
In the Federal Register of July 12, 2018 (83 FR 32309), FDA announced the availability of the draft guidance of the same title dated July 2018. FDA received several comments on the draft guidance and those comments were considered as the guidance was finalized. In addition, editorial changes were made to improve clarity. The guidance announced in this notice finalizes the draft guidance dated July 2018.

This guidance is being issued consistent with FDA’s good guidance practices regulation (21 CFR 10.115). The guidance represents FDA’s current thinking on testing of retroviral vector-based human gene therapy products for replication competent retrovirus during product manufacture and patient follow-up. 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.

II. Paperwork Reduction Act of 1995

The 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. 3501–3521). The collections of information in 21 CFR part 321 have been approved under OMB control number 0910–0014; and the collections of information in 21 CFR part 601 have been approved under OMB control number 0910–0338.

III. Electronic Access

Persons with access to the internet may obtain the guidance at either https://www.fda.gov/vaccines-blood-biologics/guidance-compliance-regulatory-information-biologics/biologics-guidances or https://www.regulations.gov.


Lowell J. Schiller,
Principal Associate Commissioner for Policy.

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an understanding of the broad spectrum of patients in a particular disease area. Almost all non-Federal members of this Committee serve as Special Government Employees, with the exception of the representatives from Industry.

III. Nomination Procedures

Any interested person may nominate one or more qualified individuals for membership on the Committee. Self-nominations are also accepted. Nominations must include a cover letter; a current, complete resume or curriculum vitae for each nominee, including current business and/or home address, telephone number, and email address, if available; and a signed copy of the Acknowledgement and Consent form available at the FDA Advisory Nomination Portal (see ADDRESSES). Nominations must specify the advisory committee for which the nominee is recommended.

Nominations must also acknowledge that the nominee is aware of the nomination unless self-nominated. FDA will ask potential candidates to provide detailed information concerning such matters related to financial holdings, employment, and research grants and/or contracts to permit evaluation of possible sources of conflicts of interest. This notice is issued under the Federal Advisory Committee Act (5 U.S.C. app. 2) and 21 CFR part 14, relating to advisory committees.


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DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA–2018–D–2258]

Human Gene Therapy for Rare Diseases; 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 final guidance entitled “Human Gene Therapy for Rare Diseases; Guidance for Industry.” The final guidance document provides recommendations to stakeholders developing a human gene therapy (GT) product intended to treat a rare disease in adult and/or pediatric patients regarding the manufacturing, preclinical, and clinical trial design issues for all phases of the clinical development program. Such information is intended to assist sponsors in designing clinical development programs for such products, where there may be limited study population size and potential feasibility and safety issues as well as issues relating to the interpretability of bioactivity/efficacy outcomes that may be unique to rare diseases or to the nature of the GT product itself. The guidance announced in this notice finalizes the draft guidance of the same title dated July 2018.


ADDRESSES: You may submit either electronic or written comments on Agency guidances at any time as follows:

Electronic Submissions
Submit electronic comments in the following way:

• Federal eRulemaking Portal: https://www.regulations.gov. Follow the instructions for submitting comments. Comments submitted electronically, including attachments, to https://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 https://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–2018–D–2258 for “Human Gene Therapy for Rare Diseases; Guidance for Industry.” Received comments will be placed in the docket and, except for those submitted as “Confidential Submissions,” publicly viewable at https://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 headings 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 https://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 dockets, see 80 FR 56469, September 18, 2015, or access the information at: https://www.govinfo.gov/content/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 guidance to the Office of Communication, Outreach and Development, Center for Biologics