

PRESS RELEASE

EU leaders commit to more action on Rare Diseases: “Europe and its rare disease patients will benefit,” say stakeholders

Brussels, 11 June 2009

On June 9, Health Ministers from the 27 EU Member States unanimously signed up to a series of political commitments on behalf of Europe's rare disease patients, by adopting a Council Recommendation on a European Action in the Field of Rare Diseases¹. This means that Member States have committed to creating a rare disease action plan at national level and to cooperating at a European level on this important and unmet public health priority.

The EBE/EuropaBio Task Force on Rare Diseases & Orphan Medicinal Products applauds the EU's Health Ministers on adopting the Recommendation. Ten years after the adoption of the legislation on Orphan Medicinal Products², it is a major proof of the continued strong political support for rare diseases as a public health priority in the EU. It brings renewed hope both to European society at large and, of course, especially to the rare disease patient community, which is estimated to number 29 million Europeans, suffering from one of between 5,000 and 8,000 rare diseases identified so far³.

The EBE/EuropaBio Task Force on Rare Diseases & Orphan Medicinal Products has been closely following and inputting into the Recommendation since its proposal by the European Commission in November 2008 and is pleased to see the Member States commit to designing and implementing National Plans for Rare Diseases and Orphan Medicines within a defined time-frame. This underlines the need to now take action at national level. The Council also committed to work together at a European level to address key issues such as diagnosis, minimizing delays in access to orphan medicines and the creation of Centres of Expertise, European networks and pan-European patient registries. In a field such as rare diseases, where expertise may be as rare as the patients, this is a strong example of European cooperation adding real value.

The biopharmaceutical industry very much welcomes increased patient involvement and empowerment in the ongoing decision-making process through routine consultation with patients' representatives. Very often when it comes to rare diseases, patients are the foremost expert on their disease. On the other hand, with rare treatments, often most of the knowledge and information on research, development, production and application actually sits within the innovator companies.

“The adoption of this Recommendation proves that there is a continued strong political momentum in the EU around rare diseases. In order to translate the political commitments contained in the Recommendation into meaningful actions at national level, we urge the creation of multi-stakeholder advisory groups to guide implementation of Rare Disease plans at national level and encourage Member States to extend invitations to a broad group of stakeholders, including patients, researchers, clinicians, payers and industry,” said Dr. Erik Tambuyzer, Chair of the EBE/EuropaBio Task Force on Rare Diseases & Orphan Medicinal Products. *“If all involved stakeholders are not represented, we believe that vital perspectives*

would be missing and that National Plans may not be as pragmatic or as focused as possible and, in the end, rare disease patients would suffer as a consequence. We want to turn innovation into meaningful new products as much as we can," he added, "and feel the pulse of our work in society".

All eyes are now on governments, to monitor the timely implementation of the Recommendation by its deadline of 2013. Time is short for rare diseases patients, but the EBE/EuropaBio Task Force on Rare Diseases & Orphan Medicinal Products believes that the unanimous endorsement of this Recommendation is a major step in the right direction by all governments in the EU. The Recommendation, if implemented correctly and in a timely manner at national level, will make a real difference for patients living with a rare disease and for their families.

ENDS

¹ Council Recommendation on action in the field of rare diseases. 2947th Employment, Social Policy, Health and Consumer Affairs Council meeting Luxembourg, 9 June 2009. Available here: http://www.consilium.europa.eu/uedocs/cms_data/docs/pressdata/en/lsa/108383.pdf

² Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products. Available here:

http://ec.europa.eu/enterprise/pharmaceuticals/eudralex/vol-1/reg_2000_141/reg_2000_141_en.pdf

³ The Voice of 12,000 Patients: Experiences and Expectations of Rare Diseases Patients on Diagnosis and Care in Europe. Eurordis. 2009

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Joint EBE/EuropaBio Task Force on Rare Diseases & Orphan Medicinal Products

EBE (European Biopharmaceutical Enterprises) and EuropaBio (European Association for Bioindustries) have established a joint EBE/EuropaBio Task Force on Rare Diseases & Orphan Medicinal Products, comprising companies who have either developed or intend to develop orphan drugs under Regulation EC/141/2000. Together, members of the Joint Task Force represent a large proportion of orphan drugs currently available on the EU market.

EBE

EBE (European Biopharmaceutical Enterprises) is the European trade association that represents biopharmaceutical companies of all sizes operating in Europe. It has 65 member companies, which are engaged in the research, development, manufacturing and marketing of new medicinal products using biotechnology. EBE also operates as the biotechnology arm of EFPIA, the European pharmaceutical industry federation.

EuropaBio

EuropaBio is the European Association for Bioindustries, solely and uniquely bringing together bioscience companies from all fields of research and development, testing, manufacturing and distribution of biotechnology products. It has 68 corporate and 7 associated members, 4 BioRegions and 25 national biotechnology associations representing some 1800 small and medium sized enterprises involved in research.