CHILDREN'S NATIONAL MEDICAL CENTER

Center of Translational Science
111 Michigan Avenue, NW
Washington, DC 20010
(202) 476-6439

CONSENT TO PARTICIPATE IN A CLINICAL RESEARCH STUDY AND AUTHORIZATION TO USE PROTECTED HEALTH INFORMATION

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

"You" refers to "You" or "Your Child" throughout this document.

INTRODUCTION: We would like to invite you to be part of a research study at Children's National Medical Center. Before you decide if you would like to participate, we want you to know why we are doing the study. We also want you to know about any risks (anything unexpected that might happen) and what you will be expected to do in the study.

This form gives you information about the study. Your study doctor will talk to you about the study and answer any questions you have. We encourage you to discuss this study with your family and anyone else you trust before making your decision. We will ask you to sign this form to show that you understand the study. We will give you a copy of this form to keep. It is important that you know:

- You do not have to join the study;
- You may change your mind and stop being in the study any time you want.
- If we make any important change to the study we will tell you about it and make sure you still want to be in the study.

A. PURPOSE OF STUDY

We know very little about Autosomal Recessive Polycystic Kidney Disease (ARPKD). It is a rare inherited disorder that occurs in 1 in 20,000 people. It is part of the hepato-renal fibrocystic diseases group. This disease affects boys and girls equally and affects the kidneys and liver. It occurs mainly in infants and children and causes serious health problems but can also occur in adults.

We want to obtain more information on Autosomal Recessive Polycystic Kidney Disease (ARPKD) and other hepato-renal fibrocystic diseases. We also want to expand our web-based resources so anyone can learn about ARPKD or other hepato-renal fibrocystic diseases.

You are invited to be in the study because you have been diagnosed with a hepato-renal fibrocystic condition and are younger than 35 years of age. You do not have any illnesses that would not allow

you to participate in this study, like autosomal dominant polycystic kidney disease (ADPKD) or any other major health problems that you were born with.

This Children's National Medical Center study (Pro00003209) is being conducted in collaboration with Duke University (**Study Title: Duke Task Force for Neonatal Genomics: Genome and Exome Sequencing and Return of Results**). Our colleagues at Duke University have a well-established system for gene**tic** studies. Therefore we are working with them to better understand the genetic cause of this set of disorders. Genetic material (DNA) for these studies can be obtained from a blood sample. For these studies, we need a blood sample from the parents and the affected patient.

B. PROCEDURE

If you choose to be in the Duke University study, you will sign this consent form and give us the information of the doctor where you prefer to have blood samples collected. Children's National Medical Center will send a mailer and a blood collection kit to the doctor of your choice. You will then have blood collected (8.5 mL if two years of age or older; 5 mL if less than two years old). When the doctor you specify obtains the blood samples, these samples will be sent to Children's National Medical Center and each sample will be processed to get the DNA. These DNA samples will be labeled with an identifier that is unique to you and stored in the Clinical Studies Resource BioRepository of the Clinical and Translational Science Institute at Children's National.

If you choose to be contacted by the Duke Task Force for Neonatal Genomics: Genome and Exome Sequencing and Return of Results Study, we will send the Duke University study team your contact information. After you speak with the Duke study team, you may choose to sign their informed consent to participate in the Duke University genetic studies. Once we receive notification that you have provided the Duke study team with a signed Informed Consent for their genetic studies, Children's National Medical Center will send your DNA to Duke University for genetic analyses.

C. POTENTIAL RISKS/DISCOMFORT

This study will involve gathering a limited amount of information from patients and parents. This information will include the patient's diagnosis and age at diagnosis as well as the patient and parent names and your contact information. This information will be assigned a unique identifier number and your Personal Health Information will be held in the strictest confidence. Your name will be known only to Dr. Guay-Woodford, the Research Coordinator, and the collaborative study team at Duke University. The information that you supply will be added to our database and referred to by a unique identifier number instead of your name.

There are some minor risks that may be associated with blood collection including discomfort from the needle stick, bruising, fainting, weakness and rarely infection at the site.

D. VOLUNTARY PARTICIPATION

Your participation in this research database is voluntary. There will be no penalty or loss of benefits to which you are otherwise entitled if you decide to withdraw from the study.

E. POTENTIAL BENEFITS

There is no direct benefit to you or your family if you choose to participate. The reason for this study is to learn more about the clinical and genetic factors that affect the clinical course of the hepato-renal

fibrocystic diseases. The genetic results will be validated in a clinical laboratory and the clinical report will be disclosed to you and your family.
There is a possibility that results from this protocol may provide important new insights for the future care of people with these diseases.

F. ALTERNATIVES TO PARTICIPATION

The alternative is to not participate.

G. QUESTIONS – WHO TO CALL

We want you to ask questions about any part of this study or consent form either now or at any time in the future. If you have any questions about this study, call the Principal Investigator, Lisa M. Guay-Woodford, MD, at 202-476-6439. If you believe you have been injured as a result of being in this study, you should call the Principal Investigator, Lisa M. Guay-Woodford, MD, at 202-476-6439. If you have any questions or concerns about your rights in this research study at any time, please call the Office for the Protection of Human Subjects at (301)-565-8452, the Chief Academic Officer, or the Chair of the Institutional Review Board of the Children's National Medical Center. The last two parties may be reached at (202) 476-6439.

H. CONFIDENTIALITY

We will keep the records of this study confidential. Only the people working on the study will know your name. They will keep this information in case we have to find you later to let you know of any new information that may affect your health. The federal government can review the study records and medical records to make sure we are following the law and protecting the children in the study. Your medical record is confidential, but just like any medical record; there are some exceptions under state and federal law.

HEALTH INSURANCE PORTABILITY AND ACCOUNTABILITY

In 1996 the government passed a law known as The Health Insurance Portability and Accountability Act (HIPAA). This privacy law protects your individually identifiable health information (Protected Health Information or **PHI**). The privacy law requires you to sign an agreement so researchers can use or share your PHI for research purposes. This describes to you how information about you may be used or shared if you are in a research study. It is important that you read this carefully and ask a member of the research team to explain anything you do not understand.

I authorize Lisa M. Guay-Woodford, MD and her research staff to create, access, use, and disclose my PHI for the purposes described below.

Protected Health Information that may be used and shared includes:

☒ Information that identifies you such as name, address, telephone number, date of birth, Social Security number, and other details about you
☒ Information that relates to your health or medical condition from your medical records
☒ Information obtained from the study procedures outlined in this consent form, for example: things done to see if you can join the study such as physical exams, blood

and urine tests, x-rays and other tests, and any other medical information we learn from you about your health history and family history
☒ Laboratory results obtained on specimens collected from you (blood, urine, tissue)

The Researchers may use and share my Protected Health Information with:

♦The Principal Investigator, other Investigators, Study Coordinators, and all administrative staff in charge of doing work for the study;
♦Government agencies that have the right to see or review your PHI, including but not limited to the Office of Human Research Protections and the Food and Drug Administration;
♦Children's National Medical Center Institutional Review Board;
♦Audit Committee of the Children's National Medical Center Institutional Review Board;
♦Quality Improvement Program Coordinator and other staff in the Office for the Protection of Human Subjects at Children's National Medical Center.
♦Duke University Medical Center, Center for Disease Modeling.

In addition to the Researchers, only the above people and organizations, may also use my Protected Health Information:

Also, your primary physician will be contacted if during the course of the study the researcher learns of a medical condition that needs immediate attention.

Should your health information be disclosed to anyone outside of the study, your information may no longer be protected by HIPAA and this Authorization. However, the use of your health information will still be regulated by applicable federal and state laws.

Storage of PHI in a Database:

We plan to store personal health information collected from you in this study in a Research Electronic Data Capture (REDCap) database is maintained at Children's National Medical Center.

Please indicate your approval of any or all of the following by initialing next to the statement:

My personal health information may be stored in the above named database.
☐ Yes ☐ No _____ initials

Researchers from the Duke Task Force for Neonatal Genomics: Genome and Exome Sequencing and Return of Results Study may contact me to discuss the genetic studies that they are performing in ARPKD and other hepato-renal fibrocystic diseases.
☐ Yes ☐ No _____ initials

Researchers may contact me to request my authorization for future studies that are not related to this study or the disease named above.
☐ Yes ☐ No _____ initials

My personal health information may be stored without any of my identifying information for use in other studies of other diseases.
☐ Yes ☐ No _____ initials

If you agree to participate in this research study, the research team, the research sponsor (when applicable) and the sponsor's representatives, may use Personally Unidentified Study Data. The Personally Unidentified Study Data does not include your name, address, telephone, or social security number. Instead, the researcher assigns a code to the Personally Unidentified Study Data. Personally Unidentified Study Data may include your date of birth, initials, and dates you received medical care. Personally Unidentified Study Data may also include the health information used, created, or collected in the research study. The research team or the research sponsor may share the Personally Unidentified Study Data with others to perform additional research, place it into research databases, share it with researchers in the U.S. or other countries, or use it to improve the design of future studies. They may also publish it in scientific journals, or share it with business partners of the sponsor and to file applications with U.S. or foreign government agencies to get approval for new drugs or health care products.

You do not have to sign this Consent/Authorization. If you decide not to sign the Authorization, you will not be allowed to participate in the research study.

After signing the Consent/Authorization, you can change your mind and:

♦ Revoke this Authorization. If you revoke the Authorization, you will send a written letter to:
Lisa M. Guay Woodford, MD
Children's National Medical Center
111 Michigan Ave NW,
Washington DC, 20010

To inform her of your decision.
♦ If you revoke this Authorization, researchers may only use and disclose the PHI that was collected for this research study before you revoked the Authorization.
♦ If you revoke this Authorization your PHI may still be used and disclosed if you should have an adverse event (unexpected side effect).
♦ If you change your mind and withdraw the Authorization, you will not be allowed to participate in the study.
♦You will be allowed to review the information collected for this research study.
This Authorization does not have an expiration date.

If you have not already received a Notice of Privacy Practices from Children's National Medical Center, you may request a copy and will be given one. If you have any questions or concerns about your privacy rights, you may contact the Children's National Medical Center Privacy Officer at 301-572-6348.

I. Payment for Medical Care for Research-related Injury:

Children's National Medical Center cannot promise that the risks we have told you about or other unknown problems will not happen. If you think that something unexpected happened because you were in the study, please call the Principal Investigator at 202-476-6439 or the Chief Academic Officer of the Children's National Medical Center at (202) 476-5000. If something unexpected happened resulting directly from your participation in this research study, we will give your child any urgent medical emergency treatment needed if the injury is reported in a timely manner. The Hospital will seek payment from your health insurance company or other third-party payor for any medical care or services you receive. The Hospital has no program to provide you with any additional payments as a result of any injuries.

J. ADDITIONAL ELEMENTS

- **Genetic Information Nondiscrimination Act (GINA)**

A new Federal law, called the Genetic Information Nondiscrimination Act (GINA), generally makes it illegal for health insurance companies, group health plans, and most employers to discriminate against you based on your genetic information. This law generally will protect you in the following ways:

- Health insurance companies may not request your genetic information that we get from this research
- Health insurance companies may not use your genetic information when deciding whether to insure you or the amount of money they will charge you.
- Employers may not use your genetic information that we get from this research when deciding to hire, promote, or fire you.

This new Federal law does not protect you against genetic discrimination by companies that sell life insurance, disability insurance, or long-term care insurance.

Research Subject Advocate:

The National Institutes of Health supports a Research Subject Advocate or RSA for the research study that you are being asked to join. The RSA, Dr. Tomas Silber, is here to answer your questions or concerns about taking part in this research. Dr. Silber does not work for the study doctors who are doing this research and they do not pay him. He is here only to help and protect you during any research.

You may contact Dr. Silber at any time. This can be done before you decide to take part in the research, during the study, or even after you finish the study. You can call Dr. Silber at 202-476-3066 or reach him by e-mail at tsilber@cnmc.org.

CONSENT/AUTHORIZATION:

I am the participant or I am authorized to act on behalf of the participant. I have read this information and will receive a copy of this form after it is signed.

By signing this form, you agree that you have talked to your study doctor about the study and understand it, and you want to be in the study. You agree that we have talked to you about the risks and benefits of the study, and about other choices. You may decide to stop being in this study at any time and no one will mind and nothing will change about your medical care other than not being in the study. Copies of this form will be:

(1) Kept in the study file by the Principal Investigator;
(2) Put in your medical record; and
(3) Given to you to keep.

Please call the Principal Investigator, Lisa M. Guay-Woodford, MD, at 202-476-6439 if you have any questions.

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Printed Name of Participant: _____
Medical Record Number: _____
Printed Name of the Mother_____
Printed Name of the Father_____

Signature of Participant: _____Date:_____
(Participant must be 18 years of age or older)

Signature of the Mother _____Date:_____

Signature of the Father: _____Date: _____

AFFIDAVIT OF PERSON OBTAINING ASSENT FOR CHILDREN 7-11 YEARS OLD:

I have explained all aspects of the research study to the child participant to the best of his/her ability to understand.

I have answered all of the child participant's questions relating to the research study.

I believe the child participant's decision to enroll is voluntary. I have explained to the child participant that he/she can withdraw from the research study at anytime.

The study doctors and study staff agree to respect the child participant's physical or emotional dissent at any time during this research study when that dissent pertains to anything being done solely for the purpose of the research.

Printed Name of Individual Obtaining Assent: _____

Title: _____ Signature: _____ Date: _____