Patient information and consent form
for participation in "ADPedKD"

An international multicenter study on
Autosomal Dominant Polycystic Kidney Disease (ADPKD)

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Dear parent,

Your child is diagnosed / followed with "Autosomal Dominant Polycystic Kidney Disease" (ADPKD). Therefore, he/she is asked to participate in an international ADPKD patient registry, which is called ADPedKD. With this document, we want to inform you about the aim of this study and what will be expected from your child when participating it. Take your time to carefully read this document. If you have any questions, please asks them. Your treating physician and the investigators will be happy to answer them.

This study was approved by the ethics committee (Commissie Medische Ethiek van de Universitaire Ziekenhuizen Leuven). However, this approval should not be the reason of participating in this study.

What is known on ADPKD?
Autosomal Dominant Polycystic Kidney Disease (ADPKD) is the most common monogenic cause of kidney failure, affecting more than 1 in 400 to 1000 live births. ADPKD arises as a consequence of mutations in *PKD1* gene, accounting for 85% of cases, or *PKD2* gene, accounting for 15% of cases. The disease is characterized by the progressive development and enlargement of cysts in the kidneys, often leading to kidney failure. 50% of patients need renal replacement therapy (dialysis and/or transplantation) by the age of 60 years. Cyst formation can occur in other organs as well, mostly in the liver. Moreover, patients with ADPKD have an higher risk of cardio-vascular complications and it represent the most frequent cause of death.
Currently, there is no curative treatment apart from Tolvaptan in selected patients. Different drugs are tested in animal models or/and in adult ADPKD patients, however, the results are very disappointing and the drugs have a lot of side effects. The question is if these products could be more efficient when given at a younger age, when there is less kidney damage. Indeed, the disease starts at an early age, even in utero, and children might present with symptoms such as hematuria, urinary tract infections, pain. Moreover, 23% of ADPKD children have abnormal levels of protein in their urine and 5 to 44% have a high blood pressure.

What is the aim of the registry?
The aim of ADPedKD is to collect data of ADPKD patients from their childhood or even prenatally if available. What are the presentation features and especially the age and the mode of diagnosis. Was the disease diagnosed because the patient had symptoms or because the patient was screened? How is patient follow-up organized, how are patients treated and what is the result of these treatments? We want to have a better understanding on factors influencing disease progression such as hypertension and proteinuria. In the registry, we would like to include information on the initial presentation of the disease, birth data, genetic analysis and follow-up of the disease.
This study is supported by the European society for pediatric nephrology (ESPN).

How to participate this study?
Only medical staff can transmit data to ADPedKD, you nor your child can do this. All ADPKD patients with a follow-up from childhood, can participate in the registry. Someone of your child's medical team can only enter your data after you and your child signed the consent forms.

Who can't participate this registry?
Patients with renal cysts due another disease then ADPKD cannot participate this registry.

Which data will be collected in this registry?
After you and your child signed the consent forms, someone of your child's medical team can enter your child's data (symptoms, radiology results, laboratory results, familial history, genetic analysis etc.) into the database, which is accessible on the web. Names will be replaced by codes, part of which is standing for the participating center, and part of it is your child's personal code. Only the principal investigator or an authorized member of the research team will be able to link his/her data with his/her name, via a list, which is not accessible to the coordinators of the registry. Every time your child has a control appointment, new data will be entered in the database. In case of substantial changes in the scientific aim of ADPedKD, you will receive additional information on this from your treating physician.
The coded data might be used in the future in international collaborations.

Are there additional investigations or analyses, only for ADPedKD?
No. Someone of your child's medical team will enter your child's data in the registry, obtained only from his/her regular appointments. No additional consultations, investigations or analyses will be performed.

What are the risks of ADPedKD ?
ADPedKD is a registry study. Only existing data will be kept in the database. Your child will not be taken care of differently when participating, nor when refusing to participate.

Insurance.
The study originator (UZ Leuven) is liable for the harm, (in)directly related to the registry, done to the participator. UZ Leuven has an insurance, as stated in article 29 of the 'Wet van 7 mei 2004', regarding human experiments.

Will your child or his/her doctor experience advantages from participating this study?
There are no immediate advantages for your child. However, all ADPKD patients could benefit

from the results of this study. Participating this study does not imply any costs nor any compensation.

How will the data be treated?
All data will be handled confidentially. Someone of your child's medical team will enter the data in a database, accessible online and secured with a password. The webpage is SSL (Secure Sockets Layer) secured. The database will be put on a server, managed in Köln, Germany. No one is allowed to talk about the data with a third party.
At the end of the study, we will publish the results in a scientific journal. In this article(s), it won't be possible to identify any of the participants.
Data will be saved during an indefinite period of time.

Does my child has to participate?
No. Participating this registry is voluntary. You can withdraw your child from the study at any moment and without the need to explain why. If so, this will have no further consequences for your child's medical care.

Who is your contact person?
Your contact person for this study is the treating physician of your child:

Name:
Tel/email:

Any other questions?
Do not hesitate to contact one of the investigators in case of any further questions:

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Prof. Dr. Djalila Mekahli
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Dr. Max Christoph Liebau,
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Consent form ADPedKD

I am well informed regarding this registry. I have read this information thoroughly. I could ask all my questions regarding this research and my questions were answered satisfactorily. I reflected well regarding the participation of my child. I know I can withdraw my consent at any moment without any justification.

I declare I want my child to participate in this research:

Child's name, first name: …….

Child's date of birth: …….

Parent/ Legal guardian:

Name, First Name :…….

Date of Birth :…….

Signature :…….

Date :……..

I hereby certify that the person mentioned above was well informed, both oral and written, regarding the study as mentioned. I declare that premature termination of participation by this person's child will not have any impact on the medical care the child will be provided with.

Name, First Name :…….

Function :…….

Signature :…….

Date :……..