



DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2011-N-0015]

Agency Information Collection Activities; Submission for Office of Management and Budget Review; Comment Request; Orphan Products Development; Food and Drug Administration Orphan Drug Designation Request Form and The Common European Medicines Agency/Food and Drug Administration Form for Orphan Medicinal Product Designation

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing that a proposed collection of information has been submitted to the Office of Management and Budget (OMB) for review and clearance under the Paperwork Reduction Act of 1995.

DATES: Fax written comments on the collection of information by [INSERT DATE 30 DAYS AFTER DATE OF PUBLICATION IN THE *FEDERAL REGISTER*].

ADDRESSES: To ensure that comments on the information collection are received, OMB recommends that written comments be faxed to the Office of Information and Regulatory Affairs, OMB, Attn: FDA Desk Officer, Fax: 202-395-7285, or emailed to oir_submission@omb.eop.gov. All comments should be identified with the OMB control number 0910-0167. Also include the FDA docket number found in brackets in the heading of this document.

FOR FURTHER INFORMATION CONTACT: Amber Sanford, Office of Operations, Food and Drug Administration, Three White Flint North, 10A-12M, 11601 Landsdown St., North Bethesda, MD 20852, 301-796-8867, PRAStaff@fda.hhs.gov.

SUPPLEMENTARY INFORMATION: In compliance with 44 U.S.C. 3507, FDA has submitted the following proposed collection of information to OMB for review and clearance.

Orphan Products Development; Food and Drug Administration Orphan Drug Designation Request Form and The Common European Medicines Agency/Food and Drug Administration Form for Orphan Medicinal Product Designation (Formerly Orphan Drugs; Common European Medicines Agency/FDA Application Form for Orphan Medicinal Product Designation (Form FDA 3671))--21 CFR Part 316

OMB Control Number 0910-0167--Extension

Sections 525 through 528 of the Federal Food, Drug, and Cosmetic Act (the FD&C Act) (21 U.S.C. 360aa-360dd) give FDA statutory authority to do the following: (1) provide recommendations on investigations required for approval of marketing applications for orphan drugs, (2) designate eligible drugs as orphan drugs, (3) set forth conditions under which a sponsor of an approved orphan drug obtains exclusive approval, and (4) encourage sponsors to make orphan drugs available for treatment on an "open protocol" basis before the drug has been approved for general marketing. The implementing regulations for these statutory requirements have been codified under part 316 (21 CFR part 316) and specify procedures that sponsors of orphan drugs use in availing themselves of the incentives provided for orphan drugs in the FD&C Act and sets forth procedures FDA will use in administering the FD&C Act with regard to orphan drugs.

Section 316.10 specifies the content and format of a request for written recommendations concerning the nonclinical laboratory studies and clinical investigations necessary for approval of marketing applications. Section 316.12 provides that, before providing such recommendations, FDA may require results of studies to be submitted for review. Section 316.14 contains provisions permitting FDA to refuse to provide written recommendations under certain circumstances. Within 90 days of any refusal, a sponsor may submit additional information specified by FDA. Based on past experience, FDA estimates that there will be one respondent to §§ 316.10, 316.12, and 316.14 requiring 50 hours of human resources annually.

Section 316.20 specifies the content and format of an orphan drug application which includes requirements that an applicant document that the disease is rare (affects fewer than 200,000 persons in the United States annually) or that the sponsor of the drug has no reasonable expectation of recovering costs of research and development of the drug. Section 316.21 specifies content of a request for orphan drug designation required for verification of orphan-drug status. Section 316.26 allows an applicant to amend the applications under certain circumstances. Based on past experience, FDA estimates 496 respondents to §§ 316.20, 316.21, and 316.26, requiring 83,700 hours of human resources annually.

The Common EMEA/FDA Application For Orphan Medicinal Product Designation form for orphan designation of drugs intended for rare diseases or conditions (Form FDA 3671) is intended to benefit sponsors who desire to seek orphan designation of drugs intended for rare diseases or conditions from both the European Commission and FDA by reducing the burden of preparing separate applications to meet the regulatory requirements in each jurisdiction. It highlights the regulatory cooperation between the United States and the European Union mandated by the Transatlantic Economic Council. The FDA Orphan Drug Designation Request

Form (Form FDA 4035) is intended to benefit sponsors who desire to seek orphan designation of drugs intended for rare diseases or conditions from only FDA. The form is a simplified method for sponsors to provide only information required by 21 CFR 316.20 for FDA to make a decision. Based on past experience, FDA estimates there will be 496 respondents using the form requiring 19,840 hours of human resources annually.

Section 316.22 specifies requirement of a permanent resident agent for foreign sponsors. Based on past experience, FDA estimates 70 respondents requiring 140 hours of human resources annually. Section 316.24(a) specifies a requirement that sponsors respond to deficiency letters from FDA on designation requests within 1 year of issuance of the deficiency letter, unless within that time frame, the sponsor requests an extension of time to respond. Based on past experience, FDA estimates 20 respondents requiring 40 hours of human resources annually.

Section 316.27 specifies content of a change in ownership of orphan-drug designation. Based on past experience, FDA estimates 63 respondents requiring 315 hours of human resources annually. Section 316.30 requires submission of annual reports, including progress reports on studies, a description of the investigational plan, and a discussion of changes that may affect orphan status. Based on number of orphan-drug designations, the number of respondents is estimated as 744 requiring 2,232 hours of human resources annually. Finally, § 316.36 describes information required of sponsor when there is insufficient quantity of approved orphan drug. Based on past experience, FDA estimates two respondents requiring 90 hours of human resources annually.

The information requested will provide the basis for an FDA determination that the drug is for a rare disease or condition and satisfies the requirements for obtaining orphan drug status.

Secondly, the information will describe the medical and regulatory history of the drug. The respondents to this collection of information are biotechnology firms, drug companies, and academic clinical researchers.

In the *Federal Register* of June 19, 2017 (82 FR 27836), FDA published a 60-day notice requesting public comment on the proposed collection of information. No comments were received.

FDA estimates the burden of this collection of information as follows:

Table 1.--Estimated Annual Reporting Burden¹

21 CFR Section/Form FDA	No. of Respondents	Annual Frequency per Response	Total Annual Responses	Hours per Response	Total Hours
Content and format when seeking written recommendations; results of studies; and amendments (§§ 316.10, 316.12, and 316.14)	1	1	1	50	50
Content and format of a request for designation; request for verification of status; amendment to designation	496	1.25	620	135	83,700
Form FDA 3671 or 4035 FDA Orphan Drug Designation Request Form (§§ 316.20, 316.21, and 316.26)		1.25	620	32	19,840
Notifications of changes in agents (§ 316.22)	70	1	70	2	140
Deficiency letters and granting orphan-drug designation (§ 316.24(a))	20	1	20	2	40
Submissions to change ownership of orphan-drug designation (§ 316.27)	63	1	63	5	315
Annual reports (§ 316.30)	744	1	744	3	2,232
Assurance of the availability of sufficient quantities of the orphan drug; holder's consent for the approval of other marketing applications for the same drug (§ 316.36)	2	3	6	15	90
Total					106,407

¹There are no capital costs or operating and maintenance costs associated with this collection of information.

FDA has experienced increases in: (1) the number of submissions to change ownership of orphan-drug designation (§ 316.27), (2) the number of annual reports (§ 316.30), and (3) assurances of the availability of sufficient quantities of the orphan drug and the holder's consent for the approval of other marketing applications for the same drug (§ 316.36).

Dated: December 6, 2017.

Leslie Kux,

Associate Commissioner for Policy.

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