

COMMISSION STAFF WORKING DOCUMENT

on the experience
acquired as a result of the application of Regulation (EC) No 141/2000 on
orphan medicinal products
and account of the public health benefits obtained

Document on the basis of Article 10 of Regulation (EC) No 141/2000

COMMENTS FROM PUBLIC CONSULTATION

As required by Article 10 of Regulation (EC) No 141/2000 on orphan medicinal products, the European Commission drafted a report on the experience acquired as a result of the application of Regulation No (EC) No 141/2000 on orphan medicinal products and account of the public health benefits obtained.

The draft report was published on the Commission's website and all interested parties were invited to comment on the draft until 8 March 2006. Comments were received from:

- § Amgen
- § Association Internationale de la Mutualité
- § Daniel Hagn
- § EuropaBio – EBE
- § Genzyme
- § Medicines Evaluation Committee (MEDEV)

A summary of the outcome of the consultation follows:

It has been recognised that the report will serve as a reference tool and an information source for interested parties, and most importantly it is a useful instrument to raise awareness. Overall, stakeholders re-confirm that a successful and fully functional orphan system in the EU has been initiated and that its full development requires cooperative action between EU and Member States levels. Particular comments regarding designation, incentives, pricing/reimbursement and transparency follow:

1. Designation

It has been suggested distinguishing between orphan medicinal products and *super orphan* medicinal product by means of introducing a further prevalence figure in the designation criteria. It has also been proposed that a distinction is made in terms of incentives and, possibly go a step further and formulate a continual differentiation of incentives inversely proportional to prevalence. It is proposed, for example, to make the duration of market exclusivity dependent on patient numbers. Finally, it is recognised that, any proposals for solutions would have to be drawn up in agreement with politicians, the biopharmaceutical / pharmaceutical industry and patients' organisations.

Regarding the significant benefit criterion, it has been suggested that assessment includes always, next to the improved clinical safety and/or efficacy, an improvement of the quality of life.

2. Incentives

There has been support of an acknowledgement made in the report that the major incentive for orphan medicinal product designation is the 10 year period of market exclusivity.

Some years after the adoption of the EU orphan regulation, a 10 year period of data protection given to all innovative products was introduced into the EU pharmaceutical legislation. Some stakeholders have shown concerns about having the benefits of orphan designation being reduced by the new provisions on data protection.

It should be noted though that the market exclusivity granted to orphan medicines remains a much stronger incentive than the provisions on data protection of the revised legislation.

Indeed, a company that decides to submit a marketing authorisation application for a product that has been authorised for less than eight years in a Member State or in the Community shall be required to provide the results of new pre-clinical tests and clinical trials for that product. The secondly authorised product will not be considered a generic and the data protection rules will be respected. However, if the authorised product is a designated orphan medicine, it cannot be put on the market by a second company before the end of the period of market exclusivity, even if the second company performs new tests and trials.

3. Pricing and reimbursement

It has been noted that although the EU orphan regulation was a valuable and needed instrument to encourage the development of orphan medicines, the legislation may have unintended consequences, particularly for payers. A more in-depth analysis of the prices of the orphan medicinal products has been requested in order to achieve better transparency.

Some stakeholders ask for the review of the orphan status of medicines that have become blockbusters. Moreover, it has been pointed out that while the cost of orphan medicines is not a concern over high spending today (accounts today for 0.7-1.0% of the national pharmaceutical budgets), it is likely to rise considerably in the coming years with the expected increase in numbers of authorised orphan medicines reaching the market. Some claim that more attention is paid to cost-effectiveness issues. It has been suggested that all marketing authorisation holders of orphan medicines are required to provide an annual financial declaration (with number of units sold and prices) that is made public.

4. Transparency

The work of the COMP working group with interested parties is welcome by stakeholders due to structure and working organisation. Nevertheless, it is suggested that its activities are enhanced and that the agenda setting is made more transparent