U.S. Food and Drug Administration
Expedited Programs for Serious Conditions--Drugs and Biologics
OMB Control No. 0910-0765 - Extension

SUPPORTING STATEMENT

Part A: Justification

1. Circumstances Making the Collection of Information Necessary

This information collection supports regulations governing Food and Drug Administration (FDA) expedited programs for serious conditions. These provisions are set forth in 21 CFR part 312, subpart E (21 CFR part 312). The subpart E regulations are intended to speed the availability of new therapies to patients with serious conditions, especially when there are no satisfactory alternative therapies, while preserving appropriate standards for safety and effectiveness. The regulations call for earlier attention to drugs that have promise in treating such conditions, including early consultation with FDA for sponsors of such products. Respondents to the information collection are sponsors of drug or biologic product applications submitted to FDA.

To assist respondents with the information collection, we developed agency guidance entitled, "*Guidance for Industry Expedited Programs for Serious Conditions – Drugs and Biologics*" (May 2014). The guidance describes threshold criteria generally applicable to expedited programs, including what is meant by serious condition, unmet medical need, and available therapy. The guidance addresses the applicability of expedited programs to rare diseases, clarification on available therapy, and additional detail on possible flexibility in manufacturing and product quality. It also clarifies the qualifying criteria for breakthrough therapy designation; provides examples of surrogate endpoints and intermediate clinical endpoints used to support accelerated approval; and priority review.

In addition, the agency guidance entitled, "*Expedited Programs for Regenerative Medicine Therapies for Serious Conditions*," (February 2019) describing the criteria for participation in the Regenerative Medicine Advanced Therapy (RMAT) program. The RMAT expedited program was approved as part of the 21st Century CURES Act, signed December 13, 2016. An RMAT product is intended to treat, modify, reverse, or cure a serious or life-threatening disease or conditions and preliminary clinical evidence indicate that the drug has the potential to address unmet medical needs for such disease or condition. This is a CBER program and is included as an expedited program available for serious conditions.

A sponsor or applicant who seeks fast track, priority, breakthrough, RMAT or accelerated approval designation review, approval is required to submit a request showing that the drug product: (1) is intended for a serious or life-threatening condition and (2) has the potential to a) address an unmet medical need, b) demonstrate substantial improvement over available therapy, or c) fill an unmet need to be approved based on a surrogate endpoint. We expect that most information to support a designation request will have been gathered under existing requirements for preparing an investigational new drug (IND), new drug application (NDA),

or biologics license application (BLA). If such information has already been submitted to us, the information may be summarized in the designation request. A designation request should include, where applicable, additional information not specified elsewhere by statute or regulation. For example, additional information may be needed to show that a product has the potential to address an unmet medical need where an approved therapy exists for the serious or life-threatening condition to be treated. Such information may include clinical data, published reports, summaries of data and reports, and a list of references. The amount of information and discussion in a designation request should be sufficient to permit a reviewer to assess whether the criteria for fast track, priority, breakthrough, RMAT or accelerated approval designation have been met.

Once FDA makes a priority, fast track, breakthrough, RMAT or accelerated approval, designation, a sponsor or applicant may submit a premeeting package that may include additional information supporting a request to participate in any of these expedited review programs for serious conditions in drugs and biologics. The premeeting package serves as background information for the meeting and should support the intended objectives of the meeting. We expect that most sponsors or applicants will have gathered such information to meet existing requirements for preparing an IND, an NDA, or a BLA. These may include descriptions of clinical safety and efficacy trials not conducted under an IND (e.g., foreign studies) and information to support a request for participation in one of the expedited approval pathways. If such information has already been submitted to us, the information may be summarized in the premeeting package.

We therefore request extension of OMB approval for the information collection provisions associated with regulations in 21 CFR 312, subpart E as applicable in the Guidance for Industry on Expedited Programs for Serious Conditions--Drugs and Biologics" and the "Guidance for Industry on Expedited Programs for Regenerative Medicine Therapies for Serious Conditions" as discussed in this supporting statement.

2. Purpose and Use of the Information Collection

We use this information to determine whether a particular drug or biological product can be designated as a drug in a fast track, priority, breakthrough, RMAT or accelerated approval drug development program and whether a drug or biological product so designated continues to meet the criteria for fast track, priority, breakthrough, RMAT or accelerated approval designation.

3. Use of Improved Information Technology and Burden Reduction

To improve the use of information technology in the submission of marketing applications for human drugs and related reports, FDA has developed and issued guidance for industry on electronic submissions. These guidance documents are available on our website at: http://www.fda.gov/drugs/GuidanceComplianceRegulatoryInformation/Guidances/default.htm.

4. Efforts to Identify Duplication and Use of Similar Information

We are unaware of duplicative information collection.

5. Impact on Small Businesses or Other Small Entities

The information collection poses no undue burden on small entities. At the same time, FDA provides assistance to small businesses in complying with statutory and regulatory requirements administered by the agency.

6. Consequences of Collecting the Information Less Frequently

Information collection is consistent with applicable statutory and regulatory requirements. There are no technical obstacles to reducing the burden.

7. Special Circumstances Relating to the Guidelines of 5 CFR 1320.5

An applicant may be required to submit to FDA proprietary trade secrets or other confidential information when submitting a drug or biological product license application or supplement. FDA has instituted security measures to protect confidential information received from manufacturers and will, to the extent permitted by law, protect this information.

8. Comments in Response to the Federal Register Notice and Efforts to Consult Outside the Agency

In accordance with 5 CFR 1320.8 (d), we published a 60-day notice for comment in the *Federal Register* of January 8, 2024 (89 FR 11011). Although one comment was received, it was not responsive to the four collection of information topics solicited.

9. Explanation of Any Payment or Gift to Respondents

There are no incentives, payments or gifts associated with this information collection.

10. Assurance of Confidentiality Provided to Respondents

In preparing this supporting statement, we consulted our Privacy Office to ensure appropriate identification and handling of information collected.

This ICR collects personally identifiable information (PII). PII is collected in the context of the subject individuals' professional capacity and the FDA-related work they perform for their employer (e.g., point of contact at a regulated entity). The PII submitted using Form 356h "Application to Market a New or Abbreviated New Drug for Human Use" and Form 1571 "Investigational New Drug Application" (IND) includes name, work email address, work telephone numbers, work fax number, and applicant DUNS number for the primary contact at a business. The PII submitted using Form 1572 "Statement of Investigator" includes name, and work email address for the primary contact at a business. Other potential

PII submitted using Forms 356h, 1571 and 1572 include identifying job title, credentials, and the country of the contact. FDA determined that although PII is collected, the collection is not subject to the Privacy Act of 1974 and the particular notice and other requirements of the Act do not apply. Specifically, FDA does not use name or any other personal identifier to retrieve records from the information collected. Through appropriate form and webpage design, FDA limited submission fields and minimized the PII collected to protect the privacy of the individuals.

Under the Freedom of Information Act (FOIA) (5 U.S.C. 552), the public has broad access to government documents. However, FOIA provides certain exemptions from mandatory public disclosure of government records (5 U.S.C. 552(b)(1-9)). FDA will make the fullest possible disclosure of records to the public, consistent with the rights of individuals to privacy, the property rights of persons in trade and confidential commercial or financial information. Data will be kept private to the extent allowed by law.

11. Justification for Sensitive Questions

The collection of information does not involve sensitive questions.

12. Estimates of Annualized Burden Hours and Cost

12a. Annualized Hour Burden Estimate

Table 1.—Estimated Annual Reporting Burden[1,2]

Activity	No. of Respondents	No of Responses per Responden	Total Annual Responses	Average Burden per Response	Total Hours
CDER					
Priority Review Designation Requests	81	1.53	124	30	3,720
Breakthrough Therapy Designation Requests	71	1.08	77	70	5,390
Fast Track Designation Requests	235	1.18	277	60	16,620
Accelerated Approval Designation	26	1.27	33	100	3,300
Premeeting Packages	163	1.01	165	100.	16,500
CDER SUBTOTAL			**676**		**42,530**
CBER					
Priority Review Designation Request	8	1	8	30	240
Breakthrough Therapy Designation Requests	15	1.1	17	70	1,190
Fast Track Designation Requests	64	1.2	77	60	4,680

RMAT Designation Requests	33	1.1	36	60	2,100
Premeeting Packages	146	1.9	277	100	27,700
CBER SUBTOTAL			**415**		**35,910**
TOTAL			**1,091**		**81,440**

[1]*There are no capital costs or operating and maintenance costs associated with this collection of information.*
[2]*Numbers have been rounded..*

Based on FY 2022 receipts, we estimate that for CDER products, 81 respondents will submit 124 requests for priority review designation annually, and we assume 30 hours is needed to prepare such a request. We estimate 71 respondents will submit 77 requests for breakthrough designation annually, and we assume 70 hours is needed to prepare such a request. We estimate that 235 respondents will submit 277 requests for fast track designation requests annually, and we assume 60 hours is required to prepare such a request. We estimate 26 respondents will submit 33 accelerated approval designation requests annually and we assume 100 hours are required to prepare such a request. Finally, CDER received 165 pre-meeting package submissions from 163 respondents. We assume 100 hours are needed to prepare a pre-meeting package.

Similarly, also based on FY 2022 receipts, we estimate that for CBER products, 8 applicants will submit 8 requests for priority review designation annually, and we assume 30 hours are required to prepare such a request. We estimate 15 respondents will submit 17 requests for breakthrough designation annually, and we assume 70 hours are needed to prepare such a request. We estimate that 64 respondents will submit 78 requests for fast track designation annually, and we assume 60 hours is required to prepare such a request. We also estimate 33 respondents will submit 35 requests for RMAT designation annually, and assume that 60 hours are needed to prepare each RMAT designation request. Finally, CBER received 283 pre-meeting package submissions from 146 respondents. We assume 100 hours are needed to prepare a pre-meeting package.

Numbers of receipts are reported by the reviewing center as derived from differing databases. The FDA totals for the information collection are derived by adding the number of responses from each of the reviewing centers.

12b. Annualized Cost Burden Estimate

We assume labor costs for the expedited programs for serious conditions designation requests are based on an average pharmaceutical industry loaded wage rate of $85.00 per hour for compiling and submitting the requests. When we multiply the total burden hours estimated above (79,070 hours) by $85/hour, we assume a total cost to respondents for these expedited programs for serious conditions of $6,720,950.

13. Estimates of Other Total Annual Costs to Respondents/Recordkeepers or Capital Costs

There are no capital, start up, operating or maintenance costs associated with this information collection.

14. Annualized Cost to the Federal Government

FDA has allocated approximately 5 FTEs to reviewing drug and biologic submissions under the expedited programs for serious conditions. Where the cost of each FTE is approximately $325,348 (fully-loaded), the total cost burden to the Federal Government is estimated at $1,626,740. These costs are supplemented by industry submission of application and program user fees for prescription drug, biologic, and generic drug products.

15. Explanation for Program Changes or Adjustments

Based on a review of the information collection since our last request for OMB approval, we have increased our burden estimate by 143 responses and 10,350 hours to reflect actual submissions we have received. We attribute these changes to increased interest in the expedited programs.

16. Plans for Tabulation and Publication and Project Time Schedule

There are no tabulated results to publish for this information collection.

17. Reason(s) Display of OMB Expiration Date

Consistent with established practice FDA will publish a *Federal Register* notice announcing OMB approval of the information collection associated with this guidance document and will display in that notice both the OMB control number and its current expiration date. In addition, the OMB control number will be displayed on the guidance document cover page and include a link to www.reginfo.gov to identify the current expiration date.

18. Exceptions to Certification for Paperwork Reduction Act Submissions

There are no exceptions to the certification.