FRPath.org
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 Orphan 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?	Before the marketing authorisation 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	The Orphan Drug Act (ODA) provides for granting special status to a drug or biological product ("drug") to treat a rare disease or condition upon request of a sponsor. This status is referred to as orphan designation (or sometimes "orphan status"). For a drug to qualify for orphan designation both the drug and the disease or condition must meet certain criteria specified in the ODA and FDA's implementing regulations at 21 CFR Part 316. Orphan designation qualifies the sponsor of the drug for various development incentives of the ODA, including tax credits for qualified clinical testing. For Verification of orphan-drug status, follow this link. *The granting of an orphan designation request does not alter the standard regulatory requirements and process for obtaining marketing approval. Safety and effectiveness of a drug must be established through adequate and well-controlled studies.
Must the product address an unmet medical need or serious condition?	Yes
If a fee is required, what is the amount (in US$ equivalent)	A human drug NDA/BLA application for an orphan drug is not subject to an application fee unless the human drug application includes an indication other than the rare condition. Form FDA 3397, the PDUFA user fee cover sheet, is designed to provide the minimum necessary information to determine whether a fee is required for review of an application (Center for Drug Evaluation and Research, "PDUFA User Fee Cover Sheet"). If an application qualifies for an orphan exemption, the applicant does not need to send the FDA a written request. The applicant should simply notify the FDA that they are claiming the orphan exemption when they complete and submit FDA Form

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	3397. This form should be included with the application or supplement, and a brief statement claiming the orphan exception should be included in the cover letter (Center for Drug Evaluation and Research, "User Fee Waivers, Reductions, and Refunds for Drug and Biological Products").
Total target (agency) time for assessment (calendar days)	Click here to enter text.
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?	The sponsor is expected to submit as part of the 'Content and format of a request for orphan-drug designation': - A summary of the regulatory status and marketing history of the drug in the United States and in foreign countries, e.g., IND and marketing application status and dispositions, what uses are under investigation and in what countries; for what indication is the drug approved in foreign countries; what adverse regulatory actions have been taken against the drug in any country. Include: - Pre-IND and IND numbers with respective indication(s)

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	- NDA and BLA numbers with respective indication(s) - EMA designation status and designated use, if applicable - Brief regulatory history for drug both inside and outside of the US - Relevant regulatory determinations for combination products - Any orphan drug designations held for the drug in other uses • Self certification • Do not include listing of all orphan drug designations for the drug and/or use held by other sponsors
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?	Longer than 5 years
Any other details you wish to provide?	- A sponsor seeking orphan designation for a drug must submit a request for designation to OOPD with the information required in 21 CFR 316.20 and 316.21. Each designation request must stand on its own merit. Sponsors requesting designation of the same drug for the same rare disease or condition as a previously designated product must submit their own data and information in support of their designation request. - Following receipt of the orphan drug designation request at OOPD, the review process is as follows: the request is assigned a designation request number, logged into OOPD database, and an acknowledgement letter is sent to the sponsor (or sponsor's agent). The assigned OOPD reviewer completes the review of the request, which may require consultation with an FDA Center. The review is forwarded to the Director of the Orphan Drug Designation Program for a second level review and concurrence. Following the OOPD Office Director's concurrence, a designation letter, a deficiency letter requesting additional information, or a denial letter is prepared for the OOPD Office Director's signature and the letter is then issued to the sponsor.

	- If a product receives an orphan drug designation, certain information (sponsor's name, address and contact information, name of drug, orphan designated use and date of designation) about the orphan designated product is posted in the searchable database on the OOPD website. Per 21CFR 316.28(b), FDA will post the generic and trade name of the drug, or if neither is available, the chemical name or a meaningful descriptive name of the drug provided by the sponsor and subject to approval by OOPD. If a designated product is approved for marketing, certain additional information is available on the website (approval date, approved indication, and exclusivity status). - A foreign sponsor is required to have a U.S. permanent-resident agent to file a request for an orphan drug designation. See 21CFR 316.22 for full details. OOPD requires that all correspondence to and from OOPD related to international sponsors go through the U.S. agent. This includes submitting subsequent annual reports after a product is designated. A U.S. agent can be anyone residing in the U.S. who is responsible for the paperwork involved with the designation request and if a designation is granted, will serve as the contact person afterwards. Generally, a U.S. sponsor is associated with a regulatory consulting firm or a contact person at a U.S. university. If the sponsor's agent changes, then OOPD must be notified immediately. - The benefits of obtaining orphan designation: Eligibility for 7-year marketing exclusivity ("orphan exclusivity") upon marketing approval.
Date of this update	12 APRIL 2020
References	1. Designating an Orphan Product: Drugs and Biological Products. https://www.fda.gov/industry/developing-products-rare-diseases-conditions/designating-orphan-product-drugs-and-biological-products Accessed on 12 April 2020. 2. Frequently Asked Questions (FAQ) About Designating an Orphan Product. https://www.fda.gov/industry/designating-orphan-product-drugs-and-biological-products/frequently-asked-questions-faq-about-designating-orphan-product Accessed on 12 April 2020.

3. Electronic Code of Federal Regulations. e-CFR data is current as of April 9, 2020. Accessed on 12 April 2020.
4. Recommended Tips for Creating an Orphan Drug Designation Application. A Webinar by the Office of Orphan Products Development (OOPD) 2018. https://www.fda.gov/media/111762/download Accessed on 12 April 2020.
5. Orphan Drugs: Understanding the FDA Approval Process. Academic Entrepreneurship for Academic and Health Scientists. Vol. 1, Issue 3, Article 13. 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.