

New registry for rare diabetes

EURO-WABB is a new project aiming to establish a registry for Alström, Wolfram and Bardet Biedl syndromes and improve diagnostics, care, knowledge and treatment.

Within the EU Health Programme 2008-2013 and its call for promoting health through the creation of new registers for rare diseases, [EURO-WABB](#) is supported by the EU Directorate General for Health and Consumers (DG Sanco). The EURO-WABB project began on 1 January 2011 and will run for 3 years.



“The general objective of EURO-Rare Diabetes is to support efficient diagnosis, treatment and research for the rare genetic

diseases [Wolfram](#), [Alström](#) and [Bardet Biedl](#) (WABB) syndromes in Europe,” says Professor Timothy Barrett (Birmingham University, UK) leader of the project. Currently, there are 13 participating members from 10 countries.



The idea for the EURO-Rare Diabetes project came from Nolwenn Jaffre, President of the [French Wolfram Association](#), who gathered Wolfram syndrome researchers from around the world in Paris in October 2009. The final application included researchers in Alström syndrome, Bardet Biedl syndrome and [Alström Syndrome-UK](#) as partners. Alström Syndrome-UK is a charity dedicated to Alström patients and those caring for them.

EURO-Rare Diabetes is divided into six components called [Work Packages](#) (Coordination, Dissemination, Evaluation, Core Datasets and Pathways, Genetics, Virtual Registry and Information Environment) and Kay Parkinson of Alström Syndrome-UK is one of the partners in charge of Registry and Information Environment. “This is the largest European project in which our charity has ever been involved. Most of the time we are invisible and being involved in EURO-Rare Diabetes raises awareness of the charity and the syndrome. We are seeing benefits already, and in 2011, the charity’s role is to provide stakeholder analysis, and I would be pleased to hear from anyone with an interest. We will develop a dissemination plan and a report on the learning needs of health professionals,” declares Kay Parkinson.

Rare Diabetes Syndromes are a group of rare, inherited disorders linked by intolerance of the body to glucose, and though the register is mainly directed towards the three WABB syndromes, its scope includes some other rare diabetes syndromes. Long-term studies on these syndromes are desperately needed to understand their natural history, relate genetic diagnosis to predicting outcomes, establish a basis for evidence-based management, and to develop new treatments. “There are to date no orphan drug treatments available, nor access to well characterised cohorts of patients. The lack of specific health policies for these diseases and the scarcity of expertise, translate into delayed diagnosis and difficult access to care. There are almost no multidisciplinary teams of experts in these diseases. Genetic testing centres are concentrated in a few member states and there is unequal patient access to testing across the EU, America and Japan,” states Professor Timothy Barrett.

The Registry will tackle all these problems by establishing the natural history of the three diseases; assessing clinical effectiveness of management and quality of care; providing a database of anonymous patient data for recruitment into treatment studies; establishing genotype-phenotype correlations. Professor Barrett and the other participants are keen to hear from health professionals, inside and outside of the EU, caring for patients with one of these syndromes, as well as affected patients and family members. “In practice this means we will use questionnaires and focus groups of health professionals, to find out what support they need to take part; then decide on the core dataset for the web based registry, to make it easy for health professionals to use. We will create a rare disease gene chip to identify all the known mutations quickly and make it available across Europe. We will also do questionnaires and focus groups for patients and health professionals to find

out their learning and information needs; write education material and patient information on Alström and the other diseases; and finally use the registry to support ‘meet the expert’ platforms, and advice for families,” declares Professor Barrett.

Increasing knowledge of Alström, Bardet Biedl and Wolfram syndromes, supporting new research by allowing controlled access to investigators for epidemiological, clinical, genetic and interventional studies, advocating on behalf of families for improved quality of services both at national and European levels: the objectives of EURO-Rare Diabetes are ambitious and eagerly awaited by rare diabetes patients.

Read more:

[The EURO-WABB project](#)

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