Developing a European registry for Rare Anaemias

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To understand the real burden of disease is an important issue in public health and this is particularly so in the field of rare diseases [RD]. Registries and databases are essential tools for gathering information which help to define the epidemiology, geomap, clinical outcomes and natural history of these rare conditions. Such information will help to improve quality of care and to plan services as well as to assist in research projects including clinical trials and the recruitment of volunteer patients. EU policies concerning reference centres, networking and cross-border health, make the development of registries at healthcare facility, national and European level imperative tools to facilitate the implementation of these policies.

Enerca, as the European forum for rare anaemias, has undertaken to develop a registry for this group of hereditary disorders. Enerca, in this task, has followed guidelines already promoted by European rare disease organisations and projects. A registry is understood to be a collection of standardised data and information concerning patients with a common disorder. Standardisation has been studied by EURORDIS and by projects such as EPIRARE in the European setting but also in the North American setting and internationally by the IRDiRC, and there has been general agreement on the general content of a rare disease registry. In a joint EURORDIS-NORD-CORD declaration, 10 key principles for the development of RD registries were defined. These include the adoption of a minimum set of common data elements. This set of elements has been agreed by several projects including EPIRARE, EUCERD, IRDiRC and epSOS.

In developing the rare anaemias registry, Enerca has complied with these directives and has studied the current state of the art for standards and legal issues which concern data ownership and confidentiality, incorporating the necessary safeguards in the electronic registry. The Enerca registry is being developed, for the primary aim of epidemiological surveillance and the secondary aim of facilitating clinical research, and includes the following fields, arranged in a modular manner:

- A minimal dataset based on the needs of networking (cross border health) as well as the needs of collecting epidemiological data on rare anaemias
- Emphasis on the diagnosis and the accuracy of the diagnosis
- Patient outcome measures in order to assess the effectiveness of the service

In order to gather data from centres across Europe the registry is designed for interoperability but also it allows countries or centres who cannot, by local legislation, share individual patient data, to contribute collective or metadata. The data collected will not only support the functions described above but will also support a future Pan-European Reference Network for rare haematological disorders.