Informed Consent and HIPAA Authorization Form

Study Title: **Capturing quality of life and priorities for clinical research in parents and patients with autosomal recessive polycystic kidney disease (PROM study)**

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You, or your child, may be eligible to take part in a research study. This form gives you important information about the study. It describes the purpose of this research study, and the risks and possible benefits of participating.

In the sections that follow, the word "we" means the study doctor and other research staff. If you are a parent or legal guardian who is giving permission for a child, please note that the word "you" refers to your child.

Study Overview

The purpose of this study is to better understand your and your child's experience with autosomal recessive polycystic kidney disease (ARPKD) in order to take a more patient-centered approach in future clinical trials.

The study has two aims: first, we want to find out which aspects of the disease are particularly important to you and your child by asking questions about developmental milestones, activity level, fatigue, pain, appetite level, high blood pressure, abnormal lab values, and maintaining kidney function.

The second goal is to find out how the disease affects the health-related quality of life of your child, yourself, and your family. We will ask you to fill out questionnaires to see whether these questions will be suitable for future ARPKD studies.

If you agree to take part, your participation will last until study questionnaires are complete and will not involve study visits. The main risks of this study are from risks of medical record review and breach of confidentiality.

You will not benefit directly from participating in this study.

If there is anything in this form you do not understand, please ask questions. Please take your time. You do not have to take part in this study if you do not want to. If you take part, you can leave the study at any time.

Please see below for additional details about the study.

How many people will take part?

40 ARPKD children, 60 of their caregivers, and 20 healthcare professionals will be recruited at Children's Hospital of Philadelphia (CHOP).

What are the study procedures?

We will ask you to answer a series of questions via an online tool. The entire process should not take longer than 1 hour. If this is too long for you, you can take a break, save your answers, and come back to finish the questions later.

The first part focuses on you and your child's priorities when it comes to ARPKD.

In the second part, we will ask you to fill out questionnaires about the health-related quality of life, strengths and challenges of your child, the impact of the disease on your family life and your own health-related quality of life.

The study team will conduct a medical record review and will ask for abdominal circumference and potentially height to be recorded by the families at home.

What will be done with my data during this study?

The research study team will enter your coded data into the ARPKD PROM database.

Will I receive any results as part of this study?

The results for this study will be done for research-puposes only and may not have valuable health care use. If we think that there are research results that will be beneficial for you, we will contact you and/or your clinician.

What are the risks of this study?

Taking part in a research study involves inconveniences and risks. The main risks of taking part in this study are discussed below.

Risks of Medical Record Review, Breach of Confidentiality, Completion of questionnaires and abdominal/height measurement.**:**

As with any study involving collection of data, there is the possibility of breach of confidentiality of data. Every precaution will be taken to secure participants' personal information to ensure confidentiality.

At the time of participation, each participant will be assigned a study identification number. This number will be used on data collection forms and in the database instead of names and other private information. A separate list will be maintained that will links each participant's name to the study identification number for future reference and communication.

Are there any benefits to taking part in this study?

There are no direct benefits to you from participating in this study. An indirect benefit would be that results from this research may provide important insight for the future care of people with these conditions.

Do you need to give your consent in order to participate?

If you decide to participate in this study, you must sign this form. A copy will be given to you to keep as a record.

What are your responsibilities?

Please consider the study time commitments and responsibilities as a research subject when making your decision about participating in this study.

What happens if you decide not to take part in this study?

Participation in this study is voluntary. Your health care provider at CHOP will continue to provide you with health care services even if you refuse to sign this form.

If you decide not to take part or if you change your mind later there will be no penalties or loss of any benefits to which you are otherwise entitled.

Can you stop your participation in the study early?

You can stop being in the study at any time. You do not have to give a reason.

Can the study doctor take you out of the study early?

The study doctor may take you off of the study if:

- Your condition worsens.
- The study is stopped.
- You cannot meet all the requirements of the study.
- New information suggests taking part in the study may not be in your best interests.

What choices do you have other than this study?

There are options for you other than this study including:

- Not participating in this study.
- You may discuss other options available to you with your doctor.

What about privacy, authorization for use of Personal Health Information (PHI) and confidentiality?

As part of this research, health information about you will be collected. This will include information from medical records, abdominal/height measurements and interviews questionnaires. Medical records are available to CHOP staff. Staff will view your records only when required as part of their job. Staff are required to keep your information private. Information that could identify you will not be shared with anyone – unless you provide your written consent, or it is required or allowed by law. We will do our best to

keep your personal information private and confidential. However, we cannot guarantee absolute confidentiality. Your personal information may be disclosed if required by law.

The results of this study may be shown at meetings and published in journals to inform other doctors and health professionals. We will keep your identity private in any publication or presentation.

Several people and organizations may review or receive your identifiable information. They will need this information to conduct the research, to assure the quality of the data, or to analyze the data or samples. These groups include:

- Members of the research team and other authorized staff at CHOP.
- People from agencies and organizations that perform independent accreditation and/or oversight of research; such as the Department of Health and Human Services, Office for Human Research Protections.

By law, CHOP is required to protect your health information. The research staff will only allow access to your health information to the groups listed above. By signing this document, you are authorizing CHOP to use and/or release your health information for this research. Some of the organizations listed above may not be required to protect your information under Federal privacy laws. If permitted by law, they may be allowed to share it with others without your permission.

The identifiable information from this study will be destroyed six years after the study is completed.

Your permission to use and share the information and data from this study will continue until the research study ends and will not expire. Researchers continue to analyze data for many years and it is not possible to know when they will be completely done.

Certificate of Confidentiality (CoC)

A Certificate of Confidentiality (CoC) issued by the NIH covers this research. A CoC helps protect your identifiable information.

A CoC protects your private information from all legal proceedings. Unless you consent, information from this research study that identifies you will not be shared outside this research.

- No one can be forced to share your identifiable information for a lawsuit.
- Your information can't be used as evidence even if there is a court subpoena.

If you consent, your information could be shared for:

- other scientific research;

The CoC does not prevent some disclosures.

- You can still share information about yourself. You can also freely discuss your involvement in this research.
- The researchers must disclose things required by law. This includes suspected child abuse and neglect, harm to self or others, or communicable diseases.

Can you change your mind about the use of personal information?

You may change your mind and withdraw your permission to use and disclose your health information at any time. To take back your permission, it is preferred that you inform the investigator in writing.

Dr. Lisa Guay-Woodford
The Children's Hospital of Philadelphia
Division of Nephrology
34th Street and Civic Center Blvd.
Philadelphia, PA 19104

In the letter, state that you changed your mind and do not want any more of your health information collected. The personal information that has been collected already will be used if necessary for the research. No new information will be collected. If you withdraw your permission to use your personal health information, you will be withdrawn from the study.

Financial Information

While you are in this study, the cost of your usual medical care – procedures, medications and doctor visits – will continue to be billed to you or your insurance.

Will there be any additional costs?

There will be no additional costs to you by taking part in this study.

Will you be paid for taking part in this study?

You will not receive any payments for taking part in this study.

Who is funding this research study?

NIH is providing funding for this study.

Please ask Dr. Lisa Guay-Woodford if you have any questions about how this study is funded.

What if you have questions about the study?

If you have questions about this study or how your data is going to be used, call the study doctor, Dr. Lisa Guay-Woodford at (267) -425-0315. You may also talk to your own doctor if you have questions or concerns.

The Institutional Review Board (IRB) at The Children's Hospital of Philadelphia has reviewed and approved this study. The IRB looks at research studies like these and makes sure research subjects' rights and welfare are protected. If you have questions about your rights or if you have a complaint, you can call the IRB Office at 215-590-2830.

What will be done with my data when this study is over?

By agreeing to participate in the study, your de-identified information will be used to :

- Expand our multi-media, web-based resource for families and their physicians affected by these conditions.

Your data will not be used for any future research after this study is complete.

Consent to Take Part in this Research Study

The research study and consent form have been explained to you by:

Person Obtaining Consent	Signature of Person Obtaining Consent
	Date

By signing this form, you are indicating that you have had your questions answered, you agree to take part in this research study and you are legally authorized to consent to your child's participation. **NOTE:** *A foster parent is not legally authorized to consent for a foster child's participation.*

Name of Subject	
Signature of Subject (18 years or older)	Date
Name of Authorized Representative (if different than subject)	Relation to subject: ☐ Parent ☐ Legal Guardian
Signature of Authorized Representative	Date

Child Assent to Take Part in this Research Study

For children capable of providing assent:

I have explained this study and the procedures involved to _____ in terms they could understand and that they freely assented to take part in this study.

_____

Person Obtaining Assent

_____ _____

Signature of Person Obtaining Assent Date

This study has been explained to me and I agree to take part.

_____ _____

Signature of Subject (optional) Date