IMI consultation on advanced therapies

Response from Helena Kelly

Please find below my comments on the Advanced Therapies Concept Paper which was issued for consultation.

If you require clarification or further information in relation to any of these points please do not hesitate to contact me.

Kind regards

Helena

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Additional information: Deputy Co-ordinator of the AMCARE (Advanced Materials in Cardiac Regeneration, (www.amcare.eu) and DRIVE (Diabetes Reversing Implants for enhanced Viability and long-term efficacy (www.project-drive.eu) funded respectively through FP7 and H2020.

Commenting in a personal capacity

- **Have the key challenges that can be addressed through collaborative public-private initiatives been properly identified?**
- **Are any areas missing?**

In relation to these questions, the concept paper addresses a broad range of the key challenges that are facing ATMPs and the organisations trying to develop them. The one area that I feel merits further consideration is combined ATMPs. Combined ATMPs face specific challenges due to the number of components that may be involved in their development and manufacture, and the fact that these components will generally be from across multiple sectors including pharmaceutical, biopharmaceutical and medical device industry. This combined with the fact that a large amount of ATMP development is happening at an academic level means there is a very significant need for cross-sectoral and cross-functional collaboration, with a heightened need for collaborative public-private partnerships for commercialisation of such products to become a reality. With respect of combined ATMPs, the development of any one component, without the expertise and engagement of the other sectors involved will, in my view, ultimately lead to delays and challenges in bringing products to market. Inter-sectoral engagement on a single product brings with it complexities associated with product development and validation, regulation, procurement, manufacturing and logistics, management of intellectual property, marketing, pricing and reimbursement. While there is now considerable inter-sectoral activity between the medical device and pharma sectors combined ATMPs would bring this to a new level of complexity. Therefore mechanisms or activities which promote engagement or integration between different sectors with a specific interest in ATMPs is to be welcomed, especially in relation to the development of combined ATMPs.

Secondly, one area that I feel is not addressed is the approach to delivery at the bedside. ATMPs represent a new type of treatment within the clinical environment, and to date their delivery is primarily in a small number of clinical sites on an ad hoc basis through the HE scheme. If ATMPs are to become more widely available in a clinical setting the management of these therapies within the clinical setting requires consideration. The majority of modern therapies come in 'off the shelf' format or with minimal manipulation/compounding required. It is likely that many ATMPs will have more complex requirements in their, storage, preparation and delivery. How can this be managed and where will facilities, infrastructure and responsibility for such activities sit within the clinical environment? Historically, ownership of the management of drug related therapies sat with the hospital pharmacy in terms of procurement, inventory control, dispensing and provision of technical support in relation to these therapies. ATMPs present an entirely new therapeutics sector which will require greater levels of cross-functional collaboration within the clinical environment, but in parallel

clear ownership to ensure risk minimisation. How this will be done within the clinical environment is a key question if clinical adoption is to be successful once products have been approved. A forum creating engagement between academia, industry and the clinical end-user team (not just the clinician) is critical to ensure that issues of 'usability' within the clinical environment are addressed early in the development process, and to assist with upskilling of clinical staff who will in future years be responsible for the management of these sorts of therapies within the clinical environment. Consideration to centres of excellence to support this clinical translation would be useful to increase the likelihood of adoption, especially in smaller market sectors such as Ireland.

- **Which of the proposed potential initiatives should be prioritised**

Key areas for prioritisation include;

- The implementation of initiatives that promote and improve the approach to manufacturing and quality control testing of ATMPs. This includes initiatives that promote innovation in cost-effective aseptic manufacturing processes at a small scale for ATMPs, as well as those that focus on final presentation and packaging to improve usability at point of use and more widespread clinical adoption. Initiatives in the area of closed-loop manufacturing, in-process controls (IPC) and in-line testing, and process analytical technology (PAT) would all be beneficial.
- The development of databases which can provide central sources of information on knowledge gained to date in relation to ATMPs including in areas mentioned such as inventory of existing clinical programmes, raw materials, manufacture, testing and standardisation of cell supplies. The availability of such information (subject to IP considerations) would be beneficial to early stage programmes and would prevent replication of work, enabling faster 'product to market' timelines.
- The development of educational material not only for the general public but also for relevant sectors who will contribute to ATMP development and delivery (bench to bedside) in the future. The development of standard modules on aspects such as regulatory, clinical, manufacturing aspects of ATMPs would be a valuable tool to a wide variety of participants, including universities, regulators, clinical environments, and the pharmaceutical and medical device industry. If the development of ATMPs is to be future proofed, knowledge of ATMP therapies needs to be introduced to the undergraduate programmes of health sciences faculties, engineering faculties and clinical training programmes. This will ensure there is adequate expertise in the workplace to support their adoption as their use becomes more widespread. Such content, in my opinion, would be best developed by multi-disciplinary, cross-sectoral and cross functional teams with experience in the area of ATMP development, which reflects the reality of the complexity of ATMPs.

- **What are the key European or National initiatives that IMI shall synergise with?**

At a European level synergy with Horizon 2020 would appear critical. There is a specific focus on biomaterials and Key Enabling Technologies (KETs) throughout H2020 as discussed in the report ‘BIOMATERIALS FOR HEALTH A Strategic Roadmap for Research and Innovation HORIZON 2020’

At a national level, in Ireland engagement with Enterprise Ireland (EI) and scientific centres with a specific focus on cell therapies, biomaterials, medical devices, and the integration between these would offer a point of synergy. Examples of two such centres are the Science Foundation Ireland (SFI) centres, AMBER (Advanced Materials and Bioengineering Research, www.ambercentre.ie) and CÚRAM (Centre for Research in Medical Devices, www.curamdevices.ie).

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