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Mr A Monsterrat
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European Commission
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Dear Mr A Montserrat

Re: Public Consultation on Rare Diseases: Europe's Challenges

I would like to thank you or the opportunity to respond to the Commission's consultation on Rare Diseases.

The BIA is the trade association for innovative enterprises in the UK bioscience sector. It represents over 300 members, the majority of which are involved in realising the human health benefits that bioscience promises, including research in and the manufacture of Orphan Medicinal Products to treat rare diseases across Europe.

As a member of EuropaBio, the BIA welcomes and supports its contribution paper submitted jointly with the European Biopharmaceutical Enterprises (EBE). This paper puts forward a number of good suggestions for the Commission's future communication on rare diseases. A number of BIA members have made important contributions to this paper along with other members of EuropaBio and EBE which represents over 1800 SMEs across Europe.

BIA is particularly supportive of EBE/EuropaBio's comments in answer to Question 8:

> **"Question 8: Do you envisage the solution to the orphan drugs accessibility problem on a national scale or on an EU scale?**
>
> …
>
> 4. Orphan medicinal products… should be approved for reimbursement in the Member States following the granting of a Marketing Authorisation, on the basis that they are too rare to be able to follow HTA rules and on the fulfilment of the EU's legal requirement to have proven either that no other treatment exists or that they offer clinical superiority.
> 5. If this is the case, sponsors should be required to gather experience from the field in order to develop the data sets – including clinical effectiveness and outcomes – which should be submitted to the reimbursement authorities at pre-determined points. These should be realistic according to the rarity of the disease. However, complexity increases in correlation to rarity. The system should reflect this sliding scale of complexity, allowing for thorough examination on a case-by-case basis. For "more common" rare diseases, therefore, the review points of the data gathered could be earlier than for those rarer conditions, where prevalence may be less than 1 in 150,000 or even fewer, these time points could be set further out, to allow for the development of meaningful data in the rarer conditions..."

Once again, I would like to thank you for the opportunity respond to this consultation. I very much look forward to being involved in the future.

Yours sincerely

Joseph Wildy
EU Public Affairs Manager