



5th Workshop

Eurordis Round Table of Companies

*" Rare Disease Patient Registries: an Essential Tool
in the Development of Therapies? "*

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Concept Paper

In the Rare Disease field more than in any other therapeutic area, the lack of basic knowledge on the natural history and pathophysiology of the disease hampers the development of any form of therapy or care.

Thus, effective tools such as registries are needed to facilitate a more rapid increase of the knowledge necessary for progress toward new therapeutic avenues. Because of their intrinsic nature, patient registries offer real-time up-to-date information about the epidemiology of a specific disease: the age breakdown of a patient population, the prevalence of disease-specific related complications, treatments currently used, genetic mutations, etc.

The morning session of our 5th ERTC workshop will focus on the general and fundamental aspects linked to the creation, development, management and use of national/international registries, including an overview of the current European legal framework.

A number of ethical concerns are also raised by the use of patient registries, such as the level of informed consent, protection and use of the data, property of the registry, the data and of all possible outcomes. These will also be discussed during the meeting.

During the afternoon session, we will see to which extent could a tool like a patient registry be important for the clinical development of an orphan medicinal product prior to the MA



application, including the designation process. Such an instrument, in fact, can be beneficial all along the life cycle of a medicinal product - from its identification for a specific indication to the compulsory follow-up of pharmacovigilance.

Furthermore, the experience of patient organisations, sponsors and academics in this field will also be presented. Several rare disease patient organisations have long realised that even the establishment of a sample data base restricted to their members is the crucial point from which to start understanding their disease and collecting the minimum knowledge necessary to imagine any scientific/ therapeutic project. Moreover, at the time of a clinical study, a registry allows for a more rapid and effective collaboration between the patient organisation and the sponsor which finally accelerates and improves the quality of the trials.

For this workshop, we have invited more patient representatives with relevant experience, as well as national, European and American competent authorities. During the meeting, each interested party is invited to draw the attention of the audience on his own vision thus offering ideas for improvement in the rare disease registries area toward the definition of good practices in this area.