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Where the Roads to Accelerated Assessments Converge

FRPath.org Country and FRP Information Input Form		
Country: United States of America	**Agency Name:** United States Food and Drug Administration (USFDA)	
Name of FRP: FDA Regenerative Medicine Advanced Therapy Designation		
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?	At the time of the submission
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	As described in Section 3033 of the 21st Century Cures Act, a drug is eligible for regenerative medicine advanced therapy (RMAT) designation if: a) The drug is a regenerative medicine therapy, which is defined as a cell therapy, therapeutic tissue engineering product, human cell and tissue product, or any combination product using such therapies or products, except for those regulated solely under Section 361 of the Public Health Service Act and part 1271 of Title 21, Code of Federal Regulations; b) The drug is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and c) Preliminary clinical evidence indicates that the drug has the potential to address unmet medical needs for such disease or condition *Based on FDA's interpretation of Section 506(g) of the Food, Drug, and Cosmetic Act (as added by Section 3033 of the 21st Century Cures Act), certain human gene therapies and xenogeneic cell products may also meet the definition of a regenerative medicine therapy. *The request for RMAT designation must be made either concurrently with submission of an Investigational New Drug application (IND) or as an amendment to an existing IND. We will not grant a RMAT designation if an IND is on hold or is placed on hold during the designation review.
Must the product address an unmet medical need or serious condition?	Yes
If a fee is required, what is the amount	FDA User Fee Programs

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(in US$ equivalent)	
Total target (agency) time for assessment (calendar days)	No later than 60 calendar days after receipt of the designation request, the Office of Tissues and Advanced Therapies (OTAT) will notify the sponsor as to whether RMAT designation has been granted. If OTAT determines that the RMAT designation request was incomplete or that the drug development program does not meet the criteria for RMAT designation, OTAT will include a written description of the rationale for such determination.
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 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?	Not applicable
Is a CPP (Certificate of Pharmaceutical Product) required for approval?	Not applicable
Can an alternate form of reference documentation to the CPP be used? If so, what types of documents?	Not applicable.
If this process is through a Regional Regulatory Initiative, which countries participate in this process?	No, this process is not through a Regional Regulatory Initiative.
Does the product have to have been marketed in another country? For a specific amount of time? If so, for how long?	Not applicable.
How are queries to the companies sent?	Choose an item.
Are external reviewers (e.g. non-agency) involved in the assessment?	Choose an item.
Post-authorization study commitments	Always required
For how long is the initial approval or designation valid?	Choose an item.

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Any other details you wish to provide?	- Advantages of the RMAT designation include all the benefits of the fast track and breakthrough therapy designation programs, including early interactions with FDA. Section 506(g)(5) of the FD&C Act specifies that these early interactions may be used to discuss potential surrogate or intermediate endpoints to support accelerated approval. - A request for designation as an RMAT should describe the preliminary clinical evidence supporting designation. A description of the preliminary clinical evidence should include, for example, the conditions for product administration, outcome assessment, and patient monitoring; a description of the patients and their outcomes, including the number of patients who have received the drug; and the design, conduct, and analyses of any clinical investigations. - As with other expedited development programs, if RMAT designation has been granted but, later in development, the product no longer meets the qualifying criteria, then CBER may rescind the RMAT designation. This is because FDA needs to focus its resources on RMAT product development programs that continue to meet the program's qualifying criteria.
Date of this update	12 APRIL 2020
References	1. Regenerative Medicine Advanced Therapy Designation. https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/regenerative-medicine-advanced-therapy-designation Accessed on 12 April 2020. 2. Expedited Programs for Regenerative Medicine Therapies for Serious Conditions. Guidance for Industry. https://www.fda.gov/media/120267/download Accessed on 12 April 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.

By submitting this form, I agree that the information is true to the best of my knowledge and I consent that it can be used without restriction by FRPath.