Therefore, approval of the applications listed in the table, and all amendments and supplements thereto, is hereby withdrawn as of August 13, 2018. Introduction or delivery for introduction into interstate commerce of products without approved new drug applications violates section 301(a) and (d) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 331(a) and (d)). Drug products that are listed in the table that are in inventory on August 13, 2018 may continue to be dispensed until the drug products have reached their expiration dates or otherwise become violative, whichever occurs first.

Dated: July 9, 2018.
Leslie Kux,
Associate Commissioner for Policy.

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration
[Docket No. FDA 2018–D–2238]

Human Gene Therapy for Hemophilia; Draft Guidance for Industry; Availability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(FDA or Agency) is announcing the availability of a draft document entitled “Human Gene Therapy for Hemophilia; Draft Guidance for Industry.” The draft guidance document provides recommendations to stakeholders developing human gene therapy (GT) products for the treatment of hemophilia. The draft guidance provides recommendations on the clinical trial design and related development of coagulation factor VIII (hemophilia A) and IX (hemophilia B) activity assays, including how to address discrepancies in factor VIII and factor IX activity assays. The draft guidance also includes recommendations regarding preclinical considerations to support development of GT products for the treatment of hemophilia.

DATES: Submit either electronic or written comments on the draft guidance by October 10, 2018 to ensure that the Agency considers your comment on this draft guidance before it begins work on the final version of the guidance.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: 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–2238 for “Human Gene Therapy for Hemophilia; Draft Guidance for Industry.” Received comments will be placed in the docket and, except for those submitted as “Confidential” and “PUBLICLY VIEWABLE” and do not contain information which the submitter has marked as “CONFIDENTIAL,” all comments will be available to the public. The docket is available for public inspection on any business day between 8 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, you should make the information public before submitting it 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.

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 20993–0602. Send one self-addressed adhesive label to assist the office in returning these requests. The draft guidance may also be obtained by mail by calling CBER at 1–
The draft guidance, when finalized, will provide recommendations regarding preclinical and related development of GT products for the treatment of hemophilia. The draft guidance provides recommendations on the clinical trial design and related development of coagulation factor VIII (hemophilia A) and IX (hemophilia B) activity assays, including how to address discrepancies in factor VIII and factor IX activity assays. The draft guidance also includes recommendations regarding preclinical considerations to support development of GT products for the treatment of hemophilia. Hemophilia therapy in the United States has progressed from replacement therapies for on-demand treatment of bleeding to prophylaxis to reduce the frequency of bleeding. GT products for hemophilia are being developed as single-dose treatments that may provide long-term expression of the missing or abnormal coagulation factor in the patient at steady levels to reduce or eliminate the need for exogenous factor replacement.

Elsewhere in this issue of the Federal Register, FDA is announcing the availability of a draft document entitled “Human Gene Therapy for Hemophilia; Draft Guidance for Industry.” The draft guidance document provides recommendations to stakeholders developing GT products for the treatment of hemophilia. The draft guidance provides recommendations on the clinical trial design and related development of coagulation factor VIII (hemophilia A) and IX (hemophilia B) activity assays, including how to address discrepancies in factor VIII and factor IX activity assays. The draft guidance also includes recommendations regarding preclinical considerations to support development of GT products for the treatment of hemophilia. Hemophilia therapy in the United States has progressed from replacement therapies for on-demand treatment of bleeding to prophylaxis to reduce the frequency of bleeding. GT products for hemophilia are being developed as single-dose treatments that may provide long-term expression of the missing or abnormal coagulation factor in the patient at steady levels to reduce or eliminate the need for exogenous factor replacement.

II. Paperwork Reduction Act of 1995

This draft 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. 3501–3520). 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 211 have been approved under OMB control number 0910–0139; 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 draft guidance at either https://www.fda.gov/BiologicsBloodVaccines/ GuidanceComplianceRegulatoryInformation/Guidances/default.htm or https://www.regulations.gov.

Dated: July 5, 2018.

Leslie Kux,
Associate Commissioner for Policy.
[FR Doc. 2018–14875 Filed 7–11–18; 8:45 am]
BILLING CODE 4164–01–P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

Chemistry, Manufacturing, and Control Information for Human Gene Therapy Investigational New Drug Applications; 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 guidance for industry entitled “Chemistry, Manufacturing, and Control (CMC) Information for Human Gene Therapy Investigational New Drug Applications (INDs): Draft Guidance for Industry.” The draft guidance document provides sponsors of a human gene therapy IND with recommendations regarding CMC information required to assure product safety, identity, quality, purity, and strength (including potency) of the investigational product. The draft guidance applies to human gene therapy products and to combination products that contain a human gene therapy in combination with a drug or device.


DATES: Submit either electronic or written comments on the draft guidance by October 10, 2018 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: 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. Since 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