

How would you describe the treatment and support you get from your medical doctor?

Since about 10 years there is the opportunity to visit the **one-stop clinic in Rosenheim** in Bavaria where **adults and children** get comprehensive advice from a team of clinicians covering all relevant aspects of Cystinosis.

There are also pediatric nephrologists for children in different **University clinics** all over Germany available. The exchange and **collaboration** between these clinics and the clinic in Rosenheim **is crucial**.

A **specialized company** (Medipolis) that provides an assessment of negative interactions of different medications, insights on how to minimize side-effects and provides specific blistering of medications to **potentially improve compliance** is available.

Local doctors have only **limited knowledge** of Cystinosis and its systemic nature which is sometimes a challenge

Are all the medicines in your country available and affordable?

- **Procysbi and Cystagon** are both **available**
 - some people take Cystagon during the day and Procysbi during the night
- **Cystadrops** and **pharmacy made eyedrops** are **available**
 - two pharmacies with specific knowledge on making eyedrops
- **Growth hormone** and medication to treat **Fanconi syndrome** are **available**
- All medications that need to be subscribed are **generally reimbursed**
 - a limited amount of co-payment can occur
- OTC-**supplements** are **not reimbursed** (eg Lysin)

What should be improved in the treatment of cystinosis in your country?

- Provide **newborn screening** for everybody as a standard
- Establish a reliable and easy **cystine level measurement**
- Establish a **standard protocol for treatment** of Cystinosis
- Identify ways to **improve adherence**
- **Easy transition** from child to adult care
 - Strong separation of pediatrics and adult medicine in the German health care system. Cystinosis specialist are only pediatricians
 - Especially adult nephrologists outside the cystinosis center have often very little knowledge about cystinosis and are sometimes not willing/do not have the time to gain specialized knowledge if they have a cystinosis patient
 - Adults sometimes face difficulties to find a doctor that subscribes expensive medication due to specific budget restrictions for local doctors as well as getting reimbursement for supplements

Tell what you think is unique on living with cystinosis in your country? How it is to live with cystinosis in your county?

The combination of:

- very good access to generally all treatments
- a one stop clinic with comprehensive expertise in Rosenheim (reference center of the European Rare Kidney Disease Reference Network)
- a strong Cystinosis community with big annual family gathering plus age group related activities for teenagers and adults
- online exchange to get supportive feedback in specific situation and general exchange
- increasing activities by the Cystinosis foundation to fund and support research and further improve the treatment options

What do you expect for the future?

- Ensure know how transfer to next generation of researchers and clinicians
- Continue the very good treatment especially for children
- Further improve the support for adults
- Standardize treatment