

Public Consultation on “Rare Diseases – Europe’s Challenges”

**Question 8: Compassionate Use
“Coordinated Compassionate Use Programme”
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BioMarin, Genzyme and Shire Human Genetic Therapies believe that a better system is needed in Europe to provide medicines to patients in need before approval and/or reimbursement. We would like to advocate for an expedited access to treatments for rare diseases, as mentioned in our contribution to existing joint industry submissions in response to Question 8. However, given that our companies are often treating very rare diseases, we would like to also explore the possibility of improving Compassionate Use in Europe, particularly where it applies to named-patient basis programmes.

Compassionate use is an important tool to provide patients access to treatment in the case of severe and urgent medical need and when patients have need but are, for some reason, ineligible for / cannot access an active clinical trial. Compassionate use programmes should not be used to replace participation in a trial. Compassionate use is often considered for Orphan Medicinal Products, since they are designated for “life-threatening, seriously debilitating or serious and chronic” conditions, in the field of rare disease treatment.

Administrative procedures can delay access to treatments. Where clinical data supports a positive risk-benefit profile (e.g., prior to approval of a Marketing Authorisation) it may be an appropriate decision to allow early access to the treatment under development.

Successful compassionate use often depends on the ability of the patient to call on political or high-profile connections to plead on their behalf. Requests on behalf of patients in Member States from e.g., the Royal family, church leaders or government Ministers have all been instrumental in removing obstacles to access to compassionate use of treatments in development. An improved European system should promote transparent and equal access to compassionate use, based on patient needs rather than socio-economic advantages or political or social connections.

Article 83 of 726/2004 aims to facilitate the EU Compassionate Use process and the EMEA attempted to give some clarity and transparency in its Guideline EMEA/27170/06 on compassionate use based on this Article, however, the approach has only gone some way towards achieving this and challenges still remain.

Although a welcome start, the Guideline only focuses on cohort approaches and the majority of countries have Compassionate Use systems that are laid down on a named-patient basis, which can be more appropriate for very rare diseases. Harmonisation between Member State Compassionate Use programmes has yet to be achieved.

The points of contact and the data package requirements make it challenging to implement a compassionate use programme across the EU, especially for smaller companies.

In order to address the challenges, we would suggest the establishment of a specific sub-committee of European experts experienced in the field of Compassionate Use procedures to evaluate the appropriateness of compassionate use in a given case in future. This would remove the need for 27 different procedures. This would also allow a pooling of expertise at a European level and a development of experience in the field as the Committee and its members would be able to build up expertise based on experience of evaluations. Currently, this is in the hands of the CHMP but maybe it needs to be more tailored to the Compassionate Use systems.

Additionally, a European guideline should be developed, clarifying the legal responsibilities in a Compassionate Use situation to the Member States. Awareness of such responsibilities by the Member States is of critical importance. Improved accountability and clarity of responsibilities will greatly improve the time to decisions, and the harmony of such decisions both within the Member State and the EU.

A single source for the requirements for import licenses and labelling should also be of considerable value – possibly as an annexe to the Guideline.

Thank you for taking our contribution into consideration. We are happy to provide more information or elaborate further on these suggestions, should it be required.

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