



4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2017-D-6159]

Expedited Programs for Regenerative Medicine Therapies for Serious Conditions; Draft Guidance for Industry; Availability

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of availability.

SUMMARY: The Food and Drug Administration (FDA or Agency) is announcing the availability of a draft document entitled "Expedited Programs for Regenerative Medicine Therapies for Serious Conditions; Draft Guidance for Industry." The draft guidance, when finalized, will provide stakeholders engaged in the development of regenerative medicine therapies with FDA's current thinking on the expedited development and review of these products. The draft guidance describes the expedited programs available to sponsors of regenerative medicine therapies for serious or life-threatening diseases or conditions (referred to in the draft guidance as serious conditions), including those products designated as regenerative advanced therapies (which FDA refers to as "regenerative medicine advanced therapy" (RMAT) designation); describes how the Center for Biologics Evaluation and Research (CBER) will work with sponsors and encourage flexibility in clinical trial design to facilitate the development of data to demonstrate the safety and effectiveness of regenerative medicine therapies being developed to address unmet medical needs in patients with serious or life-threatening diseases or conditions; and describes the opportunities for sponsors of regenerative medicine therapies to interact with CBER review staff.

DATES: Submit either electronic or written comments on the draft guidance by [INSERT DATE 90 DAYS AFTER DATE OF PUBLICATION IN THE *FEDERAL REGISTER*] to ensure that the Agency considers your comment on this draft guidance before it begins work on the final version of the guidance.

ADDRESSES: You may submit comments on any guidance at any time as follows:

Electronic Submissions

Submit electronic comments in the following way:

- Federal eRulemaking Portal: <http://www.regulations.gov>. Follow the instructions for submitting comments. Comments submitted electronically, including attachments, to <http://www.regulations.gov> will be posted to the docket unchanged. Because your comment will be made public, you are solely responsible for ensuring that your comment does not include any confidential information that you or a third party may not wish to be posted, such as medical information, your or anyone else's Social Security number, or confidential business information, such as a manufacturing process. Please note that if you include your name, contact information, or other information that identifies you in the body of your comments, that information will be posted on <http://www.regulations.gov>.
- If you want to submit a comment with confidential information that you do not wish to be made available to the public, submit the comment as a written/paper submission and in the manner detailed (see "Written/Paper Submissions" and "Instructions").

Written/Paper Submissions

Submit written/paper submissions as follows:

- Mail/Hand delivery/Courier (for written/paper submissions): Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.
- For written/paper comments submitted to the Dockets Management Staff, FDA will post your comment, as well as any attachments, except for information submitted, marked and identified, as confidential, if submitted as detailed in "Instructions."

Instructions: All submissions received must include the Docket No. FDA-2017-D-6159 for "Expedited Programs for Regenerative Medicine Therapies for Serious Conditions; Draft Guidance for Industry." Received comments will be placed in the docket and, except for those submitted as "Confidential Submissions," publicly viewable at <http://www.regulations.gov> or at the Dockets Management Staff between 9 a.m. and 4 p.m., Monday through Friday.

- Confidential Submissions--To submit a comment with confidential information that you do not wish to be made publicly available, submit your comments only as a written/paper submission. You should submit two copies total. One copy will include the information you claim to be confidential with a heading or cover note that states "THIS DOCUMENT CONTAINS CONFIDENTIAL INFORMATION." The Agency will review this copy, including the claimed confidential information, in its consideration of comments. The second copy, which will have the claimed confidential information redacted/blacked out, will be available for public viewing and posted on <http://www.regulations.gov>. Submit both copies to the Dockets Management Staff. If you do not wish your name and contact information to be made publicly available, you can provide this information on the cover sheet and not in the body of your comments and you must identify this information as "confidential."

Any information marked as "confidential" will not be disclosed except in accordance with 21 CFR 10.20 and other applicable disclosure law. For more information about FDA's posting of comments to public docket, see 80 FR 56469, September 18, 2015, or access the information at: <https://www.gpo.gov/fdsys/pkg/FR-2015-09-18/pdf/2015-23389.pdf>.

Docket: For access to the docket to read background documents or the electronic and written/paper comments received, go to <https://www.regulations.gov> and insert the docket number, found in brackets in the heading of this document, into the "Search" box and follow the prompts and/or go to the Dockets Management Staff, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.

You may submit comments on any guidance at any time (see 21 CFR 10.115(g)(5)).

Submit written requests for single copies of the draft guidance to the Office of Communication, Outreach and Development, Center for Biologics Evaluation and Research (CBER), Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, Rm. 3128, Silver Spring, MD 20993-0002. Send one self-addressed adhesive label to assist the office in processing your requests. The draft guidance may also be obtained by mail by calling CBER at 1-800-835-4709 or 240-402-8010. See the SUPPLEMENTARY INFORMATION section for electronic access to the draft guidance document.

FOR FURTHER INFORMATION CONTACT: Tami Belouin, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, Rm. 7301, Silver Spring, MD 20993-0002, 240-402-7911.

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a draft document entitled "Expedited Programs for Regenerative Medicine Therapies for Serious Conditions; Draft Guidance for Industry." The draft guidance document describes the expedited programs available to sponsors of regenerative medicine therapies for serious or life-threatening diseases or conditions (referred to in the draft guidance as serious conditions), including those products designated as RMATs; provides information about the provisions in the 21st Century Cures Act (Cures Act) (Pub. L. 114-225) regarding the use of the accelerated approval pathway for regenerative medicine therapies that have been granted designation as an RMAT; describes how CBER will encourage flexibility in clinical trial design to facilitate the development of data to demonstrate the safety and effectiveness of regenerative medicine therapies that are being developed to address unmet needs in patients with serious conditions; and describes the opportunities for sponsors of regenerative medicine therapies to interact with CBER review staff.

The draft guidance document addresses regenerative medicine therapies, which are defined in section 506(g)(8) of the Federal Food, Drug, and Cosmetic Act (the FD&C Act), as amended by the Cures Act, as including cell therapies, therapeutic tissue engineering products, human cell and tissue products, and combination products using any such therapies or products, except for those regulated solely under section 361 of the Public Health Service Act (PHS Act) (42 U.S.C. 264) and 21 CFR part 1271. Gene therapies, including genetically modified cells, that lead to a durable modification of cells or tissues can meet the definition of a regenerative medicine therapy. Under section 506(g) of the FD&C Act, RMAT designation is available for a drug (i.e., a human drug, including a drug that is a biological product) that meets the definition of regenerative medicine therapy; is intended to treat, modify, reverse, or cure a serious or life-threatening disease or condition; and with respect to which preliminary clinical evidence

indicates the potential to address unmet medical needs for such disease or condition. A combination product (biologic-device, biologic-drug, or biologic-device-drug) can be eligible for RMAT designation when the biological product provides the greatest contribution to the overall intended therapeutic effects of the combination product (i.e., the primary mode of action in the combination product is conveyed by the biological product component).

Elsewhere in this issue of the *Federal Register*, FDA is announcing the availability of a document entitled “Evaluation of Devices Used with Regenerative Medicine Advanced Therapies; Draft Guidance for Industry.”

This draft guidance is being issued consistent with FDA's good guidance practices regulation (21 CFR 10.115). The draft guidance, when finalized, will represent the current thinking of FDA on "Expedited Programs for Regenerative Medicine Therapies for Serious Conditions; Draft Guidance for Industry." It does not establish any rights for any person and is not binding on FDA or the public. You can use an alternative approach if it satisfies the requirements of the applicable statutes and regulations. This guidance is not subject to Executive Order 12866.

II. Paperwork Reduction Act of 1995

This draft guidance refers to previously approved collections of information found in FDA regulations. These collections of information are subject to review by the Office of Management and Budget (OMB) under the Paperwork Reduction Act of 1995 (44 U.S.C. 3501-3520). The collections of information in 21 CFR part 312 have been approved under OMB control number 0910-0014; the collections of information regarding formal meetings in “Guidance for Formal Meetings Between the FDA and Sponsors or Applicants of PDUFA Products” have been approved under OMB control number 0910-0429; the collections of

information in 21 CFR part 601 have been approved under OMB control number 0910-0338; the collections of information for expedited programs in “Guidance for Industry: Expedited Programs for Serious Conditions--Drugs and Biologics,” have been approved under OMB control number 0910-0765; the collections of information in 21 CFR part 314 have been approved under OMB control number 0910-0001.

III. Electronic Access

Persons with access to the internet may obtain the draft guidance at either <https://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guidances/default.htm> or <https://www.regulations.gov>.

Dated: November 13, 2017.

Anna K. Abram,

Deputy Commissioner for Policy, Planning, Legislation, and Analysis.

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