



**- Press Release -**

## **Millions of Europeans Suffer from Rare Diseases – Why Are They Not Higher on the Healthcare Agenda?**

*Patients' Organisations, Scientists, Biotechnology and Pharmaceutical Industries identify 7 potential solutions to the huge unmet need to treat rare diseases during Spanish "Rare Diseases Week" in Madrid*

**MADRID, Spain, 27<sup>th</sup> October 2006** – One in 33 babies is born with a rare and serious genetic disease – most of which have no treatment<sup>(1)</sup>. Yet the burden of rare diseases is immense and affects millions of individuals across Europe<sup>(2)</sup>. This week more than 100 stakeholders from patients' organisations, academia, the biotechnology and pharmaceutical industries came together for the 7th EPPOSI Workshop on Partnering for Rare Disease Therapy & Development during the Spanish Rare Disease Week<sup>(3)</sup> to assess how European society can better serve the needs of Europe's rare disease community.

During the first day of the workshop, participants developed seven key recommendations to help tackle the continuing issue of rare disease treatment, including:

- 1. National Health Technology Assessment bodies** should cooperate more on the evaluation methodology and how to conduct assessments – to avoid that each new drug is evaluated 25 times in different ways by the authorities in the 25 EU Member States. Evidence-gathering can thus be carried out "globally" through collaboration between national agencies, while the decision on what to do with that information can remain local.

2. The **pharmaceutical industry** should be more transparent about development costs and price-setting to help wider society understand why rare disease treatments are often higher than those for conventional treatments.
3. **Member States governments** should cooperate on an EU level to facilitate funding and patient access to such products – as treatments for rare diseases are not suited to market forces, central EU funding might contribute to solving the issue. And public funding should be made available for further investigation of existing molecules in the rare disease area.
4. The **Regulatory Authorities** should be more transparent on the elements of the decision-making on the granting or not of reimbursement. Difficult decisions are only acceptable if society understands why they are taken. Alternative paradigms for reimbursement need to be discussed to safeguard the future of the system.
5. The **European Commission** and **European Medicines Agency (EMA)** should emphasise the message that the EU's Orphan Regulation is intended to support innovative research into unmet medical needs in the field of rare diseases and that this research will be rewarded.
6. **All elements of the rare disease community** should build understanding and acceptability (awareness) of orphan diseases in society at large. If people do not understand, governments will not want to invest in making these treatments available to patients.
7. **All elements of the rare disease community** should work together to help the European Commission to recast the EU Clinical Trials Directive, whose goals are not questioned, but which is – in practice – raising challenges that are particularly critical in the rare disease environment.

Commenting on the recommendations, Yann Le Cam, speaking on behalf of the European Rare Diseases Patients' Associations (Eurordis), stated:

***“European rare disease patients need European solutions. Today all aspects of new drug development in Europe are centralised***

***through EU procedures. But access to those treatments is still in the hands of national governments. Some European rare disease patients can receive treatment while others cannot. Something needs to change”.***

EPPOSI aims to positively influence policies in human healthcare in Europe based on joint views developed by its stakeholders. The joint recommendations developed during the Madrid workshop will be shared with European decision-makers at all levels.

**– ENDS –**

**For further Information or interview opportunities, please contact:**

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**NOTES TO EDITORS:**

- The European Platform for Patients’ Organisations, Science & Industry (EPPOSI) was founded in 1994 and is a Europe-wide partnership of patients, industry and academic science. EPPOSI aims to advance healthcare policies for the prevention and treatment of serious diseases ([www.epposi.org](http://www.epposi.org)).
- EU Regulation 141/2000 – the European Orphan Medicinal Products Regulation, which creates a framework for incentivising research and development into rare diseases – came into force in 2000.  
([http://www.ec.europa.eu/enterprise/pharmaceuticals/orphanmp/doc/141\\_2000/141\\_2000\\_en.pdf](http://www.ec.europa.eu/enterprise/pharmaceuticals/orphanmp/doc/141_2000/141_2000_en.pdf))
- Since 2000, more than 400 products have been registered in the EU as potential treatments for rare conditions, of which more than 30 are already allowed on the European market, listed on the official EU Register of Orphan Medicinal Products.  
(<http://www.ec.europa.eu/enterprise/pharmaceuticals/register/orphreg.htm>)
- For more information on the estimated 7,000 rare diseases, please see the Orphanet website: [www.orpha.net](http://www.orpha.net)

**REFERENCE NOTES:**

1. Jeans for Genes Appeal website ([www.jeansforgenes.com](http://www.jeansforgenes.com))
2. European Organisation for Rare Diseases (EURORDIS) website ([www.eurordis.org](http://www.eurordis.org))
3. Semana de las Enfermedades Raras, 21-27 October 2006, organised by the Spanish Federation for Rare Diseases, FEDER: ([www.enfermedades-raras.org](http://www.enfermedades-raras.org)).