



***EuropaBio and EBE congratulate EU leaders on the Commission Communication on Rare Diseases and calls for European action to turn principles and proposals into reality***

Brussels, 13 November 2008

EuropaBio and EBE would like to congratulate the European Commission on its draft Communication and proposed Council Recommendation on Rare Diseases (1). These are timely and vital documents, not only for the rare disease community, but also for the European society at large. This is a unique opportunity to identify core areas where a concerted effort can make a real difference to the effective diagnosis, treatment and care for rare diseases in the EU. The most relevant topics include improving awareness about rare diseases; supporting national plans for rare diseases in the Member States; and strengthening cooperation and coordination for rare diseases at European level including on access to orphan medicines.

The joint EuropaBio-EBE Rare Diseases Taskforce now calls for EU member state governments to take action to address the issues facing patients affected by rare diseases in Europe, by ensuring that the principles and proposals in the Recommendation and the Communication are turned into reality. Also, further actions are needed to improve the opportunities to conduct dedicated research and development for rare diseases in the European Union.

Since the adoption of the EU's Orphan Regulation almost 10 years ago, 50 treatments for rare diseases have been granted a positive opinion by the European Medicines Agency (EMA). Patients, however, may not have access to these treatments even after their authorisation. Thus, while in the past, the main problem for rare diseases patients was the lack of *treatments*, now the potential lack of *access* to these treatments is an area of concern. Therefore, EuropaBio and EBE call for a dedicated series of efforts related to diagnosis, provision of treatment, and a tailored reimbursement process and care, that will take into account the specific nature of orphan medicinal products, all stakeholders, and the high, and hitherto unmet medical needs that they are intended to address.

The EuropaBio-EBE Rare Diseases Taskforce therefore calls on Member States to take the next steps in implementing the concepts they agreed to in the High Level Pharmaceutical Forum. The paper "Improving Access to Orphan Medicines for all affected EU citizens" (2) contained a series of guiding principles to which Member States agreed and which should form the framework for the next steps to improving access to treatment. This would be the logical next step, having been agreed to and having the possibility to make a real difference.

EuropaBio and EBE strongly endorse the notion of creating suitably funded national plans for rare diseases. National plans are a vital element in ensuring effective research into disease mechanisms, diagnosis, treatment and care for these diseases, because healthcare provision remains a national responsibility. National plans should be designed and implemented around a core group of activities that will make a concrete contribution to foster R&D and to improve access to diagnosis, treatment and care. In addition, if implemented in a coordinated way in each EU Member State, they will contribute to building a European rare disease area and facilitate cooperation between the countries, while still respecting the subsidiarity principle.

With regards to strengthening the research efforts in this area of exceptional unmet medical need EuropaBio and EBE specifically welcome the notion of exploring additional incentives at national or European level to strengthen research into rare diseases and development of

orphan medicinal products. EuropaBio and EBE strongly believe that the creation of Centres of Expertise, European networks and pan-European patient registries are vital tools in this field as they are necessary prerequisites for the pooling of scattered information, data and expertise. There are between 5,000 and 8,000 known rare diseases and it is not realistic to expect every physician to be able to recognise and treat each of them. To make such a system of Centres of Expertise work, EuropaBio and EBE believe it is vital that treatment for rare diseases should be funded at a national level – not at the regional, local or hospital level. That way, physicians or hospitals that diagnose or care for rare disease patients will not be financially “penalised”.

The EuropaBio-EBE Rare Diseases Taskforce welcomes the creation of an EU Advisory Committee on Rare Diseases (EUACRD) which shall advise on the implementation of the Communication and replace the current EU RD Task Force. As industry is a key player in the field – being the only relevant source for innovative medical therapies for rare disease patients – we would welcome the opportunity to actively participate in this very important initiative which we feel may otherwise lack a vital perspective if the private sector is unable to contribute.

Time is short for rare diseases patients, who need to gain quick access to new treatments being developed. EuropaBio and EBE believe that this Communication is a further step in the right direction and particularly this Communication, combined with adequate funding, can make a real difference for patients living with a rare disease.

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### **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 79 corporate and 5 associated members, 5 BioRegions and 25 national biotechnology associations representing some 1800 small and medium sized enterprises involved in research.

### **European Biopharmaceutical Enterprises (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 research, development and marketing of new medicinal products using biotechnology.

These two organizations have established a joint Task force on Rare Diseases, comprising interested member companies who have either developed or intend to develop orphan medicinal products under Regulation 141/2000. Together, members of the Joint Task force represent a large proportion of orphan drugs currently available on the European market.

### **Notes**

(1) European Commission Communication and a proposal for a Council Recommendation on rare diseases [http://ec.europa.eu/health/ph\\_threats/non\\_com/rare\\_10\\_en.htm](http://ec.europa.eu/health/ph_threats/non_com/rare_10_en.htm)

(2) European Commission Pharmaceutical Forum; Improving Access to Orphan Medicines for all affected EU citizens [http://ec.europa.eu/pharmaforum/docs/pricing\\_orphans\\_en.pdf](http://ec.europa.eu/pharmaforum/docs/pricing_orphans_en.pdf)

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