FRPath.org
Where the Roads to Accelerated Assessments Converge

FRPath.org Country and FRP Information Input Form		
Country: Europe	**Agency Name:** European Medicines Agency (EMA)	
Name of FRP: EMA Advanced Therapy Medicinal Products		
Is this FRP Proposed or Active? Active		
Date FRP was officially enacted: Click here to enter a date.		
1. Facilitates activities during development	**2. Accelerates the regulatory review process**	**3. Relies on or recognizes a prior regulatory decision**
☒	☒	☐

Is a Guidance or SOP describing how to apply this FRP publicly available?	Yes- see reference below
When should the FRP be requested?	Choose an item.
Does the agency provide assistance/advice to the sponsor?	Yes- For any product type
For which types of product(s) can this FRP be used? E.g. NMEs, generics, biologics, biosimilars, all products	Advanced therapy medicinal products (ATMPs) are medicines for human use that are based on genes, tissues or cells. They offer groundbreaking new opportunities for the treatment of disease and injury. ATMPs can be classified into three main types: (1) gene therapy medicines: these contain genes that lead to a therapeutic, prophylactic or diagnostic effect. They work by inserting 'recombinant' genes into the body, usually to treat a variety of diseases, including genetic disorders, cancer or long-term diseases. A recombinant gene is a stretch of DNA that is created in the laboratory, bringing together DNA from different sources; (2) somatic-cell therapy medicines: these contain cells or tissues that have been manipulated to change their biological characteristics or cells or tissues not intended to be used for the same essential functions in the body. They can be used to cure, diagnose or prevent diseases; (3) tissue-engineered medicines: these contain cells or tissues that have been modified so they can be used to repair, regenerate or replace human tissue; In addition, some ATMPs may contain one or more medical devices as an integral part of the medicine, which are referred to as combined ATMPs. An example of this is cells embedded in a biodegradable matrix or scaffold. All advanced therapy medicines are authorised centrally via the European Medicines Agency (EMA). They benefit from a single evaluation and authorisation procedure. As with all medicines, the Agency continues to monitor the safety and efficacy of advanced therapy medicines after they are approved and marketed. The Agency also gives scientific support to developers to help them design pharmacovigilance and risk management systems used to

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	monitor the safety of these medicines.
Must the product address an unmet medical need or serious condition?	Yes
If a fee is required, what is the amount (in US$ equivalent)	The legislation provides for scientific and financial incentives to encourage research and development in the area of advanced therapies. Developers of ATMPs can obtain reductions in the fees payable to EMA of: (1) 65% fee reduction for a request for scientific advice for ATMPs (90% for SMEs); (2) 90% fee reduction for the certification procedure. The Innovation Task Force provides a forum for informal dialogue between EMA and developers of ATMPs in the early stages of the medicine development process. Registered SMEs may approach the SME office to request a briefing meeting to discuss their planned regulatory strategy.
Total target (agency) time for assessment (calendar days)	277 days. *The principles for the accelerated assessment procedure in accordance with Article 14(9) of Regulation (EC) 726/2004 also apply for ATMPs as per the "guideline on the Procedure for Accelerated Assessment Pursuant to Article 14 (9) of Regulation (EC) No 726/2004". In case of Advanced Therapy Medicinal Product (ATMP)s, the Committee for Advanced Therapies (CAT) decides on the accelerated assessment request. The timetable will be arranged to include the review by the Committee for Advanced Therapies. The initial assessment phase will last 120 days similarly to the standard marketing authorization procedure; the second phase of assessment will last 30 days - the timetable therefore is 120 + 30 days.
Total target (company) time for responses to agency questions (If stated)	Click here to enter text.

Select one of the following (* see definitions at end of document)

Is this a verification review (a recognition pathway)?*	**Is this an abridged* review (selected dossier portions)? (a reliance pathway)?***	**Is this a full* review of all parts of the dossier?**
☐	☐	☒

If this is a reliance or recognition pathway, what are the accepted reference agencies?	No, this process is not a reliance or recognition pathway.
How many reference agency decisions are required?	Not applicable
Does this FRP require submission of Assessment Reports from prior decisions?	Choose an item.
Is a CPP (Certificate of Pharmaceutical	Choose an item.

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Product) required for approval?	
Can an alternate form of reference documentation to the CPP be used? If so, what types of documents?	Click here to enter text.
If this process is through a Regional Regulatory Initiative, which countries participate in this process?	Member States of the European Union (EU) and the European Economic Area (EEA).
Does the product have to have been marketed in another country? For a specific amount of time? If so, for how long?	Click here to enter text.
How are queries to the companies sent?	At specified times during the assessment
Are external reviewers (e.g. non-agency) involved in the assessment?	Yes- as needed
Post-authorization study commitments	Always required
For how long is the initial approval or designation valid?	Choose an item.
Any other details you wish to provide?	- As provided for in the ATMP Regulation (EC) No 1394/2007, the scientific evaluation of Marketing Authorisation Applications (MAAs) for ATMPs is primarily performed by the Committee for Advanced Therapies (CAT). The CAT prepares a draft opinion on the quality, safety and efficacy of each ATMP subject to marketing authorisation application (MAA) which is sent for final approval to the Committee for Medicinal Products for Human Use (CHMP). The CHMP recommendation is then sent to the European Commission, which adopts a decision binding in all Member States.
Date of this update	30 May 2020
References	1. Advanced therapy medicinal products: Overview. https://www.ema.europa.eu/en/human-regulatory/overview/advanced-therapy-medicinal-products-overview Accessed on 30 May 2020. 2. Procedural advice on the evaluation of advanced therapy medicinal product in accordance with Article 8 of Regulation (EC) No 1394/2007. https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/procedural-advice-evaluation-advanced-therapy-medicinal-product-accordance-article-8-regulation-ec/2007_en.pdf Accessed on 30 May 2020. 3. Support for advanced therapy developers. https://www.ema.europa.eu/en/human-

regulatory/research-development/advanced-therapies/support-advanced-therapy-developers
Accessed on 30 May 2020.

*Definitions:

Verification review: A checklist review based on recognition of a prior regulatory decision. Recognition is the routine acceptance of the regulatory decision of another regulator or other trusted institution. Recognition indicates that evidence of conformity with the regulatory requirements of economy A is sufficient to meet the regulatory requirements of economy B.

Abridged review: An abbreviated review of selected portions of the dossier and the reliance on prior assessment decisions. Reliance is the act whereby a regulatory authority in one jurisdiction may take into account/give significant weight to work performed by another regulator or other trusted institution in reaching its own decision

Full review: A comprehensive review of all components of the dossier. This may or may not be CPP-dependent. This may form part of a reliance or recognition pathway.