REQUEST FOR ORPHAN DRUG DESIGNATION

Name of Sponsor Investigator, MD

X Professor, Department

Institution

Date of Submission

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# **REQUEST STATEMENT**

*A statement that the sponsor requests orphan-drug designation for a rare disease or condition, which shall be identified with specificity.*

# **NAME AND ADDRESS OF THE SPONSOR**

The name and address of the sponsor; the name of the sponsor's primary contact person and/or resident agent including title, address, telephone number, and email address; the generic and trade name, if any, of the drug, or, if neither is available, the chemical name or a meaningful descriptive name of the drug; and the name and address of the source of the drug if it is not manufactured by the sponsor.

# **DESCRIPTION OF DISEASE OR CONDITION**

A description of the rare disease or condition for which the drug is being or will be investigated, the proposed use of the drug, and the reasons why such therapy is needed.

# **DESCRIPTION OF THE DRUG**

*A description of the drug, to include the identity of the active moiety if it is a drug composed of small molecules, or of the principal molecular structural features if it is composed of macromolecules; its physical and chemical properties, if these characteristics can be determined; and a discussion of the scientific rationale to establish a medically plausible basis for the use of the drug for the rare disease or condition, including all relevant data from in vitro laboratory studies, preclinical efficacy studies conducted in an animal model for the human disease or condition, and clinical experience with the drug in the rare disease or condition that are available to the sponsor, whether positive, negative, or inconclusive. Animal toxicology studies are generally not relevant to a request for orphan-drug designation. Copies of pertinent unpublished and published papers are also required.*

# **JUSTIFICATION FOR VARIATION**

Where the sponsor of a drug that is otherwise the same drug as an already approved drug seeks orphan-drug designation for the subsequent drug for the same rare disease or condition, an explanation of why the proposed variation may be clinically superior to the first drug.

# **ORPHAN SUBSET DEMONSTRATION**

*Where a sponsor requests orphan-drug designation for a drug for only a subset of persons with a particular disease or condition that otherwise affects 200,000 or more people (“orphan subset”), a demonstration that, due to one or more properties of the drug, the remaining persons with such disease or condition would not be appropriate candidates for use of the drug.*

# **REGULATORY STATUS OF THE DRUG**

*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.*

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# **DOCUMENTATION**

*Documentation, with appended authoritative references, to demonstrate that:*

*(i) The disease or condition for which the drug is intended affects fewer than 200,000 people in the United States or, if the drug is a vaccine, diagnostic drug, or preventive drug, the persons to whom the drug will be administered in the United States are fewer than 200,000 per year as specified in § 316.21(b), or*

*(ii) For a drug intended for diseases or conditions affecting 200,000 or more people, or for a vaccine, diagnostic drug, or preventive drug to be administered to 200,000 or more persons per year in the United States, there is no reasonable expectation that costs of research and development of the drug for the indication can be recovered by sales of the drug in the United States as specified in § 316.21(c).*