Department of Health and Human Services
Food and Drug Administration

FDA ORPHAN DRUG DESIGNATION REQUEST FORM

Form Approved

OMB Control Number: 0910-0167
Expiration Date: January 31, 2021

See OMB Statement on final page.

*Required field

Note: Include in a separate PDF document a copy of every reference used to document prevalence, and used to support the scientific rationale for the use of the drug or biologic in the treatment of the rare disease. References obtained from public websites must include a copy of the website and the website address. The word "drug" in this form includes biologics. Additional guidance is available at the **FDA Orphan Products website**.

1. ***Date of Request** (*mm/dd/yyyy*)	2. **Designation Request Number** *(for amended requests only)*

Note: Amendment requests should include updated contact information, and address only the deficient portions of the previous request.

3. Sponsor Contact Information

*Business Country	*Primary Contact Country
*Business Name	*Primary Contact Business Name
Business Address (*if different from primary contact*)	*Primary Contact Prefix (Mr., Mrs., Ms., Dr., Sir, Mx.) *Primary Contact Degrees/Credentials *Primary Contact Name & Title *Primary Contact Address
Telephone Number *(if different from primary contact)*	*Primary Contact Office Number Ext: Primary Contact Cell Number Primary Contact Other Number
** FDA recommends that sponsors provide an e-mail address that is FDA secure. FDA will send the response to this email address. See the FDA Orphan Products website	*Primary Contact Email Address:**

4. Sponsor U.S. Resident Agent Information *(if applicable)*

Note: A foreign sponsor is required to have a U.S. permanent-resident agent in order to file a request for an orphan drug designation *(See* 21 CFR 316.22*)*. For foreign sponsors, FDA will send the response to the email address of the U.S. Resident Agent.

Business Name

Prefix (Mr., Mrs., Ms., Dr., Sir, Mx.) | Degree/Credentials

U.S. Resident Agent Name and Title | Office Number | Ext:

Address | Cell Number | Other Number

Email Address:**

5. Product Information

☐ Biologic ☐ Drug ☐ Combination Product

UNII

Chemical Name

*Generic/Descriptive Name *(if no generic name, provide a meaningful descriptive name)*

Trade Name | Code *(other)* Name

Provide an explanation as to what makes the product a combination product.

Additional Information

6. Manufacturer

Is the sponsor the manufacturer?

☐ Yes ☐ No

Name

Address

7. Requested Orphan Disease or Condition. *(Identify it with specificity)*

Note: Designation is given to a drug / biologic for the treatment, diagnosis, or prevention of a rare disease or condition, not to proposed drug indication or how a sponsor may wish to study a drug.

Requesting Sponsor Name

This designation request is for:
☐ Treatment ☐ Prevention ☐ Diagnosis

Drug Type/Class

Diagnosis Request: (for Diagnosis only)
☐ Initial Diagnosis ☐ Management of Disease

Proposed Orphan Disease or Condition

8. Description of the Disease or Condition, the Proposed Use of the Drug, and the Reasons why such Therapy is Needed.

Describe the disease or condition in a few paragraphs. Briefly summarize the proposed use of the drug and the reasons for its use.

Add Attachment

Note: Attachments are only for tables, charts, illustrations, and photos. Do not attach text or references. Name attachments for this section starting with the number 8. **Click the paper clip on the left toolbar to view and delete attachments**.

9. Scientific Rationale Relevant to the Disease/Condition.

The scientific rationale should support a medically plausible basis for the drug to be effective in the disease/condition *(for more information see* 21 CFR 316.20(b)(4)*).*

Briefly describe the product in one paragraph detailing the active ingredient(s), the drug type/class, structure, physical/ chemical properties, route of administration and formulation.

Briefly describe in one paragraph the drug's mechanism of action explaining how the drug works in the relevant disease/ condition.

Note: Limit the data provided in the scientific rationale section to the drug and its use in the disease or condition. Data describing the use of the drug in other diseases or conditions typically are not relevant in supporting the request.

Note: For a treatment use, the study drug must be administered *after* the disease/condition has developed. For a prevention use, the study drug must be administered *before* the disease/condition develops.

Note: The scientific rationale is best supported by human data that show the clinical benefit of using the drug in patients with the rare disease or condition. If clinical data for the drug/active moiety in treating, diagnosing, or preventing the rare disease are available, present the clinical data in this section; preclinical in vitro and in vivo studies should only be briefly mentioned. Clinical data may include preliminary results as well as case reports.

Note: In absence of human data, the request for orphan drug designation may be satisfactorily supported with preclinical data using a relevant animal model for the human disease. Animal toxicology data describing the safety of the drug in animals do not provide efficacy data and are not generally relevant in supporting the scientific rationale. Only in rare situations, where there is an absence of both human data and a relevant in vivo model, will FDA consider a combination of alternative data that include the pathogenesis of the disease, a clear description of the drug and its mechanism of action specific to the disease, and supporting in vitro data.

Has the drug been evaluated in humans with the disease that is the subject of the current orphan drug designation request?

☐ Yes ☐ No

Please provide up to several paragraphs describing only the relevant clinical data to support the scientific rationale.

Has the drug been evaluated in animals with the disease that is the subject of the current orphan drug designation request?

☐ Yes ☐ No

Please provide up to several paragraphs describing only the relevant animal data to support the scientific rationale.

Are there any in vitro or other supportive data?

☐ Yes ☐ No

Please provide up to several paragraphs describing the in vitro or other supportive data to support the scientific rationale.

Add Attachment

Note: Attachments are only for tables, charts, illustrations, and photos. Do not attach text or references. Name attachments for this section starting with the number 9. **Click the paper clip on the left toolbar to view and delete attachments**.

10. Clinical Superiority. Has the same drug already been approved for that same use? *(For more information see* 21 CFR 316.3(b)(3).

☐ Yes ☐ No *If no, go to Orphan Subset.*

Note: The approved drug need not have previously been granted orphan designation to be considered the same drug *(for more information see* 21 CFR 316.3(b)(14)***).***

Note: Preliminary information or data for your proposed product should be provided to support a foundation for the hypothesis that your proposed product is clinically superior to the previously same approved drug(s). If applicable, cite all supporting publications and include them in the references.

Note: A major contribution to patient care (MC-to-PC) is intended to be a narrow category in which each determination is on a case-by-case basis. Factors that the FDA cannot consider when determining whether a drug makes a MC-to-PC include: cost of therapy and compliance to therapy.

If yes, provide a plausible hypothesis for clinical superiority based on greater effectiveness, greater safety in a substantial portion of the target population, or a major contribution to patient care, over all previously approved same drugs. List these drug(s) including brand name(s) and formulation(s).

Add Attachment

Note: Attachments are only for tables, charts, illustrations, and photos. Do not attach text or references. Name attachments for this section starting with the number 10. **Click the paper clip on the left toolbar to view and delete attachments**.

11. Orphan Subset. Is the request for an orphan subset of a common disease or condition? *(For more information see 21 CFR316.3(b)(13)).*

☐ Yes ☐ No *If no, go to Regulatory Status / Marketing History*

Note: An orphan subset is not based on the plan to study the drug for a select indication, cost of the drug, clinical trial eligibility, or disease grade or stage.

Note: An explanation for an orphan subset should include an analysis as to why the drug could not be used in the remaining population with the disease or condition.

If yes, provide an explanation to support that a feature (e.g., mechanism of action, toxicity profile, prior clinical experience) of the drug would restrict its use to the desired orphan subset of the common disease or condition.

---

12. Regulatory Status / Marketing History. *(For more information see 21 CFR 316.20(b)(7)).*

Provide the regulatory status of the drug in the U.S. Include the following:

- preIND / IND or NDA / BLA numbers if available. Provide the indication that is associated with each one.
- Any relevant regulatory determinations if the drug is a combination product.
- Any orphan drug designations you may hold for the drug in other uses.

Note: Do not include a listing of all orphan drug designations for the proposed drug and/or use held by other sponsors.

In order to assure that the regulatory section is complete, please self-certify whether or not you ever submitted a marketing application to the FDA for the same drug for the same rare disease or condition prior to the time you submitted this designation request.

Sponsor Self - Certification

Sponsor has not previously submitted a marketing application to the FDA for the same drug for the same rare disease or condition prior to the submission of this request for orphan drug designation.

☐ Agree ☐ Disagree

Briefly provide the regulatory history outside the US. Include

- A list of the approvals and investigations of the drug outside the US.
- Whether you have submitted an European Medicines Agency (EMA) designation request for the same drug, for the same disease or condition. If so, provide the current status of the request.

---

13. Population Estimate. Document that the number of people affected by the disease or condition for which the drug is to be developed is less than 200,000 persons. “Prevalence” is defined as the number of persons in the United States who have been diagnosed as having the disease or condition at the time of the submission of the request for orphan-drug designation *(for more information see 21 CFR 316.20(b)(8) and 21 CFR 316.21(b)).*

Note: Provide all calculations and cite references used to make the population estimate.

Note: Estimate should be current at the time of the submission of the orphan drug designation request. US Census Bureau data can be used to update any population estimate.

Note: When a range of estimates exists, FDA accepts only the largest estimate unless a justification is provided why another estimate is more accurate.

Note: If the drug is intended for diagnosis or prevention of a rare disease or condition, provide the estimated number of people to whom the drug will be administered annually.

Note: If a disease is an acute condition (i.e., less than one year duration) incidence may be used as an estimate of the population. However, note that if the disease is a relapsing/remitting disease where each episode is acute in duration, a prevalence estimate may still be required.

Note: If using data from a claims database or foreign data, clearly explain how such data are generalizable to the US population and the limitations of the data.

Note: The National Cancer Institute's Surveillance, Epidemiology and End Results (SEER) Program is one recommended resource for determining cancer statistics in the United States. A complete prevalence is required.

Is the current population estimate of the disease / condition or orphan subset less than the 200,000 threshold to qualify for orphan drug designation?

☐ Yes ☐ No *(If no, go to Documentation for No Cost Recovery)*

Population Estimate: Provide all calculations and cite references used to make the estimate.

Documentation for No Cost Recovery: *(for more information see 21 CFR 316.21(c)).* **Note:** Complete this section only if the number of people affected by the disease or condition for which the proposed drug is greater than 200,000.

Provide documentation for No Cost Recovery

Add Attachment

Note: Attachments are only for tables, charts, illustrations, and photos. Do not attach text or references. Name attachments for this section starting with the number 13. **Click the paper clip on the left toolbar to view and delete attachments**.

This section applies only to requirements of the Paperwork Reduction Act of 1995.

DO NOT SEND YOUR COMPLETED FORM TO THE PRA STAFF EMAIL ADDRESS BELOW.

The burden time for this collection of information is estimated to average 32 hours per response, including the time to review instructions, search existing data sources, gather and maintain the data needed and complete and review the collection of information. Send comments regarding this burden estimate or any other aspect of this information collection, including suggestions for reducing this burden, to:

Department of Health and Human Services
Food and Drug Administration
Office of Chief Information Officer
Paperwork Reduction Act (PRA) Staff
PRAStaff@fda.hhs.gov

"An agency may not conduct or sponsor, and a person is not required to respond to, a collection of information unless it displays a currently valid OMB number."