CHILDREN'S NATIONAL MEDICAL CENTER

Center of Translational Science
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Washington, DC 20010
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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**
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 get 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. You do not have any illnesses that would not allow

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

B. PROCEDURE

If you choose to be in this study, we will ask your permission to see your past, current, and future medical information. This study does not require a clinic visit to our center. If you choose to be in the study, we will ask for your medical information related to your disease. Some information that we could collect, would be clinic notes, lab results, and physician consult reports. You will be asked to sign a release of medical information form to allow the study team access to your medical information.

When we receive the information, the research study team will be able to enter your medical data into the Hepato/Renal Fibrocystic Diseases clinical database. There will be initial data entry in our database and follow up data entries lasting for the duration of this study or until you choose to not participate in the study anymore. We will remove your name or any other identifiable health information (such as name, address) from your received records before entering your medical data into the Hepato/Renal Fibrocystic Diseases clinical database.

If you choose to participate in the optional genetic material testing portion of the study, we will send a mailer and a blood collection kit to the doctor of your choice. You will then have blood collected (~5 mL or a teaspoon). 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 only 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. They will be tested to search for a genetic basis for ARPKD. As recommended by the National Institutes of Health, the sequences (without your name or any further identifier) will be submitted to national databases to facilitate research into genetic causes of conditions.

We will ask you to drop out of this study if:

- ☐ We have not been able to get medical information from you/your doctors.
- ☐ Your diagnosis of ARPKD or another hepato/renal fibrocystic disease is not verified.

C. POTENTIAL RISKS/DISCOMFORT

This study will involve gathering information from patients, parents of minor patients, and your doctor and/or medical record review when necessary. There is no direct physical risk to you related to information gathering. Your medical information will be given a unique identifier number and your Personal Health Information will be held in the strictest confidence. Your name will only be known only to Dr. Guay-Woodford and the Research Coordinator. Your name and medical record number are on the data forms. The information your doctor supplies will be put into the database and referred to by a unique identifier number instead of your name.

Genetic testing may provide information about how health or illness is passed on to you by your parents or from you to your children. This knowledge may affect you emotionally. Other family members may also feel stress, anxiety or depression because how this genetic information affects them. Some genetic testing can also determine if people are directly related, meaning that it can show that a person was adopted, or that their biological parent is not their legal parent.

If this information wasn't previously known, this may cause distress. Dr. Guay-Woodford and the research team can help you understand what the genetic information from this study could mean for you and your family.

Any stored genetic material will be labeled by study identifier and not with any Personal Health Information. Your Personal Health Information will not be provided to anyone outside of the study team. There is a still a small risk, however, that you may be identified from your DNA. 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. These risks are only applicable if you choose to participate in the optional genetic material portion of the study.

Blood draw for genetic study: To extract DNA from your blood, a medical professional will perform a blood draw. About 1 teaspoon of blood will be taken from your vein using a needle. This is the standard method used to obtain blood for routine hospital tests. This will take approximately 5 minutes. You can expect to experience some momentary pain when your blood is drawn; the discomfort should be minimal. In a few cases, a small amount of bleeding under the skin will produce a bruise known as a hematoma. The risk of more serious complications including temporary clotting of the vein, infection of a hematoma or the surrounding tissues, or significant external blood loss is very low.

Genetic information:

Sometimes genetic information suggesting different parentage is obtained during research. We do not plan to report such findings to you.
Since some genetic variations can help to predict the future health problems of you and your relatives, this information might be of interest to health providers, life insurance companies, and others. Patterns of genetic variation also can be used by law enforcement agencies to identify a person or his/her blood relatives. Therefore, your genetic information potentially could be used in ways that could cause you or your family distress, such as by revealing that you (or a blood relative) carry a genetic disease.

Most tests done on samples in research studies are only for research purposes and do not have valuable health care use. If we find certain research data or results that would be helpful for your health care, we will contact you and/or your clinician.

If we think that there are genetic or other research test results that will be beneficial for you, we will recommend that these tests be re-done by a certified clinical laboratory. The study does not have the funding to complete such testing in a certified clinical laboratory. Consult your physician, professional genetic or other counselor. You or your insurance company will have to pay for those additional services.

For subjects seen at Children's National Hospital and some participating sites:
CAP- approved genetic information will be shared as a report from the approved clinical genetic testing laboratory with the family through ACMG guidelines for genetic counseling return of results.

This study may involve risks that are not currently foreseeable.

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 disease in people with hepato/renal fibrocystic diseases. The DNA samples will be stored de-identified for possible future studies.
There is a possibility that results from this protocol may provide important insight 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.

Genetic information in your sample: *Possible limits to individual confidentiality:*
Your privacy is very important to us and we will use many safety measures to protect your privacy. It is possible that other investigators conducting other research might request that data collected for this study be shared for further research. For examples, investigators associated with the government agency supporting this study might make this request. In this case, your name or other personal identifying information would not be revealed. However, in spite of all of the safety measures that we will use, we cannot guarantee that your identity will never become known. While neither public nor controlled-access databases developed for this project will contain information that is traditionally used to identify you, such as your name, address, telephone number or social security number, people may develop ways in the future that would allow someone to link your genetic or medical information in our databases back to you. Although your genetic information is unique to you, you do share some genetic information with your children, parents, brothers, sisters, and other blood relatives. Consequently, it may be possible that genetic information from them could be used to help identify you. Similarly, it may be possible that genetic information from you could be used to help identify them. For example, someone could compare information in our databases with information from you (or a blood relative) in another database and be able to identify you (or your blood relative). It also is possible that there could be violations to the security of the computer systems used to store the codes linking your genetic and medical information to you.

HEALTH INSURANCE PORTABILITY AND ACCOUNTABILITY ACT

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.

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 would like to store personal health information collected from you in this study in a Research Electronic Data Capture (REDCap) database for future research. The database is maintained by 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 for future analysis related to this study.

☐ Yes ☐ No _____initials

My personal health information may be stored in the above-named database for future analysis related to **Core A: The Hepato/Renal Fibrocystic Diseases Translational Resource**.

☐ Yes ☐ No _____initials

My DNA will be collected and may be tested and analyzed for genetic studies.

☐ 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 in the above-named database. 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:

  Dr. 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 payer 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)
In the United States, a 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.

The American Care Act (ACA or "Obamacare") also provides some protection against discrimination on genetic basis.

Research Subject Advocate:

This is the administration office for the Institutional Review Board, which is a group of doctors, nurses, and non-medical people who review research studies for safety and the protection of people who participate in research. You can call the Office for the Protection of Human Subjects at 301-565-8447.

Children's National has a research participant and family advocate. The advocate is here to answer your questions or concerns about taking part in this research. The advocate does not work for the doctors who are doing this research and is not paid by the researchers. The advocate is here only to help and protect you during any research.

You may contact the research advocate 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 contact the research advocate at 202-476-5586 or by email at RSA@childrensnational.org. In urgent situations the research advocate and pediatric ethics program team can be reached at the pager number: 202-259-2082.

CONSENT/AUTHORIZATION:

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.

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.

Printed Name of Participant: _____ Date: _____

Printed Name of Parent/Guardian: _____ Date: _____

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

Signature of Parent(s)/Guardian(s): _____ Date: _____

Printed Name of Individual Obtaining Consent: _____ _____

Title: _____ Signature: _____ 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 any time.

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: _____