



- Press Release -

Pharma, Government and Patients Come Together in Aid of Rare Diseases

Stakeholders speak out on the huge unmet need in the funding, research and discovery of treatment for rare diseases

LONDON (27th October 2005) - 1 in 33 babies is born with a rare genetic disease – most of which have no cure¹. Yet the burden of rare diseases is immense and affects over 30 million individuals across Europe². This week saw the 6th workshop on partnering for rare disease therapy development meet under the guise of the European Platform for Patients' Organisations, Science and Industry (EPPOSI). EPPOSI aims to discuss and influence policies in human healthcare in Europe based on joint views by its stakeholders. This year's conference was the first that was international in scope, supported by both the National Institutes of Health (NIH) and the Food & Drug Administration's (FDA) Office for Rare diseases.

“The comprehensive coverage by expert speakers of all aspects of the topic is a very important element of the event. But the interactive nature of the workshop is the cornerstone of its importance: the ability for anyone to make recommendations means that conclusions coming out of the EPPOSI workshops are truly inclusive” Alastair Kent,
Conference Co-Chair for Patients Organisations, Genetic Interest Group,
UK.

During the workshop the EPPOSI conference developed several recommendations to help tackle the continuing issue of rare disease treatment, including the following:

1. **Industry should be willing to consider the opportunities to develop products for rare diseases** i.e., through creatively designed clinical trials or collaborating with others on a global basis

2. **Regulators at all levels should be more creative with regard to looking at the design of clinical trials**, i.e., the FDA and EMEA have stated that there is room to develop and implement a more harmonized approach to providing industry with advice for working practices while still respecting differences in law and culture

“long term, I believe that the more progressive-thinking regulators will be shown to be right.” Dr Agnes Saint-Raymond, Head of Sector, EMEA

3. **Patients, especially children, should be at the centre of the whole process**

These outcomes will now be passed on to major regulatory authorities and conferences on orphan products all over the globe, including the COMP committee at the EMEA. Documents will also be sent to local governments via health working groups and it is hoped that due to the consensual nature of the development of these recommendations that further significant progress on the treatment of rare diseases will be achieved.

“Patients’ organisations can play an important role in the development of treatments for rare conditions. EPPOSI provides the opportunity for the cooperation of science, industry and patient organisations together with the authorities, which is crucial if we want to speed up development and access to treatments”. Peter Streng, patient.

- ENDS -

For Further Information or interview opportunities, please contact:

Natalie Fairbank 0207 344 1308 natalie.fairbank@edelman.com

Amy Lawrence 0207 344 1205 amy.lawrence@edelman.com

Matt Foster 0207 344 1344 matt.foster@edelman.com

About EPPOSI:

The European Platform for Patients' Organisations, Science and Industry (EPPOSI) is a EU patient-led partnership between patients, industry and academic science, founded in 1994 for the exchange of information and discussion of policies in EU human healthcare:

www.epposi.org

¹ Jeans for Genes Appeal

² Eurordis Website, www.eurordis.org