

## CASE STUDY 3: Cross-border enrollment

Crossing borders and language barriers to access treatment for an ultra-rare disease

### A phase II/III study in Primary Hyperoxaluria (PH)

- 24 patients with PH, children (>4 years) and adults
- 9 study sites (UK, Germany, Spain, Belgium, USA, Tunisia)
- Full services provided by Ergomed

### Challenge & Mitigations

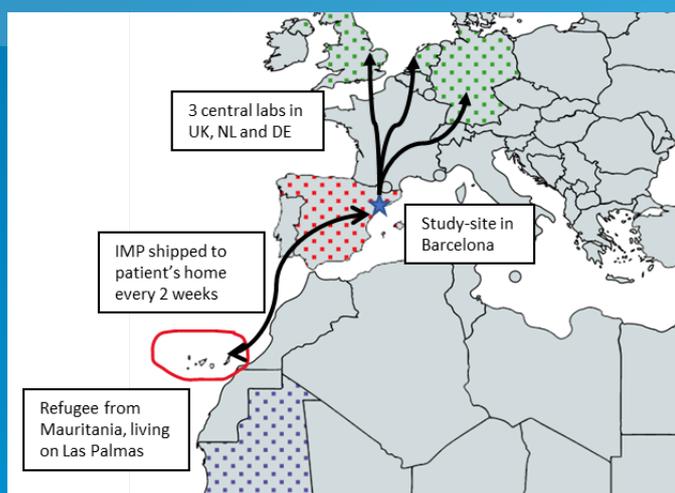
**Case:** A 5-year old refugee child from North Africa was diagnosed with PH on the Canary Islands and was referred to a study-site in Barcelona.

**Challenge:** In this study, the study medication had to be shipped directly to the patient’s home every 2 weeks. In addition, the patient needed to collect 24-hour urine and stool samples at home on several occasions these samples had to be shipped to a central lab.

**Mitigations:** In close collaboration with the study site and our qualified vendors, we made the following arrangements:

- Travel and accommodation of patient and his parents (door-to-door)
- ICF and other patient-facing documents translated into Arabic
- On-site interpreter at each visit to bridge the language-barrier
- Extended stay in Barcelona to facilitate urine- and stool-collection at site
- Premier courier to ship IMP direct to patient’s home every 2 weeks

The patient completed the pivotal study and also continued in the long-term extension study.



### Primary Hyperoxaluria

Primary Hyperoxaluria (PH) is an ultra-rare, inherited disease in which excessive amounts of oxalate are produced by the liver. The condition is characterized by recurrent kidney and bladder stones and often results in end stage renal disease (ESRD), which is a life-threatening condition that prevents the kidneys from filtering fluids and waste products from the body effectively.



### Conclusions

Ergomed treats any rare-disease patient like gold-dust and will do anything in our power to enroll a rare-disease patient into a trial or to try to prevent a patient from dropping out or stopping their trial participation.

Ergomed likes to think out-of-the-box in order to find solutions for logistical challenges.

Ergomed can utilize its Orphan Toolkit to decrease the patient’s and family’s burden related to their clinical trial participation.

Our dedicated team is looking forward to supporting you with your trial!

Contact us today at [info@ergomedplc.com](mailto:info@ergomedplc.com)