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 KYGEVVI (doxecitine and doxribtimine), manufactured by UCB, Inc., meets the criteria for a priority review voucher. KYGEVVI (doxecitine and doxribtimine) powder is indicated for treatment of thymidine kinase 2 deficiency (TK2d) in adults and pediatric patients with an age of symptom onset on or before 12 years.

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 KYGEVVI (doxecitine and doxribtimine), go to the ''Drugs@FDA'' website at *https://www.accessdata.fda.gov/scripts/cder/daf/.*

Lowell Zeta,

Deputy Commissioner for Policy, Legislation, and International Affairs.

[FR Doc. 2026–01065 Filed 1–20–26; 8:45 am]

BILLING CODE 4164–01–P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA–2025–N–6951]

Notice of Approval of Product Under Voucher: Rare Pediatric Disease Priority Review Voucher; RHAPSIDO (Remibrutinib)

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice.

SUMMARY: The Food and Drug Administration (FDA) is announcing the issuance of approval of a product redeeming a priority review voucher. 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 issuance of priority review vouchers as well as the approval of products redeeming a priority review voucher. FDA has determined that RHAPSIDO (remibrutinib), approved September 30, 2025, meets the criteria for redeeming a priority review voucher.

FOR FURTHER INFORMATION CONTACT: Quyen Tran, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 22, Room 5324, Silver Spring, MD 20993–0002, 301–796–2771, *Quyen.Tran1@fda.hhs.gov.*

SUPPLEMENTARY INFORMATION: FDA is announcing the approval of a product redeeming a rare pediatric disease priority review voucher. Under section 529 of the FD&C Act (21 U.S.C. 360ff), FDA will report the issuance of rare pediatric disease priority review vouchers and the approval of products for which a voucher was redeemed. FDA has determined that the RHAPSIDO (remibrutinib) meets the redemption criteria.

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 RHAPSIDO (remibrutinib), go to the ''Drugs@FDA'' website at *https://www.accessdata.fda.gov/scripts/cder/daf/.*

Lowell M. Zeta,

Acting Deputy Commissioner for Policy, Legislation, and International Affairs.

[FR Doc. 2026–01084 Filed 1–20–26; 8:45 am]

BILLING CODE 4164–01–P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA–2025–D–2616]

Minimal Residual Disease and Complete Response in Multiple Myeloma: Use as Endpoints To Support Accelerated Approval; Draft Guidance for Industry; Availability

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of availability.

SUMMARY: The Food and Drug Administration (FDA, Agency, or we) is announcing the availability of a draft guidance for industry entitled ''Minimal Residual Disease and Complete Response in Multiple Myeloma: Use as Endpoints to Support Accelerated Approval.'' When finalized, this guidance will provide recommendations to sponsors about using minimal residual disease (MRD) and complete response (CR) in multiple myeloma as primary endpoints in trials evaluating drug and biological products intended to treat patients with multiple myeloma to support approval under accelerated approval.

DATES: Submit either electronic or written comments on the draft guidance by March 23, 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–2025–D–2616 for ''Minimal Residual Disease and Complete Response in Multiple Myeloma: Use as Endpoints to Support Accelerated Approval.'' 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, 240–402–7500.

- 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.govinfo.gov/content/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, 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 Division of Drug Information, Center for Drug Evaluation and Research, Food and Drug Administration, 10001 New Hampshire Ave., Hillandale Building, 4th Floor, Silver Spring, MD 20993–0002. Send one self-addressed adhesive label to assist that office in processing your requests. See the **SUPPLEMENTARY INFORMATION** section for electronic access to the draft guidance document.

FOR FURTHER INFORMATION CONTACT: Bindu Kanapuru, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 22, Rm. 2102, Silver Spring, MD 20993, 240–402–1279; Philip Kurs, Center for Biologics Evaluation and Research, Food and Drug Administration, 240–402–7911; Center for Devices and Radiological Health, Food and Drug Administration, *CDRHClinicalEvidence@fda.hhs.gov.*

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a draft guidance for industry entitled ''Minimal Residual Disease and Complete Response in Multiple Myeloma: Use as Endpoints to Support Accelerated Approval.'' When finalized, this guidance will provide recommendations to sponsors about using MRD and CR in multiple myeloma as primary endpoints in trials evaluating drug and biological products intended to treat patients with multiple myeloma to support approval under accelerated approval. For the purpose of this guidance, MRD endpoint refers to MRD negativity rate as assessed in the bone marrow by either flow cytometry- or sequencing-based methods in patients who have achieved a CR. The definition of CR includes patients who achieved CR or stringent CR.

Multiple myeloma is a plasma cell malignancy and accounts for 18% of all hematologic malignancies in the United States. In multiple myeloma, accelerated approval based on an endpoint of overall response rate (ORR, defined as a partial response or better) supported by duration of response has expedited access to new therapies. However, the overall response rates observed with new therapeutics have surpassed 60–70% in the relapsed or refractory setting and 90% in the newly diagnosed setting. With the improved outcomes observed in this disease area, demonstrating statistically significant difference in overall response rates may require infeasibly large clinical trials. Additionally, more sensitive response assessments will allow for continued expeditious drug development.

MRD, which is generally assessed in the bone marrow by either flow cytometry- or sequencing-based methods, can further quantify the depth of response to treatment beyond ORR or CR. MRD is a recognized prognostic biomarker; patients who attain MRD-negativity have improved long-term outcomes. The 2016 International Myeloma Working Group (IMWG) incorporated standardized definitions of MRD-negative response resulted in greater inclusion of these assessments in clinical trials. In this treatment landscape, there has been interest in the use of MRD as a primary endpoint for clinical trials intended to support regulatory decision-making as opposed to an exploratory or a secondary endpoint.

At the April 12, 2024, Oncology Drug Advisory Committee meeting, pooled analyses of clinical trial data submitted to the Agency was presented to show the relationship between MRD and long-term outcomes (*i.e.,* Progression-Free Survival (PFS) and Overall Survival (OS)). Members unanimously agreed that it is acceptable to use MRD as an endpoint to support accelerated approval of drug or biological products intended to treat multiple myeloma.

When finalized, this guidance will provide specific recommendations for designing clinical trial using MRD as an endpoint for accelerated approval. The recommendations include general drug development considerations, trial design and statistical considerations, and assay considerations for MRD evaluation.

The draft guidance also includes considerations when proposing CR as an endpoint for accelerated approval as well as other regulatory considerations. CR is also a recognized prognostic biomarker; patients who attain CR have improved long-term outcomes. FDA conducted a pooled analysis of clinical trial data, which demonstrated an association between CR and long-term outcomes (*i.e.,* PFS and OS). The 2016 IMWG response criteria incorporate standardized definitions of CR and CR rate, which have been assessed in numerous multiple myeloma trials, often as a secondary endpoint with control of Type I error. Like MRD, CR can be used as an endpoint to support accelerated approval in trials evaluating drug and biological products intended to treat patients with multiple myeloma.

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 ''Minimal Residual Disease and Complete Response in Multiple Myeloma: Use as Endpoints to Support Accelerated Approval.'' 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 201.56 and 201.57 have been approved under OMB control number 0910–0572; the collections of information in 21 CFR part 314 have been approved under OMB control number 0910–0001; the collections of information in 21 CFR part 601 have been approved under OMB control number 0910–0338.

III. Electronic Access

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

Lowell M. Zeta,

Acting Deputy Commissioner for Policy, Legislation, and International Affairs.

[FR Doc. 2026–01068 Filed 1–20–26; 8:45 am]

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DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA–2025–D–2275]

M4Q(R2) The Common Technical Document for the Registration of Pharmaceuticals for Human Use: Quality; International Council for Harmonisation; Draft Guidance for Industry; Availability

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of availability.

SUMMARY: The Food and Drug Administration (FDA, Agency, or we) is announcing the availability of a draft guidance for industry entitled ''M4Q(R2) The Common Technical Document for the Registration of Pharmaceuticals for Human Use: Quality.'' The draft guidance was prepared under the auspices of the International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH). The draft guidance is intended to establish a globally harmonized framework to organizing and presenting quality data included in registration applications for medicinal products for human use. The draft guidance updates the quality section of the common technical document (CTD) to further improve registration and life cycle management efficiency, facilitate digitalization and knowledge management, and support provisions for emerging technologies.

DATES: Submit either electronic or written comments on the draft guidance by March 23, 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–2025–D–2275 for ''M4Q(R2) The Common Technical Document for the Registration of Pharmaceuticals for Human Use: Quality.'' 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, 240–402–7500.

- 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.govinfo.gov/content/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, 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 Division of Drug Information, Center for Drug Evaluation and Research, Food and Drug Administration, 10001 New Hampshire Ave., Hillandale Building, 4th Floor, Silver Spring, MD 20993–0002. Send one self-addressed adhesive