Orphan medicinal products

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This Commission staff working document concerns the **fourth inventory of Union and Member State incentives** to support research into, and the development and availability of, orphan medicinal products — state of play 2015.

This year marks the 15th anniversary of the Orphan Regulation. In that time, there has been **impressive progress**, in particular as regards generating significant activity by the pharmaceutical industry in this field.

After 15 years of implementation and significant advances for patients, the Commission wishes now to **take stock of progress** in this field.

This paper thus represents the fourth version of the inventory.

Main conclusions: the Commission has launched a survey to collect information on national measures to support research into, and the development and availability of, OMPs. This information was based on Member States information validated by the relevant national competent authorities in December 2015. The Commission cannot vouch for its accuracy or completeness.

R&D support: some Member States have introduced reduced fees for registration and academic clinical trials, tax reductions or waivers, public funding for research and free scientific advice.

In **France**, OMP developers are exempt from certain taxes to be paid by pharmaceutical companies. In the **Netherlands**, the registration fee can be waived if the medicinal product is already registered in one or more other Member State and the prevalence of the condition is less than 1:150 000. **Poland and the United Kingdom**, on the other hand, have no specific measures for orphan medicinal products either.

Availability of OMPs to patients: as regards measures to support the availability of OMPs to patients, many Member States have confirmed that they are implementing ‘compassionate-use’ programmes to bring unauthorised medicinal products to market. Such programmes are used for individual patients (‘patient programmes’) on the basis of a doctor’s statement or the company can make products available to a group of patients.

Reimbursement of the product: the cost of the product may or may not be reimbursed, depending on the Member State. In **Greece** for instance, orphan drugs covered by a compassionate-use programme for individual patients are reimbursed in full. In **Germany**, all OMPs are reimbursed directly after market authorisation.

The impact of reimbursement on the availability of orphan medicinal products may be a matter of concern in the EU. The budgetary impact of OMPs is expected to rise due to the newly authorised products in the coming years. In this context, it is important to highlight that some Member States have adopted specific measures for the reimbursement of OMPs.

Other measures: most Member States reported other measures that they have taken under national plans on rare diseases that cover not only OMPs, but also the prevention (e.g. pre-natal diagnosis) and detection of rare diseases, the exchange of information and cooperation with patients’ organisations. In this context, most confirmed progress for the implementation of the **Orphanet database** and of centres of expertise for rare diseases and had registers of patients with particular diseases.