

Comments on Innovative Medicines Initiative consultation “Facilitating the translation of advanced therapies to patients in Europe”

Have the key challenges that can be addressed through collaborative, public private initiatives been properly identified?

In general, the key challenges for the development of ATMPs have been identified. However, due to the diverse groups of ATMPs (gene therapy vs. somatic cell therapy vs. tissue-engineered products), specific challenges do not necessarily apply to all ATMPs, e.g.:

- 3.1. Preclinical development: assessment of vector systems will be primarily beneficial for gene therapy systems, i.e. listing the elucidation of vector systems as one of the key recommendations during preclinical development might be too specific
- 3.4. Pricing, reimbursement and access: the table mentions considerations unique to ATMPs, which might be accurate for one group of ATMPs or a specific ATMP but certainly not for all
 - Example 1: The treatment regimen for gene therapy systems can be one-time/short-term but this is not the case for all types of ATMPs. Some ATMPs (e.g. somatic cell therapy of cancer) also need to be used in multiple treatments/have a longer treatment duration.
 - Example 2: Individualized production applies e.g. to cell therapy systems containing autologous cells but not to all ATMPs.

Other specific remarks:

- 3.3. Manufacturing: “For the genetic therapies the production of the viruses is problematic and cumbersome.” There are other vectors for gene therapy like DNA or mRNA with straightforward manufacturing processes, i.e. an accurate statement could be “For the genetic therapies *using viral vectors* the production of these viruses is problematic and cumbersome.”
- 3.4. Pricing, reimbursement and access: regarding the topics in the context of market access, 2. Hospital exemption, suggestion to include an additional bullet point “need for harmonization of the regulation among member states for granting hospital exemptions”

Which of the proposed potential initiatives should be prioritized?

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Are any areas missing?

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What are the key European or national initiatives that IMI shall synergize with?

Not an initiative per se, but certainly the “Innovationsbüro” at the Paul-Ehrlich-Institut is a key stakeholder in Germany regarding ATMP regulatory requirements and offering a link to EMA and HTA bodies in Germany.