

§ 316.28 Publication of orphan-drug designations.

Each month FDA will update a publicly available list of drugs designated as orphan drugs. A cumulative, updated list of all designated drugs will be provided annually. These will be placed on file at the FDA Division of Dockets Management, and will contain the following information:

- (a) The name and address of the manufacturer and sponsor;
- (b) The generic name and trade name, if any, of the drug and the date of the granting of orphan-drug designation;
- (c) The rare disease or condition for which orphan-drug designation was granted; and
- (d) The proposed indication for use of the drug.

§ 316.29 Revocation of orphan-drug designation.

(a) FDA may revoke orphan-drug designation for any drug if the agency finds that:

- (1) The request for designation contained an untrue statement of material fact; or
- (2) The request for designation omitted material information required by this part; or
- (3) FDA subsequently finds that the drug in fact had not been eligible for orphan-drug designation at the time of submission of the request therefor.

(b) For an approved drug, revocation of orphan-drug designation also suspends or withdraws the sponsor's exclusive marketing rights for the drug but not the approval of the drug's marketing application.

(c) Where a drug has been designated as an orphan drug because the prevalence of a disease or condition (or, in the case of vaccines, diagnostic drugs, or preventive drugs, the target population) is under 200,000 in the United States at the time of designation, its designation will not be revoked on the ground that the prevalence of the disease or condition (or the target population) becomes more than 200,000 persons.

§ 316.30 Annual reports of holder of orphan-drug designation.

Within 14 months after the date on which a drug was designated as an or-

phan drug and annually thereafter until marketing approval, the sponsor of a designated drug shall submit a brief progress report to the FDA Office of Orphan Products Development on the drug that includes:

(a) A short account of the progress of drug development including a review of preclinical and clinical studies initiated, ongoing, and completed and a short summary of the status or results of such studies.

(b) A description of the investigational plan for the coming year, as well as any anticipated difficulties in development, testing, and marketing; and

(c) A brief discussion of any changes that may affect the orphan-drug status of the product. For example, for products nearing the end of the approval process, sponsors should discuss any disparity between the probable marketing indication and the designated indication as related to the need for an amendment to the orphan-drug designation pursuant to § 316.26.

Subpart D—Orphan-drug Exclusive Approval**§ 316.31 Scope of orphan-drug exclusive approval.**

(a) After approval of a sponsor's marketing application for a designated orphan-drug product for treatment of the rare disease or condition concerning which orphan-drug designation was granted, FDA will not approve another sponsor's marketing application for the same drug before the expiration of 7 years from the date of such approval as stated in the approval letter from FDA, except that such a marketing application can be approved sooner if, and such time as, any of the following occurs:

- (1) Withdrawal of exclusive approval or revocation of orphan-drug designation by FDA under any provision of this part; or
- (2) Withdrawal for any reason of the marketing application for the drug in question; or
- (3) Consent by the holder of exclusive approval to permit another marketing application to gain approval; or
- (4) Failure of the holder of exclusive approval to assure a sufficient quantity

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of the drug under section 527 of the act and § 316.36.

(b) If a sponsor's marketing application for a drug product is determined not to be approvable because approval is barred under section 527 of the act until the expiration of the period of exclusive marketing of another drug product, FDA will so notify the sponsor in writing.

§ 316.34 FDA recognition of exclusive approval.

(a) FDA will send the sponsor (or, the permanent-resident agent, if applicable) timely written notice recognizing exclusive approval once the marketing application for a designated orphan-drug product has been approved. The written notice will inform the sponsor of the requirements for maintaining orphan-drug exclusive approval for the full 7-year term of exclusive approval.

(b) When a marketing application is approved for a designated orphan drug that qualifies for exclusive approval, FDA will publish in its publication entitled "Approved Drug Products with Therapeutic Equivalence Evaluations" information identifying the sponsor, the drug, and the date of termination of the orphan-drug exclusive approval. A subscription to this publication and its monthly cumulative supplements is available from the Superintendent of Documents, Government Printing Office, Washington, DC 20402-9325.

§ 316.36 Insufficient quantities of orphan drugs.

(a) Under section 527 of the act, whenever the Director has reason to believe that the holder of exclusive approval cannot assure the availability of sufficient quantities of an orphan drug to meet the needs of patients with the disease or condition for which the drug was designated, the Director will so notify the holder of this possible insufficiency and will offer the holder one of the following options, which must be exercised by a time that the Director specifies:

(1) Provide the Director in writing, or orally, or both, at the Director's discretion, views and data as to how the holder can assure the availability of sufficient quantities of the orphan drug within a reasonable time to meet the

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needs of patients with the disease or condition for which the drug was designated; or

(2) Provide the Director in writing the holder's consent for the approval of other marketing applications for the same drug before the expiration of the 7-year period of exclusive approval.

(b) If, within the time that the Director specifies, the holder fails to consent to the approval of other marketing applications and if the Director finds that the holder has not shown that it can assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the drug was designated, the Director will issue a written order withdrawing the drug product's exclusive approval. This order will embody the Director's findings and conclusions and will constitute final agency action. An order withdrawing the sponsor's exclusive marketing rights may issue whether or not there are other sponsors that can assure the availability of alternative sources of supply. Once withdrawn under this section, exclusive approval may not be reinstated for that drug.

Subpart E—Open Protocols for Investigations

§ 316.40 Treatment use of a designated orphan drug.

Prospective investigators seeking to obtain treatment use of designated orphan drugs may do so as provided in § 312.34 of this chapter.

Subpart F—Availability of Information

§ 316.50 Guidance documents.

FDA's Office of Orphan Products Development will maintain and make publicly available a list of guidance documents that apply to the regulations in this part. The list is maintained on the Internet and is published annually in the FEDERAL REGISTER. A request for a copy of the list should be directed to the Office of Orphan Products Development (HF-35), Food and Drug Administration, 5600 Fishers Lane, Rockville, MD 20857.

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