

CASE STUDY 2: The DevelopAKUre project

Exceeding the recruitment target in an ultra-rare disease!

How PSR enrolled 140 patients with Alkaptonuria in only 9 months

The trial: a phase II/III repurposing study in Alkaptonuria (AKU)

- 140 adult patients with AKU
- 3 study sites (UK, France, Slovakia)
- Full services provided by PSR
- Part of public-private partnership (European FP7-grant) to promote repurposing of drugs for rare diseases

Challenge & Mitigations

Case: The funding for this project (European FP7-grant) only allowed a 5-year project, while we needed 4 years of follow-up in the study.

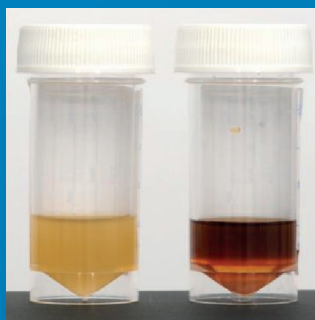
Challenge: With only 350 known AKU-patients across Europe, we needed to enroll **40% of all patients** into the study in less than 1 year. We only had 3 study sites.

Mitigations: In very close collaboration with the leading European patient organization (the AKU Society), we set-up an online trial-awareness campaign (website and social media), we organized webinars for patients (hosted by the Key Opinion Leader), and we did a roadshow with presentations at different European hospitals and at local patient meetings. In parallel, we set-up a system for cross-border enrollment and travel-support to facilitate patients from other countries joining the trial.

In the end, we managed to complete recruitment in only **9 months!** We enrolled patients from **16** different countries, including a group of 20 patients from Jordan who participated at the study-site in Slovakia.

Alkaptonuria (AKU), also known as Black Bone Disease

AKU is an ultra-rare genetic disorder characterized by accumulation of homogentisic acid (HGA). This accumulation of HGA causes black pigmentation, which leads to black urine and damage to the body, particularly the joints. Patients develop early-onset arthritis and, if left untreated, AKU may cause heart problems, kidney stones, and prostate stones.



Conclusions

PSR recognizes the importance of a very close collaboration with the patient community, in order to make the study more patient-friendly and to enhance recruitment and retention.

PSR can implement a cross-border enrollment process, to enable patients to participate at a study-site in another country.

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

PSR can utilize its Orphan Toolkit to decrease the patient's and family's burden related to clinical trial participation.

Our dedicated team is looking forward to supporting you with your trial! Contact us today at info@psr-group.com

- 1 At Liverpool, UK**
The Royal Liverpool University Hospital, UK, is home to the National AKU Centre, and some of the world's leading experts on the disease.
Lead clinician - Prof L. Ranganath
- 2 At Piestany, Slovakia**
The National Institute of Rheumatic Disease, Slovakia, has been studying AKU for 60 years, and cares for the world's largest community of AKU patients.
Lead clinician - Prof Jozef Rovinsky
- 3 At Paris, France**
The Hôpital Necker, France, houses a national metabolic centre that treats several AKU patients and uses nitisinone for tyrosinaemia patients.
Lead clinician - Prof Pascale de Lonlay