EBMT European Society for Blood and Marrow Transplantation

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) 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. 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

European Society
for Blood and Marrow
Transplantation

www.ebmt.org

- 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[1] 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
JACIE Operations Manager
eoin.mcgrath@ebmt.org

[1] https://www.ebmt.org/jacie-accreditation