

Staff, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852, 240-402-7500.

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Submit written requests for single copies of the draft guidance to the Center for Biologics Evaluation and Research (CBER), Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, Rm. 3103, Silver Spring, MD 20993-0002. Send one self-addressed adhesive label to assist the office in processing your requests. The draft guidance may also be obtained by mail by calling CBER at 1-800-835-4709 or 240-402-8010. See the **SUPPLEMENTARY INFORMATION** section for electronic access to the draft guidance document.

FOR FURTHER INFORMATION CONTACT: Janet Goldberg, Center for Biologics Evaluation and Research, Food and Drug Administration, 240-402-7911.

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a draft document titled “Individualized Human Genome Editing Products: Considerations for BLA Submission and Postapproval Management.” The draft guidance document, intended for sponsors and applicants developing individualized GE products, provides recommendations for how an individualized GE product that includes multiple product variants can be submitted in a single BLA under section 351(a) of the Public Health Service Act (PHS Act) (42 U.S.C. 262(a)). When finalized, this guidance will describe FDA’s current thinking regarding how to specify the extent of GE component variation in a BLA and how to add new product variants to the BLA after licensure. This guidance also addresses related topics, including potency and safety evaluation of product variants, manufacturing process validation, labeling and indication statements, and drug naming and identification. This guidance is intended to complement existing FDA guidances on GE products addressing investigational new drug (IND)-stage chemistry, manufacturing, and controls (CMC); nonclinical and clinical trial design; evidentiary standards for approval of individualized therapies; and leveraging of CMC, nonclinical, and clinical knowledge across GE programs^{1 2 3} with additional

advice and considerations on how to prepare and submit BLAs and BLA supplements for individualized GE products.

This draft guidance is being issued consistent with FDA’s good guidance practices regulation (21 CFR 10.115). The draft guidance, when finalized, will represent the current thinking of FDA on “Individualized Human Genome Editing Products: Considerations for BLA Submission and Postapproval Management.” 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.

As we develop final guidance on this topic, FDA will consider comments on costs or cost savings the guidance may generate, relevant for Executive Order 14192.

II. Paperwork Reduction Act of 1995

While this guidance contains no collection of information, it does refer to previously approved FDA collections of information. The previously approved collections of information are subject to review by the Office of Management and Budget (OMB) under the Paperwork Reduction Act of 1995 (PRA) (44 U.S.C. 3501-3521). The collections of information in 21 CFR parts 210 and 211 pertaining to manufacturing, processing, packing, and holding of finished pharmaceuticals and biological products for human use (CGMP regulations) have been approved under OMB control number 0910-0139. The collections of information in 21 CFR 312.23(a)(7)(i) pertaining to submission of proper identification, quality, purity, and strength of an investigational drug in an IND application have been approved under OMB control number 0910-0014. The collections of information in 21 CFR 600.14(b) pertaining to biological product deviation reporting is included in OMB control number 0910-0458. The collections of information in 21 CFR part 601 pertaining to biological license applications have been approved under OMB control number 0910-0338.

to Develop Individualized Therapies that Target Specific Genetic Conditions with Known Biological Cause,” February 2026, <https://www.fda.gov/media/191247/download>. When finalized, this guidance will represent FDA’s current thinking on this topic.

³ See Draft Guidance for Industry “Leveraging Prior Knowledge in the Development of Human Gene Therapy Products Incorporating Genome Editing,” June 2026, <https://www.fda.gov/media/192810/download>. When finalized, this guidance will represent FDA’s current thinking on this topic.

III. Electronic Access

Persons with access to the internet may obtain the draft guidance at <https://www.fda.gov/vaccines-blood-biologics/guidance-compliance-regulatory-information-biologics/biologics-guidances>, <https://www.fda.gov/regulatory-information/search-fda-guidance-documents>, or <https://www.regulations.gov>.

Grace R. Graham,

Deputy Commissioner for Policy, Legislation, and International Affairs.

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DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2026-N-11980]

Issuance of Priority Review Voucher; Rare Pediatric Disease Product; ATEBRIQ (Zilurgisertib)

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing the issuance of a priority review voucher to the sponsor of a rare pediatric disease product application. The Federal Food, Drug, and Cosmetic Act (FD&C Act) authorizes FDA to award priority review vouchers to sponsors of approved rare pediatric disease product applications that meet certain criteria. FDA is required to publish notice of the award of the priority review voucher. FDA has determined that ATEBRIQ (zilurgisertib), approved September 25, 2026, manufactured by Incyte Corporation meets the criteria for a priority review voucher.

FOR FURTHER INFORMATION CONTACT:

Scott Winiecki, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Silver Spring, MD 20993-0002, 301-771-1519, Scott.Winiecki@fda.hhs.gov.

SUPPLEMENTARY INFORMATION: FDA is announcing the issuance of a priority review voucher to the sponsor of an approved rare pediatric disease product application. Under section 529 of the FD&C Act (21 U.S.C. 360ff), FDA will award priority review vouchers to sponsors of approved rare pediatric disease product applications that meet certain criteria. FDA has determined ATEBRIQ (zilurgisertib), manufactured by Incyte Corporation meets the criteria for a priority review voucher.

¹ See Guidance for Industry “Human Gene Therapy Products Incorporating Human Genome Editing,” January 2024, <https://www.fda.gov/media/156894/download>.

² See Draft Guidance for Industry “Considerations for the Use of the Plausible Mechanism Framework

ATEBRIOZ (zilurgisertib) tablets for oral use is indicated to reduce the volume of total new heterotopic ossification (HO) in adults and pediatric patients aged 12 years and older with fibrodysplasia ossificans progressiva (FOP).

For further information about the Rare Pediatric Disease Priority Review Voucher Program and for a link to the full text of section 529 of the FD&C Act, go to <https://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseaseConditions/RarePediatricDiseasePriorityVoucherProgram/default.htm>. For further information about ATEBRIOZ (zilurgisertib), go to the “Drugs@FDA” website at <https://www.accessdata.fda.gov/scripts/cder/daf/>.

Grace R. Graham,

Deputy Commissioner for Policy, Legislation, and International Affairs.

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DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA–2026–N–12279]

Issuance of Priority Review Voucher; Rare Pediatric Disease Product; EMCITATE (Tiratricol)

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing the issuance of a priority review voucher to the sponsor of a rare pediatric disease product application. The Federal Food, Drug, and Cosmetic Act (FD&C Act) authorizes FDA to award priority review vouchers to sponsors of approved rare pediatric disease product applications that meet certain criteria. FDA is required to publish notice of the award of the priority review voucher. FDA has determined that EMCITATE (tiratricol), approved September 28, 2026, manufactured by Rare Thyroid Therapeutics International AB meets the criteria for a priority review voucher.

FOR FURTHER INFORMATION CONTACT: Scott Winiecki, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave. Silver Spring, MD 20993–0002, 301–771–1519 Scott.Winiecki@fda.hhs.gov.

SUPPLEMENTARY INFORMATION: FDA is announcing the issuance of a priority review voucher to the sponsor of an approved rare pediatric disease product

application. Under section 529 of the FD&C Act (21 U.S.C. 360ff), FDA will award priority review vouchers to sponsors of approved rare pediatric disease product applications that meet certain criteria. FDA has determined EMCITATE (tiratricol), manufactured by Rare Thyroid Therapeutics International AB meets the criteria for a priority review voucher. EMCITATE (tiratricol) tablet is indicated for the treatment of peripheral thyrotoxicosis in adults and pediatric patients with monocarboxylate transporter 8 (MCT8) deficiency (Allan-Herndon-Dudley syndrome).

For further information about the Rare Pediatric Disease Priority Review Voucher Program and for a link to the full text of section 529 of the FD&C Act, go to <https://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/RarePediatricDiseasePriorityVoucherProgram/default.htm>. For further information about EMCITATE (tiratricol) go to the “Drugs@FDA” website at <https://www.accessdata.fda.gov/scripts/cder/daf/>.

Grace R. Graham,

Deputy Commissioner for Policy, Legislation, and International Affairs.

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DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA–2026–D–11188]

Electronic Submission Template for Investigational Device Exemption (IDE) Applications; Draft Guidance for Sponsors and Food and Drug Administration Staff; 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 the draft guidance titled “Electronic Submission Template for Investigational Device Exemption (IDE) Applications.” FDA is issuing this draft guidance for sponsors submitting IDE applications to the Center for Devices and Radiological Health (CDRH) and Center for Biologics Evaluation and Research (CBER). It introduces those sponsors to the current publicly available resources and associated content developed to support IDE electronic submissions to FDA. This draft guidance, when finalized, is intended to represent one of several

steps in meeting FDA’s commitment to the development of electronic submission templates to serve as guided submission preparation tools for sponsors to improve submission consistency and enhance efficiency in the review process. This draft guidance is not final nor is it for implementation at this time.

DATES: Submit either electronic or written comments on the draft guidance by December 28, 2026 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–2026–D–11188 for “Electronic