ARM input to 'A European Health Data Space' combined evaluation roadmap/inception impact assessment

The Alliance for Regenerative Medicine (ARM, www.alliancerm.org) is the leading international advocacy organisation dedicated to realizing the promise of advanced therapy medicinal products (ATMPs). ARM promotes legislative, regulatory and reimbursement initiatives in Europe and internationally to advance this innovative and transformative sector, which includes cell therapies, gene therapies and tissue-based therapies. In its 11-year history, ARM has become the global voice of the sector, representing the interests of 370+ members worldwide and 70+ members across 15 European countries, including small and large companies, academic research institutions, major medical centres and patient groups.

As ATMPs are administered just as one or very few doses and exert a long-lasting, profound, potentially curative effect, the generation of real-world evidence (RWE) is a critical component in the evidence generation and the assessment of ATMPs across their whole lifecycle, from the early phases of their clinical development. Furthermore, a significant number of ATMPs are expected to obtain European marketing authorization in the years to come with 20 to 30 submissions per year expected by EMA from 2025. This will result in an increasingly urgent need to address inefficiencies coming from a fragmented, inconsistent and often unusable RWE ecosystem in Europe.

Due to the often rare or ultra-rare nature of the conditions that they address and to the complexity of supplying such transformative technologies, ATMPs are typically administered in a limited number of centres of expertise. In some cases, patients need to move within the EU to receive treatment in a country that is not their country of residence. In the context of ATMPs, cross-border healthcare and access are at the same time extremely needed and extremely challenging for the EU ecosystem. While the Cross Border Healthcare Directive (23/2011) and the Social Security Regulations (883/2004 and 987/2009) establish the right principles for cross-border access to ATMPs, they give only limited – if any - opportunities to EU citizens to get treated with ATMPs in countries different from their country of residence.

In July 2020, ARM made a "call to the European Commission to fast-track RWE for ATMPs" and early in 2020 published a position paper on "ARM recommendations on cross-border and regional access to Advanced Therapy Medicinal Products (ATMPs) in Europe." ARM believes that both documents are relevant to this consultation process and have been attached as annexes.

Overall, ARM is strongly supportive of the 'A European Health Data Space' initiative and the proposed new legislation. More specifically:

1) ARM is concerned about the current fragmentation of health data in the EU and believe that it represents a considerable burden to the ATMP stakeholder community.
2) Health data fragmentation comes with a significant lack of health data usability, mainly due to the lack of *interoperability through registries and other collection and analysis vehicles. All this*

largely limits the potential for doing Health Technology Assessment (HTA), for implementing innovative payment models and for realizing the promise of cross border access to ATMPs.

3) Considerable inefficiencies are also due to a proliferation of standards and platforms. These issues are hampering innovation coming from ATMPs and related health innovations, for example in developing and using best-in-class RWE.
4) The European Health Data Space legislation will need to identify solutions for secondary use of health data preserving data protection both under GDPR and national regulations.

ARM is committed to contribute to the consultation process foreseen by the Commission for this initiative and to bring the perspective of the ATMP sector.