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.


Lowell J. Schiller,
Principal Associate Commissioner for Policy.

[FR Doc. 2020–01659 Filed 1–29–20; 8:45 am]

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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 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.

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.
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 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 guidance document.

FOR FURTHER INFORMATION CONTACT: Shruti Modi, 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

The Orphan Drug Act of 1983 (Pub. L. 97–414) defines a rare disease as a disease or condition that affects fewer than 200,000 persons in the United States. Since most rare diseases have no approved therapies, there is a significant unmet need for effective treatments. However, developing safe and effective products to treat rare diseases can be challenging. For example, it may be more difficult to find and recruit such patients into clinical trials, and many rare diseases exhibit a number of variations or subtypes. Consequently, patients may have highly diverse clinical manifestations and rates of disease progression with unpredictable clinical courses. Despite these challenges, GT-related research and development continue to grow at a rapid rate, with several products advancing in clinical development.

FDA is announcing the availability of a document entitled “Human Gene Therapy for Rare Diseases; Guidance for Industry.” This guidance provides recommendations to stakeholders developing human 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.

In the Federal Register of July 12, 2018 (83 FR 32303), FDA announced the availability of the draft guidance of the same title. FDA received several comments on the draft guidance and those comments were considered as the guidance was finalized. The guidance announced in this notice finalizes the draft guidance dated July 2018.

Elsewhere in this issue of the Federal Register, FDA is announcing the availability of two other human gene therapy guidance documents entitled “Human Gene Therapy for Hemophilia; Guidance for Industry” and “Human Gene Therapy for Retinal Disorders; Guidance for Industry.”

This guidance is being issued consistent with FDA’s good guidance practices regulation (21 CFR 10.115). The guidance represents the current thinking of FDA on recommendations for stakeholders developing human GT products for retinal disorders affecting adult and pediatric patients. 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

This guidance refers to previously approved collections of information 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 50 have been approved under OMB control number 0910–0755; the collections of information in 21 CFR part 58 have been approved under OMB control number 0910–0119; the collections of information in 21 CFR part 312 have been approved under OMB control number 0910–0014; the collections of information in 21 CFR part 601 have been approved under OMB control number 0910–0338; the collections of information in the guidance entitled “Expedited Programs for Serious Conditions—Drugs and Biologics” have been approved under OMB control number 0910–0765; and the collections of information in the guidance entitled “Formal Meetings Between the FDA and Sponsors or Applicants” have been approved under OMB control number 0910–0429.

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.