4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket Nos. FDA-1999-D-0081, FDA-2008-D-0205, FDA-2018-D-2173, FDA-2018-D-2236, FDA-2018-D-2238, and FDA-2018-D-2258]

Draft Guidances Relating to the Development of Human Gene Therapy Products; Availability; Extension of Comment Period

AGENCY: Food and Drug Administration, HHS.

ACTION: Notification; extension of comment period.

SUMMARY: The Food and Drug Administration (FDA or the Agency) is extending the comment period for the notices of availability for six draft guidance documents relating to the development of human gene therapy products that appeared in the *Federal Register* of July 12, 2018. The Agency is taking this action in response to requests for an extension to allow interested persons additional time to submit comments and any new information.

DATES: FDA is extending the comment period on the six documents that published on July 12, 2018 (see SUPPLEMENTARY INFORMATION). Submit either electronic or written comments by December 10, 2018, to ensure that the Agency considers your comment on these draft guidances before it begins work on the final version of the guidances.

ADDRESSES: You may submit comments as follows. Please note that late, untimely filed comments will not be considered. Electronic comments must be submitted on or before December 10, 2018. The https://www.regulations.gov electronic filing system will accept comments until midnight Eastern Time at the end of December 10, 2018. Comments received by

mail/hand delivery/courier (for written/paper submissions) will be considered timely if they are postmarked or the delivery service acceptance receipt is on or before that date.

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-1999-D-0081 for "Testing of Retroviral Vector-Based Human Gene Therapy Products for Replication Competent Retrovirus During Product Manufacture and Patient Follow-up; Draft Guidance for Industry;" Docket No. FDA-2008-D-0205 for "Chemistry, Manufacturing, and Control Information for Human Gene Therapy Investigational New Drug Applications; Draft Guidance for Industry;" Docket No. FDA-2018-D-2173 for "Long Term Follow-Up After Administration of Human Gene Therapy Products; Draft Guidance for Industry;" Docket No. FDA-2018-D-2236 for "Human Gene Therapy for Retinal Disorders; Draft Guidance for Industry;" Docket No. FDA-2018-D-2238 for "Human Gene Therapy for Hemophilia; Draft Guidance for Industry;" or Docket No. FDA-2018-D-2258 for "Human Gene Therapy for Rare Diseases; Draft Guidance for Industry." Received comments, those filed in a timely manner (see ADDRESSES), 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 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 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.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.

FOR FURTHER INFORMATION CONTACT: Jenifer Stach, 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

In the *Federal Register* of July 12, 2018, FDA published notices of availability with a 90-day comment period for six draft guidance documents listed in the following table. Three of the

six draft guidance documents provide recommendations to stakeholders developing gene therapies for retinal disorders, hemophilia, and rare diseases. The remaining three guidance documents provide recommendations to sponsors manufacturing gene therapies; namely, how to provide chemistry, manufacturing and controls information for gene therapy products, additional recommendations regarding the testing for replication competent retrovirus during the manufacture of retroviral vector-based gene therapy products and during the follow-up monitoring of patients who received retroviral vector-based gene therapy products, and recommendations regarding the design of long-term follow-up observational studies for the collection of data on delayed adverse events following administration of a gene therapy product. Comments were requested on these draft guidances by October 10, 2018.

Six Draft Guidances Published July 12, 2018

Docket No.	Draft Guidance Document Title	FR Cite
FDA-1999-D-0081	Testing of Retroviral Vector-Based Human Gene Therapy Products for Replication Competent Retrovirus During Product Manufacture and Patient Follow-up; Draft Guidance for Industry	83 FR 32309
FDA-2008-D-0205	Chemistry, Manufacturing, and Control Information for Human Gene Therapy Investigational New Drug Applications; Draft Guidance for Industry	83 FR 32307
FDA-2018-D-2173	Long Term Follow-Up After Administration of Human Gene Therapy Products; Draft Guidance for Industry	83 FR 32311
FDA-2018-D-2236	Human Gene Therapy for Retinal Disorders; Draft Guidance for Industry	83 FR 32302
FDA-2018-D-2238	Human Gene Therapy for Hemophilia; Draft Guidance for Industry	83 FR 32306
FDA-2018-D-2258	Human Gene Therapy for Rare Diseases; Draft Guidance for Industry	83 FR 32303

The Agency has received requests for a 60-day extension of the comment period for the six draft guidance documents. These requests conveyed concern that the current 90-day comment period does not allow sufficient time to develop a meaningful or thoughtful response to the draft guidance documents.

FDA has considered these requests and is extending the comment period for the six draft guidance documents for 60 days, until December 10, 2018. The Agency believes that a 60-day extension allows adequate time for interested persons to submit comments.

II. References

The following references are on display in the Dockets Management Staff (see ADDRESSES) and are available for viewing by interested persons between 9 a.m. and 4 p.m., Monday through Friday; they are also available electronically at https://www.regulations.gov.

1. Letter from Robert Falb, Director, U.S. Policy and Advocacy, Alliance for Regenerative Medicine, to Dr. Peter Marks, Director, Center for Biologics Evaluation and Research, FDA (July 23, 2018).

2. Letter from Sesquile Ramon, Ph.D., Director, Science and Regulatory Affairs, Biotechnology Innovation Organization, to FDA Dockets Management Staff (August 3, 2018).

Dated: August 29, 2018.

Leslie Kux,

Associate Commissioner for Policy.

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