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**Paediatric Diseases**

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Directorate-General for Health and Food Safety,  
Unit SANTE B/5,  
BE-1049 Brussels

Barcelona, 30/10/2018

**Regarding:** TSC 01/2018 on GCP for ATMPs Targeted stakeholder  
consultation on the draft Guidelines on Good Clinical Practice for  
Advanced Therapy Medicinal Products: EBMT response

*Eoin McGrath ([eoin.mcgrath@ebmt.org](mailto:eoin.mcgrath@ebmt.org)) responding on behalf of the  
European Society for Blood and Marrow Transplantation (EBMT)*

Dear sir/madam,

EBMT is a not-for-profit healthcare professional organization with a  
principally academic focus working in the field of blood diseases and  
cellular therapy. Full details can be found at [www.ebmt.org/anbi-data](http://www.ebmt.org/anbi-data).  
EBMT is registered with the EU Transparency Registry with ID  
652992023103-09.

In the field of cellular therapy ATMPs, EBMT is working with other  
stakeholders including manufacturers and the European Medicines  
Agency on patient follow-up via the EBMT patient registry. EBMT offers  
an extensive programme of professional education which supports good  
clinical practice. EBMT is also actively seeking collaboration with other  
professional to facilitate basic science and the translation of novel  
therapies to the bedside while also advocating for healthcare  
professionals, patients and donors.

Overall, the EBMT considers that the Consultation Document *Good  
Clinical Practice for Advanced Therapy Medicinal Products (the Guideline)*  
is in line with what should be expected for medicinal products with  
significant inherent complexities including intricate manufacturing and  
distribution processes for very small batches or even individualised  
products, and of most concern, the unknown long-term effects.

The EBMT welcomes the following aspects of the Guideline:

- It addresses long-term follow-up (e.g. for GTMPs = 15 years) including  
via registry or NIS
- Considers particular aspects of haematopoietic stem cell  
transplantation (HSCT) including compliance with EU Directives on  
tissues and cells for their collection
- Includes traceability (from donor to subject and subject to donor)
- Recognises the intrinsic variability of the starting materials



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- Identifies the need for specific instructions and training on reconstitution before administration e.g. thawing and validation of that process
- Takes an overall risk-based approach - mitigating measures should be proportional to the risk
- Several elements are in line with the FACT-JACIE International Standards for Hematopoietic Cellular Therapy<sup>1</sup> in terms of consent and traceability for instance

The scope of the Guideline is limited to clinical trials but much of the contents are equally relevant when these therapies become standard of care.

The Guideline contains somewhat ambiguous terminology that lead to a lack of clarity. For instance, words like “sufficient” or “sufficiently” (lines 178, 335), “detailed” (lines 178, 180, 241, 243, 247, 268), “adequate” (lines 135, 262, 263, 273, 276, 295, 351) are not defined and could lead to different interpretations. Any further definition of such requirements would be very welcome.

Some aspects are not specific to ATMPs and are more widely applicable e.g. lines 91-94 and lines 106-110 whereby any treatment *including* ATMPs should contemplate the potential consequences for future transplants. Consideration should be given to separating the two ambits in the document.

Kind regards,

Eoin McGrath  
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<sup>1</sup> <https://www.ebmt.org/jacie-accreditation>