6th Workshop
Eurordis Round Table of Companies

"Do Rare Disease Patients Have Real Access to Orphan Drugs in Europe?"

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Concept Paper

Since the establishment of the Committee for Orphan Medicinal Products (COMP) at the EMEA in April 2000, 35 medicinal products have received a European centralised marketing authorisation- for a total of 42 indications if we consider the different authorised indications for a same medicinal product (Glivec and Sutent).

Among these, 25 products were granted an EC marketing authorisation (MA) more than one year ago, but how many of these products are really available today for rare disease patients in Europe?

Despite the increasing number of authorised Orphan Medicinal Products (OMP), many European patients still do not have access to the medicines they need. This has been the disappointing result of the research that Eurordis conducted through 3 different surveys on OMP availability across Europe (2001, 2003 and 2004), with the direct collaboration of MA holders. Hopefully, as a result of our past studies, the EU stakeholders and institutions have realised that specific solutions are urgently needed to translate orphan drugs centralised EU marketing authorisations into real patient access to therapies. The study performed in 2004 by the Alcimed consulting firm in response to the call for tenders launched by the European Commission, DG Enterprise, was a sign of this new awareness. The EC survey reached the same conclusions as the Eurordis surveys: there is a huge discrepancy between Member States in terms of the number of orphan drugs registered at national level; thus access of EU patients to OMPs varies depending on their MS of residence.

This 6th Eurordis Round Table of Companies workshop will open with the presentation of the results of the 4th Eurordis survey on the availability in each Member State of the orphan drugs that received a marketing authorisation more than one year ago and on the conditions of this availability.
Following the increasing difficulties we have encountered trying to obtain data from both the sponsors and the national competent authorities we will also address the issue of "secrecy" around such type of information. In fact, how confidential are the conditions of patient access to the product they need?

Furthermore, representatives from different Member States will present the procedures through which orphan drugs are registered at the national level and their price and reimbursement level defined.
Do these Member States have specific approaches for orphan drugs? Or are these products treated as any other drug centrally authorised at the EU level? Are there specific provisions in terms of allocated budget or reimbursement systems for orphan medicinal products?

During the workshop's afternoon session different stakeholders will present their vision of what makes orphan drugs <u>really</u> specific and different from other innovative drugs, as well as their opinion on the obstacles to patients' access to orphan drugs in Europe.
The following open and burning issues will be addressed by the speakers, panel and audience all along the workshop:

- How to obtain easier access to information about real patient access to orphan drugs in Europe?
- How to establish trust about the real clinical benefit of drugs that are sometimes authorised under exceptional circumstances or under conditional approval at an early stage?
- What kind of approach should be preferred for orphan drugs: price structure or HTA methodology?
- How to start a centralised assessment for these products, in order to facilitate further national procedures at the end of a completely centralised regulatory pathway up to the marketing authorisation? How could a pilot procedure be established with volunteering Member States and companies through the MEDEV? Should orphan drugs have an EU centralised procedure supported by the EU Commission?

Society at large has already made a choice when it adopted the EU orphan drug regulation: to make a special effort for rare disease patients who have been underprivileged until now. An open collaboration between stakeholders (industry, NCAs, patient organisations and academic experts) is, in our opinion, the only way to ensure real patient access to orphan drugs. We hope that this workshop will be the starting point of such a unique collaboration.