DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA–2018–D–2173]

Long Term Follow-Up After Administration of Human Gene Therapy Products; Draft Guidance for Industry; Availability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the availability of a draft document entitled "Long Term Follow-Up After Administration of Human Gene Therapy Products; Draft Guidance for Industry." The draft guidance provides sponsors, who are developing a human gene therapy (GT) product, recommendations regarding the design of long term follow-up (LTFU) observational studies for the collection of data on delayed adverse events following administration of a GT product. The draft guidance, when finalized, is intended to supersede the document entitled “Guidance for Industry: Gene Therapy Clinical Trials—Observing Participants for Delayed Adverse Events” dated November 2006. This draft guidance, when finalized, is also intended to supplement the guidance entitled “Testing of Retroviral Vector-Based Human Gene Therapy Products for Replication Competent Retrovirus during Product Manufacture and Patient Follow-up; Draft Guidance for Industry.”

DATES: Submit either electronic or written comments on the draft guidance by October 10, 2018 to ensure that the Agency considers your comment on this draft guidance before it begins work on the final version of the guidance.

ADDRESS: 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, Room. 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–2173 for “Long Term Follow-Up After Administration of Human Gene Therapy Products; Draft 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 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, Room. 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 request. The draft guidance may also be obtained by mail by calling CBER at 1–800–835–4709 or 240–402–8010. See

FOR FURTHER INFORMATION CONTACT: Ann Ferrero, Maternal and Child Health Bureau (MCHB), HRSA, in one of three ways: (1) Send a request to the following address: Ann Ferrero, MCHB, HRSA 5600 Fishers Lane, Room 18N100C, Rockville, MD 20857; (2) call 301–443–3999; or (3) send an email to AFerrero@hrsa.gov.

SUPPLEMENTARY INFORMATION:

Background: The ACHDNC provides advice and recommendations to the Secretary of HHS on the development of newborn screening activities, technologies, policies, guidelines, and programs for effectively reducing morbidity and mortality in newborns and children having, or at risk for, heritable disorders. In addition, ACHDNC’s recommendations regarding inclusion of additional conditions for screening, following adoption by the Secretary, are evidence-informed preventive health services provided for in the comprehensive guidelines supported by HRSA through the Recommended Uniform Screening Panel (RUSP) pursuant to section 2713 of the Public Health Service Act (42 U.S.C. 300gg–13). Under this provision, non-grandfathered group health plans and health insurance issuers offering group or individual health insurance are required to provide insurance coverage without cost-sharing (a co-payment, co-insurance, or deductible) for preventive services for plan years (i.e., policy years) beginning on or after the date that is one year from the Secretary’s adoption of the condition for screening.

Agenda: During the August 2, 2018, meeting, the ACHDNC will discuss issues related to long-term follow-up, timeliness, education and training, the evidence-based review process, and risk assessment in newborn screening. Information about the ACHDNC, a roster of members, and the meeting agenda, as well as past meeting summaries, is located on the ACHDNC website: https://www.hrsa.gov/advisory-committees/heritable-disorders/index.html.

Public Participation: Members of the public will have the opportunity to provide comments, which are part of the official Committee record. To submit written comments or request time for an oral comment at the meeting, please register online by 12:00 p.m. ET on July 27, 2018, at http://www.achdncmeetings.org. Oral comments will be honored in the order they are requested and may be limited as time allows. Individuals associated with review groups or who plan to provide comments on similar topics may be asked to combine their comments and

forsubjectpartnerrequestsadviceandrecommendationstothesectionfor any person and is not binding on 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 parts 50 and 56 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; and the collections of information in 21 CFR part 321 have been approved under OMB control number 0910–0014.

III. Electronic Access

Persons with access to the internet may obtain the draft guidance at either https://www.fda.gov/BiologicsBloodVaccines/GuidanceCompliancem injectable; or https://www.regulations.gov. Persons with access to the internet may obtain the draft guidance at either https://www.fda.gov/BiologicsBloodVaccines/GuidanceCompliance injectable; or https://www.regulations.gov.

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Health Resources and Services Administration

Advisory Committee on Heritable Disorders in Newborns and Children

AGENCY: Health Resources and Services Administration (HRSA), Department of Health and Human Services (HHS).

ACTION: Notice of meeting.

SUMMARY: In accordance with the Federal Advisory Committee Act, this notice announces that the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC) will hold a public meeting.

DATES: Thursday, August 2, 2018, from 9:30 a.m. to 5:00 p.m. Eastern Time (ET).

ADDRESSES: This meeting is a webinar only and requires advanced registration. Please register to view the meeting at http://www.achdncmeetings.org by 12:00 p.m. ET on July 30, 2018.

FOR FURTHER INFORMATION CONTACT: Ann Ferrero, Maternal and Child Health Bureau (MCHB), HRSA, in one of three ways: (1) Send a request to the following address: Ann Ferrero, MCHB, HRSA 5600 Fishers Lane, Room 18N100C, Rockville, MD 20857; (2) call 301–443–3999; or (3) send an email to AFerrero@hrsa.gov.

SUPPLEMENTARY INFORMATION:

Background: The ACHDNC provides advice and recommendations to the Secretary of HHS on the development of newborn screening activities, technologies, policies, guidelines, and programs for effectively reducing morbidity and mortality in newborns and children having, or at risk for, heritable disorders. In addition, ACHDNC’s recommendations regarding inclusion of additional conditions for screening, following adoption by the Secretary, are evidence-informed preventive health services provided for in the comprehensive guidelines supported by HRSA through the Recommended Uniform Screening Panel (RUSP) pursuant to section 2713 of the Public Health Service Act (42 U.S.C. 300gg–13). Under this provision, non-grandfathered group health plans and health insurance issuers offering group or individual health insurance are required to provide insurance coverage without cost-sharing (a co-payment, co-insurance, or deductible) for preventive services for plan years (i.e., policy years) beginning on or after the date that is one year from the Secretary’s adoption of the condition for screening.

Agenda: During the August 2, 2018, meeting, the ACHDNC will discuss issues related to long-term follow-up, timeliness, education and training, the evidence-based review process, and risk assessment in newborn screening. Information about the ACHDNC, a roster of members, and the meeting agenda, as well as past meeting summaries, is located on the ACHDNC website: https://www.hrsa.gov/advisory-committees/heritable-disorders/index.html.

Public Participation: Members of the public will have the opportunity to provide comments, which are part of the official Committee record. To submit written comments or request time for an oral comment at the meeting, please register online by 12:00 p.m. ET on July 27, 2018, at http://www.achdncmeetings.org. Oral comments will be honored in the order they are requested and may be limited as time allows. Individuals associated with review groups or who plan to provide comments on similar topics may be asked to combine their comments and