Patients Involved – DevelopAKUre for AKU

Introduction

An example of public-private partnership from diverse international groups in industry (Sobi), patient organisations (AKU Society and ALCAP), hospitals (Royal Liverpool, Hospital Necker, and National Institute of Rheumatic Diseases), Academia (Universities of Liverpool and Siena, Institute of Molecular Physiology and Genetics) and Small and Medium Enterprises –SME– (Nordic Bioscience, PSR and Cudos) to cure alkaptonuria (AKU).



When does it happen? – Phase II-III

Description of the case

DevelopAKUre is a series of three clinical studies, funded by the European Commission's FP7 programme. They will investigate the medicine nitisinone to find evidence if it works to treat alkaptonuria (AKU). The project involves a dose-response study (SONIA1), an efficacy study (SONIA2) to compare no-treatment to treatment, and a cross-sectional study (SOFIA) to determine the best age to begin treatment. Studies take place at three sites across Europe (UK, France and Slovakia).

DevelopAKUre is patient-led, with the AKU Society as a lead partner, ensuring patient views were considered at planning stages, and throughout the ongoing studies. The AKU Society now leads on patient recruitment and support, developing patient information documents and promoting patient retention. The AKU Society raised additional funding from a crowdfunding campaign hosted on Indiegogo in order to be able to provide a high level of patient care throughout DevelopAKUre.

The AKU Society is also coordinating dissemination for the project, ensuring project activities are shared with patients and the public.

More information at https://akusociety.org/aku-clinical-trials/

Type(s) of patient (advocates) involved

- Patients with personal disease experience.
- Expert patients / patient advocates with good expertise on disease, but little R&D experience.
- Expert patient / patient advocate with good expertise on disease and good R&D experience.

Benefits of patient involvement

DevelopAKUre is unique: a truly patient-led clinical trial. We believe it could create a new paradigm in medical research, helping to promote patient involvement in planning and running of clinical trials, and introducing the idea of involving patient groups as active partners who can lead in tasks such as patient recruitment and patient retention.

The involvement of patient groups in planning the clinical trials has improved the patient experience from advocating for a more streamlined expenses reimbursement process and funding

carers’ travel to making invasive tests optional and reducing the number of visits to test centres.

Challenges and barriers

1. Funding: We spent several years attempting to raise funding for clinical research. We eventually applied and gained funding of €6 million from the European Commission. As medical research is so expensive, we would find it unlikely national bodies could provide large enough grants for research into rare diseases.
2. Legal / Ethics: The AKU Society are leading on patient information, which requires professional advice on legal and ethical concerns. We rely on other partners (PSR) and review boards for this input.
3. Language: The biggest barrier for patient recruitment in Europe has been language issues. Translations have added a significant cost to production of patient information.
4. Regulatory issues: Resolved through external advice mainly our small and medium enterprises partners, and scientific advice from the EMA.

Learnings

We hope to see more patient-led research, where patients are involved in planning and running studies, and patient groups are given an active role. For us, patient groups are the most involved in understanding how the disease affects patients and so make a good choice for partners in research. Additional training (such as from EUPATI) in working with pharma and academics, the medicine development process and ethical / legal requirements of trials is needed. Many patient groups need encouragement to show their contribution is valued and important to medical research.

Attachments

- Case-report-DevelopAKure-v1-EN

  Size: 485,995 bytes, Format: .pdf

  An infographic describing a patients involved case report of a public and private partnership to develop a cure for alkaptonuria (AKU) led by patients.