

## Patient information and declaration of consent for participation in the NEOCYST registry

Patient age between 6 – 17

**Registry leadership:** Univ.-Prof. Dr. med. Martin Konrad



**Clinic for Pediatric and Adolescent Medicine, General Pediatrics**

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## Patient information for participation in the NEOCYST registry

Network for early onset cystic kidney disease

Dear \_\_\_\_\_,

You are in regular contact with your doctors because you are affected by a special kidney disease. The disease is very rare and an exact cause is still unknown. That is the reason why we still do not have any medication to cure your disease.

We would like to research your disease further. This is why we are asking you to participate in a study in order for us to be able to learn more about your disease.

The study is called NEOCYST and below you will find some information about it.

Together with your parents you can decide if you would like to take part in the study. There won't be any disadvantages for you in case you decline participation.

If you decide to participate you are free to withdraw from the registry at any point in time without consequences.

### What do we know about cystic kidney diseases?

Cystic kidney diseases are congenital diseases which mainly affect the kidneys. Sometimes other organs are affected as well.

They are called „cystic“, because of the appearance of little, fluid-filled bubbles (=cysts) within the kidneys. There are various forms of kidney disease which slightly differ from each other. But they all have affected kidneys in common. In Germany only a few (about 300-400) children and adults suffer from those diseases.

A lot of times the same disease affects children in different ways. Some affected children are required to see doctors regular and take medication from an early age. Others may even need a new kidney.

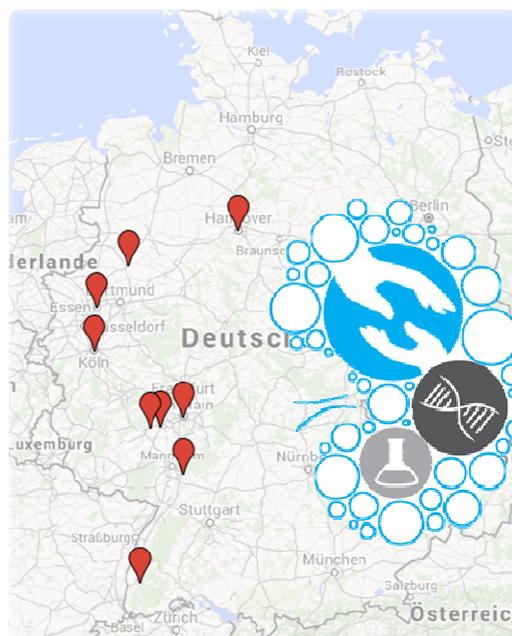
Yet some affected children have very little problems. The reason why these cystic kidney diseases differ so much is still unknown.

Tremendous progress has been made researching cystic kidney disease over the past 15 years. Scientists have detected defects (mutations) in nearly 100 genes in our genetic make-up which can be the cause for developing cystic kidney disease.

This has helped us to understand the disease much better. However we still have lots of questions and do not fully know how the disease develops or progresses. This is why we require samples and data from as many affected patients as possible.

### What is NEOCYST?

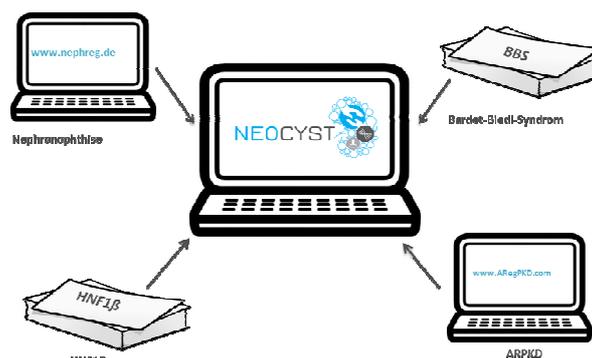
NEOCYST is a multicenter network of several clinicians and scientists within Germany who are researching cystic kidney disease. Your doctor is going to work closely with these centers.



### REGISTRY

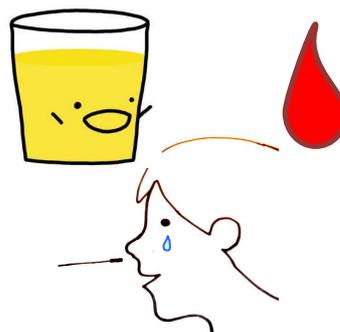
Information about your disease, e.g. your blood levels, ultrasound data and data about the progression of your disease will be collected and analyzed in a registry. This is going to help us to understand similarities, differences and the disease progression much better.

The data will be anonymized, which means that your personal data (name, date of birth etc.) won't be saved.



## **BIOLOGICAL SAMPLES**

We would like to explore the cause of the disease. Therefore we kindly ask you to provide a few milliliters of blood, urine and nasal swab samples to us.



### **This is what we aim to discover:**

- Do different diseases share a common cause?
- What kinds of genes are causing what kind of disease?
- Do markers in blood or urine exist, that can help predict the course of disease?
- What kind of therapy is providing the best outcome?
- How can we develop new therapies?

### **How does the registry work? What do I have to do if I choose to participate in the study?**

You are going to see your doctor/pediatric nephrologist as usual. No additional visits are required if you choose to participate in the study.

Firstly, we are asking for your consent to collect the data we already have to analyze your disease pattern. Secondly, we would like to collect a few different samples from you. You are free to decide if you would like to provide us with all of the different samples we are asking for or only certain ones.

#### **Registry:**

- 1) Information about your disease, like blood- and urine test results that have already been collected in the past, will be stored in a big data file. We aim to compare and analyze your data with the data from other patients.

#### **Samples:**



- 2) Blood: While a routine blood collection is done by your doctor we would like to collect additional blood vials for further testing.

- 3) Urine: When you provide urine to your doctor, we would like to take 100 ml of this sample to analyze it further.





- 4) Nasal swabs: We are using a little brush to swab the inside of your nostrils. The procedure is not invasive. It feels like getting water into your nose. For a tiny moment you may feel discomfort and you may get watery eyes. Very rarely you may experience nose bleeding. Those nasal swabs are providing us with lots of valuable information and are extremely important for our research. We will be able to analyze special structures called cilia, which are found on the outside of your cells.

### **What happens to my data?**

Data concerning you and your disease progression will be collected and electronically stored. The data will be further analyzed in a research center. All data will be anonymized, which means that your name, address and date of birth will be changed in a way that nobody can trace it back to you (E.g. Mickey Mouse = Münster 97). Only your doctor will be able to identify these data as yours, you will remain “anonymous”.

If you decide to withdraw from the study at any point in time, no new data will be saved in the registry. The existing data will remain in the registry unless you are telling us to delete those. Your parents have been provided with further information.

### **What happens to my samples?**

Your samples will be analyzed first. Afterwards they will be stored in a big laboratory in Hannover, a so-called “biobank”. The samples are being stored anonymized. If you decide to withdraw from the study at any point in time you have the option for your stored samples to be destroyed.

### **What are the advantages of participation?**

The participation in this study will most likely not lead to any personal gain. However, your participation will provide valuable new insights to cystic kidney diseases. The results from this study may lead to better patient care in the future.

### **What are the disadvantages of participation?**

There will be no disadvantages or unnecessary procedures. Blood and urine samples are being analyzed at your usual visits in any case. Any additional testing will only be done with your consent.

### **Do I have disadvantages, if I don't participate?**

No! You won't experience any disadvantages if you decline participation.

### **Can I withdraw from the study at a later point in time?**

Yes! Any participation within the NEOCYST study is voluntary. You are able to give your consent to all parts of the study or only to subprojects. You are free to withdraw from the study at any point in time. There will be no consequences in regards to your treatment.

**Who is my contact person for further questions?**

Your primary contact person is your attending doctor.

Local doctor:

Name: \_\_\_\_\_

Institution: \_\_\_\_\_

Phone: \_\_\_\_\_

**Further questions?**

Feel free to contact us with any questions:

**Coordinating office** of NEOCYST:

Pediatric nephrology and pediatric dialysis

Waldeyerstr. 22

48149 Münster,

Phone: 0251 / 8356213,

**Declaration of consent for NEOCYST**  
(Network for Early Onset CYSTic Kidney Diseases)

You can only participate in this study if you give your consent. If you decide to participate we are asking you to sign this form. With this signature you confirm that you wish to participate in the NEOCYST study voluntarily, that all your questions were answered and that you had enough time to think about your participation. You are free to withdraw from the study at any point in time. There will be no consequences in regards to your treatment. Upon request all collected data will be deleted and your samples will be destroyed.

I agree that my anonymized data will be collected, stored and analyzed within the NEOCYST registry and may be published.

Yes       No

I am willing to provide the following biological samples for research purposes in context with the NEOCYST registry:

Urine       Blood       Nasal swab

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Name of adolescent patient in printed letters

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Place, Date

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Signature adolescent

I held the educational conversation with the patient and obtained his consent to participate in the study. I am certain that the patient has comprehensive understanding of the study, no further questions and that he agrees to participate.

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Name of attending doctor in printed letters

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Place, Date

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Signature doctor