DevelopAKUre

About DevelopAKUre

DevelopAKure is an EC-funded project to develop nitisinone for the treatment of Alkaptonuria that is being co-ordinated by the Royal Liverpool University Hospital. Dr Lakshminarayan Ranganath is the co-ordinator and Chief Investigator for DevelopAKUre.

Objective of DevelopAKUre

The main objective of this proposal is to study the efficacy and safety of an orphan designated drug, nitisinone, in order to obtain its marketing authorisation for the treatment of patients with Alkaptonuria (AKU), a rare and debilitating Mendelian disease for which there is no licensed treatment. A second objective is to improve the current knowledge of the natural history of AKU, especially in young people. Thanks to our existing successful non-clinical and clinical research (cell and tissue models, animal models, natural history studies), we are now in a position to complete the clinical development of nitisinone for AKU.

Funding

The EC is providing £4.8 million in addition to the 3.2 million co-financing from a European consortium towards DevelopAKUre.

Duration of DevelopAKUre

66 months. The start date was on 1st November 2012 and will finish on 30th April 2018.

Studies in DevelopAKUre

This will involve

- a dose-response study (SONIA 1)
- an efficacy study (SONIA 2) to demonstrate improved clinical parameters and
- a cross-sectional study (SOFIA) in children and young adults to provide information on the age at which it might be most beneficial to begin treatment

Outcomes of DevelopAKUre

The results of DevelopAKUre, if positive, will allow us to make a European Marketing Authorisation Application for nitisinone for the treatment of AKU, thereby contributing to the goal of the International Rare Disease Research Consortium of 200 new therapies by 2020.

Partners in DevelopAKUre

Our consortium is unique and robust. Its partners have worked together for several years already. They include

- the Royal Liverpool University Hospital as the Coordinator
- the AKU Society (UK) and ALCAP (France) patient groups for communications/dissemination and help with patient recruitment
- three SMEs (Nordic Biosciences (Denmark) for biomarker analysis
- PSR (Netherlands) for clinical trial coordination and Cudos (Netherlands) for medical monitoring)
- a mid-sized pharma company Sobi (Swedish Orphan Biovitrum International) supplying the drug and regulatory advice
- three universities (Liverpool, Siena and the Slovak Institute of Molecular Physiology and Genetics) for the analysis and interpretation of data, and
- three clinical trial centres (Liverpool, Hôpital Necker (Paris), National Institute of Rheumatic Diseases (Slovakia)) to recruit sufficient participants

This project can only be achieved through a Europe-wide collaboration, as it allows us to recruit enough patients for an adequately powered trial and gives us access to the elite among AKU researchers.