Topic idea submitted to IHI - Reference Number: TI_001228

Are you submitting the idea:
☐ **in your personal capacity?**
☒ **on behalf of an organisation?**

Please indicate the name of the group organisation: Inserm

Please select from the list below the type of stakeholders your organization represents: Public Scientific Research Organisation

1 Title of your idea

Please provide a short title that accurately reflects the objective(s) of your idea:
Improving the production and delivery of Advanced Therapy Medicinal Products (ATMP) and patient accessibility by supporting collaboration between academic laboratories and industry

2 Scope

Explain the specific challenges/problems to be addressed by your idea and how these affect relevant stakeholders, taking into account what is already known and/or available in the field:
This proposal refers to the specific challenges represented by the production of Advanced Therapy Medicinal Products (ATMP) including Cell and Tissue therapy, Gene Therapy, Extracellular Vesicles but excluding recombinant proteins and CAR-T cells.

Three main topics were identified to address challenges such as scalability, standardisation and robustness of bioprocesses and bioproduction:
• Process and biomaterials optimization: standardisation and harmonisation of bioproduction methods at a global level and transposable to the European level, predictive tool using, for example, artificial intelligence and mathematical modelling technologies.
• Development of medical devices applied to ATMP administration or transplantation, transport and stability.
• Implementation of processes to develop high quality standards for ATMP storage and improving ATMP shelf-life, particularly in the context of off-the-shelf products.

The proposed project should be, at least TRL3 at the start and implement strategies to improve the sustainability of the developed bioprocesses.

Please indicate which IHI specific objective(s) (SO), as described in the IHI Strategic Research and Innovation Agenda (SRIA), your idea addresses:

["SO2: integrate fragmented health research and innovation efforts bringing together health industry sectors and other stakeholders, focusing on unmet public health needs, to enable the development of

tools, data, platforms, technologies and processes for improved prediction, prevention, interception, diagnosis, treat- ment and management of diseases, meeting the needs of end-users"]

Please select the keywords that are most relevant to your idea:
["Health technology"]

In alignment with the IHI specific objective(s) selected above, specify the objectives of your idea:

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3 Expected impacts to be achieved by your idea

Briefly describe the expected impacts to be achieved by your idea, ensuring that they contribute to IHI general and relevant specific objectives, as described in the IHI SRIA:

Impacts *are wider long-term effects on society (including the environment), the economy and science, enabled by the outcomes of R&I investments. Impacts generally occur sometime after the end of the project, e.g. successful implementation of digital solutions supporting people-centred care.*

IHI general objectives: *1. contribute towards the creation of an EU-wide health research and innovation ecosystem that facilitates translation of scientific knowledge into innovations, notably by launching at least 30 large-scale, cross-sectoral projects, focussing on health innovations; 2. foster the development of safe, effective, people-centred and cost-effective innovations that respond to strategic unmet public health needs, by exhibiting, in at least 5 examples, the feasibility of integrating health care products or services, with demonstrated suitability for uptake by health care systems. The related projects should address the prevention, diagnosis, treatment and/or management of diseases affecting the EU population, including contribution to 'Europe's Beating Cancer Plan'; 3. drive cross-sectoral health innovation for a globally competitive European health industry and contribute to reaching the objectives of the new Industrial Strategy for Europe and the Pharmaceutical Strategy for Europe.*

The creation of a joint force at European level in order to improve ATMPs safety and bioproduction. To accelerate the passage of ATMP from early phase to phase 3 clinical trials and therefore access to patients of those innovative therapies.

ATMPs bioproduction, in particular, require bringing together transversal competencies to allow innovative technologies from academic laboratories to reach the later stages of clinical trials that can only be supported by industrial companies. These collaborations will be key to address common hurdles such as scalability and consistency of the bioproduction process, quality and stability of the product, and process optimisation in order to reduce ATMP costs for the patient.

4 Why should your idea become an IHI call topic?

Explain why collaboration through a cross-sectoral and multidisciplinary public private partnership is needed in particular:

Why does it require collaboration among several industry sectors (*e.g.* pharma, vaccines, biotech, medical devices, in vitro diagnostics, radiotherapy, medical imaging health ICT)?

Why does it require collaboration between private (industry) and public partners (*e.g.* academia, healthcare practitioners, patients, regulators)?

Currently, the development of most ATMPs faces difficulties in moving beyond phase 1 or 2 clinical trials, which are focusing on proving their feasibility and safety.
The promotion of academic/industrial partnerships is required to carry ATMPs (cell and tissue therapy, gene therapy, extracellular vesicles) through phase 3 clinical trials. Industrials' added value is particularly tangible in terms of financing, large-scale production capacity and logistics to ensure the quality and security of ATMPs.

The establishment of common European standards and guidelines for ATMPs has emerged (such as the CE marking) and is difficult to sustain for academic labs without the support of industrials.

The involvement of regulatory agencies, legislators is important to establish common guidelines. Patient organizations are also important to ensure the acceptability of ATMPs and new development processes by the end-users.

Why is the contribution of industry needed to achieve the expected impacts?

***Contribution of industry**: Large companies that are members of the IHI industry partners (i.e. COCIR, EFPIA, EuropaBio, MedTech Europe, Vaccines Europe) contribute to the programme, primarily through 'in-kind' contributions (e.g. their researchers' time, laboratories, data, compounds). At least 45% of each project's total costs have to be in-kind contribution.*

With the objective of increasing the frequency and number of ATMPs brought to the market it is essential to involve several stakeholders at the European level. The strike force of industrial companies is key to the development of ATMPs processes in terms of human, technological and material resources.

Industrials also have a high impact on regulatory agencies to better define and specify the current legislation and guidelines around ATMPs to create a common consistent regulatory framework.