for NIH-Funded Study: Core A: Hepato-Renal Fibrocystic Diseases Translational Resource**." Upon signing this form, you authorize the participant's treating physician to send your clinical data to our site, where we will enter the participant's clinical data into the database.

This study does not require a clinic visit to our center and does not provide free genetic testing. Thank you for your interest!

RISKS/CONFIDENTIALITY: Researchers do not expect any risks from your participation in this study. There is always a potential for a breach of confidentiality. Only the people working on the study will know your name. Researchers will take the necessary steps to prevent this risk from happening by keeping the records of this study confidential.

VOLUNTARY PARTICIPATION: Your decision to participate or not participate in this research will not affect your current or future care at Children's National.

QUESTIONS: If you have any questions, please call the Principal Investigator, Lisa Guay-Woodford, MD at: 202-476-6439 or Elena Gibson, the Clinical Research Coordinator at 202-476-2197.