



**EPPOSI 10th Workshop on Partnering for Rare Disease Therapy Development
“10 years back into the future”
Federal Parliament of Belgium, Brussels, 26-27 October 2009**

Policy continuity and funding for rare diseases threatened by economic downturn – EPPOSI calls for policy continuity and continued research to ensure best quality healthcare in Europe

Brussels, 28 October - Representatives from EU and member state institutions, clinicians, patient groups and industry, came together at the 10th EPPOSI workshop on rare disease therapy development in the Plenary Session room of the Federal Parliament of Belgium this week, in the presence of Her Royal Highness Princess Astrid of Belgium.

EPPOSI is the European Platform for Patients' Organizations, Science and Industry, an independent European forum with equal representation of all involved stakeholders. The workshop addressed the threat to funding for rare disease research, to earlier and timely diagnosis and to access to orphan medicines and care due to the economic crisis in Europe. The event, which was organized by the European Platform for Patients' Organisation's, Science & Industry (EPPOSI), also celebrated the 10th anniversary of the adoption of the EU Regulation EC 141/2000 on orphan medicinal products, unanimously approved by the European Parliament in December 1999.

Alastair Kent, Chair of EPPOSI, took stock of the progress made over the past decade and reflected on the next decade, and on upcoming challenges and opportunities for the treatment of rare diseases. Dr. Yolande Avontroodt, member of the Federal Parliament of Belgium, and Chair of its Science and Technology committee, author of the Parliament Resolution on Rare Diseases explained the progress in Belgium towards a National Plan for Rare Diseases and Orphan Drugs by a multi-stakeholder steering group, supported by government in Belgium. Michael Griffith, former Chair of EPPOSI, representing Fighting Blindness Ireland, was celebrated for his foresight in conceiving this series of workshops.

There were discussions on current policies and how to ensure that these are also taken up by Member States, as well as about the important group of rare diseases which are rare cancers and represent about a fourth of all cancer cases.

At the workshop, the following recommendations were made:

- Additional targeted policy measures and incentives need to be identified to promote R&D in the field of Rare diseases and Orphan Drugs;
- Doctors' awareness and education needs to be raised in order to improve the chances for early diagnosis of rare diseases, and for enabling more research;
- Continuation of the orphan drug regulation 141/2000 got strong support as orphan drug development and availability could not rely on a free market to attract investments and drive innovation for rare diseases;
- European collaboration for the assessment of clinical added value of orphan medicines was strongly supported and participants called for immediate creation of the Working Party at the EMA;

- Orphan medicines should be conditionally reimbursed in Member States upon approval at EU level, subject to revision when more data become available, based on the revised report on the clinical added value
- Rare cancers must continue to be included in public policies for rare diseases and orphan medicines;
- Transparency about pricing, budgets and total impact of orphan medicines needs to be improved; and
- Priority needs to be given to setting up Centers of Expertise and European Reference Networks for diseases for which orphan drugs are approved, to speed up access and promote diagnostic and care standards.

Commenting on the event, Jean-Jacques Cassiman, EPPOSI Secretary-General and Chair of the Belgian Steering Committee for Rare Diseases and orphan Drugs under the King Baudouin Foundation, said: "We cannot allow the economic crisis to take from our commitment to research or to negatively affect potentially life-changing policies in areas such as severe and rare diseases. There must be policy continuity in this area and continued investment in research to ensure the best quality healthcare for all European citizens. Put simply, we need more research instead of less. And this needs to be connected with access for patients to the fruits of this research as well."

Action needs to be taken at Member State level to work on national plans as recommended in the European Council Recommendation on the field of Rare Disease and then put these in place, including clinical added value data collection and reports at EU level with conditional reimbursement for approved orphan drugs. Also, action is needed to secure funding for research, timely diagnosis, equitable treatment and better care to patients and their families. Rare diseases are a crucial field of research and a precursor of future developments in the healthcare field, making it a "societal laboratory" for new healthcare systems such as personalized medicine and sustainable innovation-based and patient-centred healthcare.

A full report will become available on the EPPOSI website in the coming weeks.

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