



DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2014-N-0386]

Agency Information Collection Activities; Submission for Office of Management and Budget Review; Comment Request; Orphan Drugs

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA, Agency, or we) 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: Submit written comments (including recommendations) 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 submitted to <https://www.reginfo.gov/public/do/PRAMain>. Find this particular information collection by selecting "Currently under Review--Open for Public Comments" or by using the search function. The OMB control number for this information collection is 0910-0167. Also include the FDA docket number found in brackets in the heading of this document.

FOR FURTHER INFORMATION CONTACT: JonnaLynn Capezzuto, Office of Operations, Food and Drug Administration, Three White Flint North, 10A-12M, 11601 Landsdown St., North Bethesda, MD 20852, 301-796-3794, [PRASStaff@fda.hhs.gov](mailto:PRASStaff@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 Drugs

This information collection supports FDA regulations implementing sections 525, 526, 527, and 528 of the Federal Food, Drug and Cosmetic Act (FD&C Act) (21 U.S.C. 360aa, 360bb, 360cc, and 360dd), as well as related guidance. Sections 525, 526, 527, and 528 of the FD&C Act pertain to the development of drugs for rare diseases or conditions, including biological products and antibiotics, otherwise known or referred to as "Orphan Drugs." Specifically, section 525 of the FD&C Act requires written recommendations on studies required for approval of a marketing application for a drug for a rare disease or condition. The information collection in 21 CFR 316.10, 316.12, and 316.14 is approved under OMB control numbers 0910-0001 and 0910-0014. Section 526 of the FD&C Act provides for designation of drugs as orphan drugs when certain conditions are met, section 527 provides conditions under which a sponsor of an approved orphan drug enjoys exclusive FDA marketing approval for that drug for the orphan indication for a period of 7 years, and, finally, section 528 is intended to encourage sponsors to make investigational orphan drugs available for treatment of persons in need on an open protocol basis before the drug has been approved for general marketing. Open protocols may permit patients who are not part of the formal clinical investigation to obtain treatment where adequate supplies exist and no alternative effective therapy is available.

We have issued regulations in part 316 (21 CFR part 316) to implement the Orphan Drug provisions of the FD&C Act and to set forth procedures and requirements related to requesting recommendations for investigations of drugs for rare diseases or conditions, requesting designation of a drug for a rare disease or condition, or requesting exclusive approval for a drug for a rare disease or condition. To assist respondents and to be consistent with § 316.50, our Office of Orphan Products Development (OOPD) maintains and makes publicly available guidance documents that apply to the Orphan Drug provisions of the FD&C Act and regulations in part 316. The list is maintained on the internet and guidance documents are issued in

accordance with our Good Guidance Practice regulations in 21 CFR 10.115, which provide for public comment at any time.

Accordingly, we are revising the information collection to include Agency guidance. The document entitled "Meetings with the Office of Orphan Products Development: Guidance for Industry, Researchers, Patient Groups, and Food and Drug Administration Staff" provides recommendations to industry, researchers, patient groups, and other stakeholders interested in requesting a meeting, including a teleconference, with OOPD on issues related to orphan drug designation requests, humanitarian use device designation requests, rare pediatric disease designation requests, funding opportunities through the Orphan Products Grants Program and the Pediatric Device Consortia Grants Program, and orphan product patient-related topics of concern. It is also intended to assist OOPD staff in addressing such meeting requests. This guidance describes procedures for requesting, preparing, scheduling, conducting, and documenting such meetings and discusses background information we recommend be included in such requests. Information collection attendant to recommendations in the guidance are currently approved under OMB control number 0910-0787; however, for efficiency of Agency operations, we are consolidating it into this related information collection. The guidance is available at <https://www.fda.gov/media/92815/download>.

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 the information required by § 316.20 for FDA to make a decision.

During this public health emergency associated with the COVID-19 pandemic, the OOPD is providing sponsors with increased flexibility for submission of orphan drug designation requests and related submissions (amendments, annual reports, etc.). During this public health emergency, orphan drug designation, humanitarian use device designation, and rare pediatric disease designation requests and submissions may be submitted electronically by email to the

OOPD. When transmitting information to the Orphan Drug Designation Program via email, please utilize the mailbox [orphan@fda.hhs.gov](mailto:orphan@fda.hhs.gov). We recommend using the automated read receipt feature to avoid having to call to verify receipt of the email. We also strongly encourage sponsors and others who plan to email information to FDA that is considered to be private, sensitive, proprietary, or commercial confidential to send it from an FDA-secured email address, which is provided by FDA, so the transmission is encrypted. The OOPD will assume that the addresses of emails received or email addresses provided as a point of contact are FDA secure when responding to those email addresses.

In the *Federal Register* of October 2, 2020 (85 FR 62306), 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 Burden<sup>1</sup>

21 CFR Section; Activity	No. of Respondents	No. of Responses per Respondent	Total Annual Responses	Average Burden per Response	Total Hours
Content and format of a request for designation; request for verification of status; amendment to designation	534	1.25	668	135	90,180
§§ 316.20, 316.21, 316.26 (Form FDA 4035)	534	1.25	668	32	21,376
§ 316.22; Notifications of changes in agents	132	1	132	2	264
§ 316.24(a); Deficiency letters and granting orphan-drug designation	20	1	20	2	40
§ 316.27; Submissions to change ownership of orphan-drug designation	104	1	104	5	520
§ 316.30; Annual reports	744	1	744	3	2,232
§ 316.36; 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	1	3	3	15	45
Guidance Recommendations: Meeting requests to OOPD and related submission packages	2,508	1	2,508	3.595	9,016
Total					123,673

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

Based on our evaluation, we have adjusted the currently approved burden estimate we attribute to information collection activities associated with our Orphan Drug program to reflect

an increase in submissions. This notice corrects the mathematical error published in the 60-day notice, which indicated that the total burden was 123,623.

Dated: December 17, 2020.

Lauren K. Roth,

Acting Principal Associate Commissioner for Policy.

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