Dr. Patrick Le Courtois  
Head of Unit, Human Medicines Development & Evaluation  
European Medicines Agency (EMA)  
7 Westferry Circus  
London E14 4HB  
UK

2 April, 2013

Dear Dr. Le Courtois,

Thank you for your letters dated 12 December 2012, addressed to EuropaBio and to EFPIA/EBE regarding the changes in the fee reduction programme for the developers of orphan medicinal products.

These letters have been passed onto the members of the joint European industry task force on orphan medicinal products, which is jointly organised by the members of both EuropaBio and EBE – a specialised group within EFPIA. The Task Force comprises companies that are involved in the research, development and bringing to market of orphan medicinal products.

The members of the joint European industry task force have reviewed the provisions and would like to draw your attention to the industry concerns regarding the fee reduction package for orphan medicinal products in the EU.

Again with effect from 1 January 2013 further decreases in fee reductions have been published and among those are concerning the fees for orphan medicinal products. The revised rates for non-SME companies show a decrease in the fee reductions from 75% to 40% for protocol assistance related to non-paediatric products, and withdrawal of fee reductions for initial Marketing Authorisation Applications and for Pre-Authorisation inspections.

The adoption of the Orphan Medicinal Products Regulation 141/2000 at the end of 1999 introduced incentives to encourage the development of medicinal products for the diagnosis, prevention and treatment of rare diseases. The rationale for establishing incentives for rare disease research is indeed acknowledged in the EMA pamphlet ‘Orphan Medicinal Product Designation’ which states that the aim is to encourage the development of products where the cost of bringing them to the market would not be recovered by the expected return to the company responsible for doing so (the sponsor).
As a policy instrument, the incentives to encourage the development of orphan medicinal products in Europe have been successful. A study conducted by the Office of Health Economics noted that there had been a substantial increase in the number of OMPs potentially available in Europe compared to the situation before the European Regulation. The conclusion to this study was that “incentives provided in the legislation greatly fostered innovation and entry into the market of therapies addressing hitherto unmet medical needs.” Notably, the fee waivers and fee reductions were identified by the surveyed companies as the second-most-important incentive, second only to the Market Exclusivity provisions of the Regulation. The members of the Task Force have noted that these fee reductions have been gradually reduced from their original level to the proposed current status, which means almost no fee reductions for the majority of the companies reflected by the Task Force membership.

The member companies of the joint European industry task force on orphan medicinal products would like to highlight this and to signal their concern that further inroads into the incentives could undermine the effectiveness of the EU’s framework to stimulate rare disease research and development.

We would also like to highlight the potential inconsistencies between this policy and the other policies currently being developed within the European Union, such as the European participation within the International Rare Disease Research Consortium (IRDiRC), and the commitment by the national European governments to put in place National Plans to support the diagnosis, treatment and care for Rare Diseases by the end of 2013.

In addition to the long-term implications for rare disease research in Europe, there are also potential considerations for manufacturers with global programmes. The negative signal of reducing the support for OMPs contrasts with the comprehensive package put in place by the FDA and may have the unintended consequence of enhancing the attractiveness of the US as a base for rare disease research.

The EuropaBio and EBE members that are members of the joint Task Force on Orphan Medicinal products understand that the EMA must operate within strict budget parameters. We also understand that the remit and responsibilities of the Agency are being steadily increased, due to the new legislation within the European medicinal products framework. We hope that, going forward, the effective allocation for sufficient funds to continue to support and encourage rare disease research and development within the framework of the work of the Agency will be able to be appropriately secured and allocated. We look forward to working with you in this regard.

The biopharmaceutical industry remains committed to new product development in Europe and an appropriate regulatory framework will play a key role in establishing an environment which encourages research into rare diseases. We would be happy to answer any questions that you may have regarding the industry concerns on this provision and
would welcome the opportunity to arrange a meeting if you feel that this would be helpful.

We look forward to hearing from you in due course.

With kind regards,

Nathalie Moll
Secretary General
EuropaBio

Titta Rosvall-Puplett
Executive Director
EBE

*Assessment of the impact of Orphan Medicinal Products on the European Economy and Society: OHE Consulting, November 2012

C.C.
Professor Ségolène Aymé, Chair, EUCERD
Yann Le Cam, Vice-Chair, EUCERD
Kate Bushby, Vice-Chair, EUCERD
Helena Kääriäinen, Vice-Chair, EUCERD
Jaroslaw Waligora, DDG1, Unit C1, DG SANCO, European Commission
Agnès Mathieu, DDG1, Unit D5, DG SANO, European Commission
Flaminia Macchia, Director EU Public Affairs Director, EURORDIS