

Outcomes of the IMI consultation on advanced therapies

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Feedback from the consultation on the ATMP Concept paper

- Consultation closed 26 July
- 34 written submissions were received (over 120 pages of comments!)
- Submissions came from 9 European countries and also 7 EU/International bodies
- 8 submissions came from individuals in their personal capacity
- 26 submissions came on behalf of institutions

General Comments

- The paper did not make reference to existing documents of relevance
- The document did not refer to potential links with ongoing projects and initiatives
- In general it was felt that the main key areas of unmet need were covered
 - We will deal with identified gaps later
- The questions were relevant
- It was clear that an IMI initiative(s) in this space could not solve all of the issues
- A public /private initiative would indeed be hugely beneficial in tackling some of the major hurdles
 - We will be talking about specific examples today

Specific Comments on Preclinical Development

- Molecular imaging needs more prominence as a set of tools that can really help in both preclinical and clinical research
- Choosing the right models for a specific disease was crucial and lots of discussion around the maturity of organoids versus small or larger animal models (who determines what a robust preclinical assessment means?)
- Immunogenicity was a major hurdle for some of the approaches taken and several submissions appealed for more research in this area
- Defining the product for use in preclinical assessments was seen to be a challenge (quality standards/ research batches vs GMP batches etc.)
- Use the framework of risk based approach (already part of the EU regulatory system)

Specific Comments – Clinical Development

- Small number of patients involved in these types of trials (by definition)
 - Clinical trial designs are highly case and disease dependent
 - Need long term monitoring of patient and socio-economic impact
 - Potential treatment areas will expand
- Need to assess benefit risk profiles early on
 - The role and use of the Hospital Exemption
 - 85% of clinical trials involving ATMPs are academically led
 - 'HE should be considered as complementing rather than competing with CMA'
 - Suggestion of a mapping exercise to understand the national interpretations of the HE clause
 - Reminder that HE can only be obtained for 1 member state
 - HE harmonisation across the EU is crucial
 - HE can sometimes dilute regulatory oversight and disincentivise private investment
- The lack of funding for clinical trial design and implementation

Specific Comments on Manufacturing

- Lack of GMP facilities in hospitals- who should manufacture these products?
- Should we invest in improving academia based product development?
- Lack of quality standards for these products
- Lack of robust potency assays for ATMPs
- Supply of new materials (cells, vectors etc.)
- Heterogeneity of product types (viral/non-viral; cells; nucleic acids; other biologics etc.)
- Pharmaceutical grade raw materials – lack of pharmacopoeial monographs for these
- Requirement for networks for qualification of suppliers?
- Scaling up once clinical proof of concept is achieved

Specific Comments on Pricing Reimbursement and Access

- Do not assume that the current HTA methodologies and frameworks cannot work for ATMPs
- Requirement for constant dialogue among the actors (clinician researchers, patients, regulators, HTA bodies, payers)
- Mapping of existing resources (registries, HE and compassionate use records) and use of real world evidence of clinical utility is needed

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

- In general – yes.....but
 - More discussion is required to determine a focussed approach
 - Determine which areas would deliver the most value to stakeholders
 - Clinical proof of concept is a key milestone for many of these interventions

2. Which of the proposed potential initiatives should be prioritised?

- Lots of support for preclinical testing and manufacturing
- Significant interest in the education and training needs for all involved in the ATMP space
- Develop shared validated vector systems
- Develop shared validated analytical tools
- Develop regulatory pathways for ATMPs
- Set up specific manufacturing hubs (in public or private space?)
- Build economic models
- Build registries, mapping and inventories of what is available
- Addressing the current lack of funding for clinical assessments
 - first in man clinical proof of concept

3.Are there any missing areas?

- Education and training for health professionals and patients, regulators, HTA experts and payers in the ATMP space
- Full breadth of product types not articulated in enough detail
 - Define these e.g. genes, and other nucleic acids (RNA, antisense) delivered using both viral and non-viral vectors; Cells derived from autologous, heterologous or stem cell sources; other bioproducts requiring sophisticated delivery systems
- Need for economic models
- Appropriate hospital based infrastructures top address specific clinical needs for assessments of ATMPs

What are the key European or national initiatives that IMI should synergise with?

- EMA
- ECRIN
- CATAPULT
- EATRIS
- BBMRI
- Existing IMI projects
 - (ADAPT-SMART, EBiSC, GETREAL, STEMBANCC)
- EBMT, ISCT (JACIE)
- European Statements of Hospital Pharmacy
- National Societies for Gene and /or Cell Therapies
- EUnetHTA



Thank you

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