Alliance for Regenerative Medicine

Brussels, 5 July 2018

Position statement on the proposal for a Regulation of the European Parliament and of the Council on health technology assessment and amending Directive 2011/24/EU (COM(2018) 51 final)

Advanced Therapies Medicinal Products (ATMPs) represent a new class of medicines intended to augment, repair, replace, or regenerate organs, tissues, cells, genes, and metabolic processes in the body. Only a handful of ATMPs have been approved and are commercialized to date, but we anticipate that many others will enter the market in the years to come. ATMPs hold the promise of profound and durable responses, often with just a single treatment, for patients with a diverse array of serious and costly conditions, many of which lack treatment. As ATMPs aim to alter the current practice of medicine by treating the root causes of disease and disorders, the value assessment of such highly innovative products may not be adequately captured by the methods and processes currently in place in Member States and new ways to determine how these products can be assessed and financed may need to be considered. Additionally, as the majority of ATMPs are currently developed by SMEs, these often do not have the internal resources, know-how and capacity to handle the variety of specific national or regional requirements before being able to put their product on the market in Europe.

The Alliance for Regenerative Medicine (ARM) is the preeminent global advocate for regenerative and advanced therapies. ARM fosters research, development, investment and commercialization of transformational treatments and cures for patients worldwide.

ARM is generally supportive of the proposed Regulation on health technology assessment (HTA) as published by the European Commission on 31 January 2018 (ref: COM(2018) 51 final) as it has the potential to provide the following key benefits:

- The joint clinical assessment will reduce the duplication of work when companies apply for HTA of their product due to harmonization of tools and methodologies across EU countries, thereby potentially increasing the predictability of HTA systems. This will be particularly beneficial for SMEs who do not have sufficient resources to simultaneously handle several submissions with different methodologies and processes.
- The joint clinical assessment will have a positive impact on time to market: as the timing of the joint clinical assessment will be coordinated with that of the central marketing authorization procedure and as Member States will have to adopt the conclusions of the joint clinical assessment, it may be anticipated that the subsequent national/regional P&R negotiations will be initiated and completed earlier than today.
- The joint scientific consultation will provide a consolidated view from the Member States, potentially resulting in a better alignment on clinical evidence requirements, selection of comparator(s), design of studies, etc. This will improve the feasibility, consistency and robustness of HTA-driven pre- and post-approval evidence generation.

In order to ensure these potential benefits can materialize, ARM believes that the proposed Regulation should also foresee additional provisions and safeguards, such as:

- Ensuring and controlling that Member States participating in joint clinical assessment adopt the conclusion report and do not repeat any assessment of aspects dealt with in the joint clinical assessment at national, regional or local levels. The enforcement of this key requirement should not prevent national or local HTA agencies to carry out complementary clinical analysis not included in the adopted conclusion report that would be needed to take account of the national or regional context. Such enforcement should also not interfere with the Member States' responsibilities for healthcare management and allocations of resources as they will continue to

be solely responsible for drawing conclusions on the technology added value and will retain full competence with regard to pricing and reimbursement decision-making processes.

- The benefits of the joint clinical assessments can only be achieved if they are based on a consistent and appropriate methodology that meets the needs for all products, including advanced therapies. While the methodology should build on the EUnetHTA methodological guidelines and evidence submission templates, EUnetHTA methods still need to address some of the specificities of ATMPs, including in orphan indications. A sufficient level of flexibility to manage uncertainties on clinical outcomes and a coordinated effort in the development of post-approval evidence generation, including by means of registries are key to support an adequate assessment of and timely access to the next wave of transformative therapies.
- Ensuring there is an appropriate effective mechanism for applicants to appeal after a joint clinical assessment. Whilst article 6 of the proposed Regulation describes the process to adopt the joint clinical assessment report, it is not clear whether the Applicant will have an opportunity to comment during the assessment process and whether it will have an option to appeal after the adoption of the joint clinical assessment report. An appeal mechanism is a key requirement of Council Directive 89/105/EEC (so-called Transparency Directive). Given that the joint clinical assessment report will form the basis for assessment of pricing and reimbursement decisions at national level, ARM believes that the proposed Regulation should also define an appeal mechanism to revisit the report in case of divergent opinions.

ARM understands that some Member States consider that the clinical evaluation is not separable from their pricing/reimbursement decision making process and may want to remain free to carry out their own assessments, at national and/or regional level. Should the proposed regulation be amended in this way, much of the benefit for the joint clinical assessment would be lost, with the joint clinical assessment potentially becoming an additional hurdle to product developers before re-evaluations at national and/or regional level. In this respect, ARM believes that any changes to the current proposal adding layers of assessment would significantly limit opportunities for timely access to advanced therapies in the EU.

ARM welcomes the initiative proposing this Regulation and is looking forward to further dialogue with all stakeholders from European and/or national institutions and organisations, including patients, to ensure that some of its key potential benefits such as earlier patient's access to innovative treatments can be achieved.

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The Alliance for Regenerative Medicine (ARM) is the preeminent global advocate for regenerative and advanced therapies. ARM fosters research, development, investment, and commercialization of transformational treatments and cures for patients worldwide. By leveraging the expertise of its membership, ARM empowers multiple stakeholders to promote legislative, regulatory, and public understanding of, and support for, this expanding field.

ARM convenes all stakeholders with an interest in regenerative and advanced therapies to provide a unified voice for our 290+ member organizations, including companies – especially small- to medium-sized enterprises (SMEs); academic/research institutions; non-profit organizations; patients, and other members of the advanced therapies community. Our aim is to connect all parts of the innovation lifecycle to address the unmet needs of patients, particularly through supporting commercialization objectives via legislative and policy frameworks that enable next generation therapies to reach those who need them. To learn more about ARM, visit http://www.alliancerm.org.

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