EURORDIS
European Organisation for Rare Diseases

4th Workshop

Eurordis Round Table of Companies

"Common Drugs for Common Needs: the EU vs. US approach to Orphan Medicinal Product (OMP) Development"

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Concept Paper

Since the enactment of the U.S Orphan Drug Act of 1983, more than 1,500 medicinal products have been designated as potential orphan drugs, 288 of which have since been granted marketing authorisation in the USA.

The European Union adopted a similar regulation in 1999 to encourage the development of orphan drugs in its territory. Since its implementation in 2000, 360 European orphan designations have been granted, 25 of which have since received a centralised marketing approval in the EU.

As all orphan legislation in the world, the American and the European ones aim to provide incentives for the development of OMPs, either by supporting the development phase, or by providing financial incentives for OMPs after the marketing authorisation.

The adoption of similar legislative measures for orphan drugs across the world partially compensates for the low prevalence of the indications concerned as it creates a large potential market with similar key economic incentives for industry.

Patient organisations hope that this "economy of scale" will benefit the patient community at large, on both sides of the Atlantic, and that it will finally result in earlier availability of many OMPs for patients affected by rare diseases.

By gathering the different interested parties at this workshop, Eurordis plans to highlight the factors which really encourage the development of OMPs, and those which hamper it, both in the USA and in the EU.

On the larger basis provided by the high number of drugs designated in the USA, we can establish a model of dynamic development (statistics), which defines regulating factors related both to the competent authorities and to industry. The influence of the type of drug, concerned pathologies, type of company, as well as the possible development in time, can thus be explored. The same model can then be tested on the European data.

Furthermore, it is interesting, not only to compare the different regulations, but also to gain insight into the industry perspective, whether it has already developed orphan drugs in the concerned territories, or not. Learning about industry motivation and potential reluctance to deal with the Agencies in the EU and the USA is a key element in improving large-scale availability of OMPs:

- Are the criteria for designation and incentives too different in the USA and EU to justify parallel development?
- Are requirements for clinical development too demanding in one of the territories? US vs. EU ?
- Is the EU market too complex or requires too much effort for a relatively small number of patients?
- Etc.

Orphan drugs which have been developed both in Europe and in the USA go through a variety of strategies, from simultaneous bilateral development -including clinical studies on both continents- to delayed transfer after a period of commercialisation in one of the territories. The consequences of these different options for the patients and for industry will be discussed and concrete examples presented.