Orphan medicinal products

1998/0240(COD) - 20/06/2006 - Follow-up document

This Commission staff working document sets out the experience required as a result of the application of Regulation 141/2000/EC on orphan medicinal products and account of the public health benefits obtained.

Orphan medicinal products are intended for the diagnosis, prevention or treatment of life-threatening or very rare serious conditions.

The report states that the response to the orphan legislation in the EU has far exceeded initial expectations; more than 450 applications have been submitted in the period between April 2000 and April 2005. Of those, more than 260 have been designated and 2 have gone on to receive a marketing authorisation.

Although more than 5 years of experience with the Regulation has now been gained, the true impact of the EU orphan initiative on public health will only be revealed progressively as longer term experience is accumulated. Already, more than 1 million patients suffering from orphan diseases in the Community may potentially benefit from these new 22 orphan medicines authorised during the first 5 years of application of Regulation 141/2000/EC. In addition, there is good ground to assume that the legislation has stimulated industrial activity leading to company creation with promising high-tech potential.

The full benefits of the EU orphan regulations require optimal synergies between action on Community and on Member State level. Incentives at the European Union level need to be translated into rapid access for patients to the new products throughout the entire Community and they need to be supplemented by incentives at Member States level. In this regard, the past experience was not entirely satisfactory.