Our STN: BLA 125806/0

ACCELERATED BLA APPROVAL
March 26, 2026

Rocket Pharmaceuticals, Inc.
Attention: Sanchali Kasbekar, PharmD
9 Cedarbrook Drive
Cranbury, NJ 08512

Dear Dr. Kasbekar:

Please refer to your Biologics License Application (BLA) received August 1, 2023, under section 351(a) of the Public Health Service Act (PHS Act) for marnetegragene autotemcel.

We acknowledge receipt of your amendment dated September 26, 2025, which constituted a complete response to our June 14, 2024, action letter.

LICENSING

We are issuing Department of Health and Human Services U.S. License No. 2328 to Rocket Pharmaceuticals, Inc., Cranbury, NJ, under the provisions of section 351(a) of the Public Health Service Act controlling the manufacture and sale of biological products and pursuant to section 506(c) of the Federal Food, Drug, and Cosmetic Act (FDCA) and the regulations for accelerated approval, 21 CFR 601.41. The license authorizes you to introduce or deliver for introduction into interstate commerce, those products for which your company has demonstrated compliance with establishment and product standards.

Under this license you are authorized to manufacture the product marnetegragene autotemcel. Marnetegragene autotemcel is indicated for the treatment of pediatric patients with severe leukocyte adhesion deficiency-I (LAD-I) due to biallelic variants in *ITGB2* without an available human leukocyte antigen (HLA)-matched sibling donor for allogeneic hematopoietic stem cell transplant.

The review of this product was associated with the following National Clinical Trial (NCT) number: NCT03812263.

ACCELERATED APPROVAL REQUIREMENTS

Under accelerated approval statutory provisions and regulations we may grant marketing approval for a biological product on the basis of adequate and well-controlled studies establishing that the biological product has an effect on a surrogate endpoint

that is reasonably likely, based on epidemiologic, therapeutic, pathophysiologic, or other evidence, to predict clinical benefit or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity. This approval requires you to study the biological product further, to verify and describe its clinical benefit, where there is uncertainty as to the relation of the surrogate endpoint to clinical benefit, or of the observed clinical benefit to ultimate outcome.

Approval under these statutory provisions and regulations requires, among other things, that you conduct adequate and well-controlled studies to verify and describe clinical benefit attributable to this product. Clinical benefit is evidenced by effects such as improved overall survival or allogeneic HSCT-free survival.

Accelerated Approval Required Studies

We remind you of your postmarketing requirements specified in your submission of March 25, 2026.

1. Submit analyses of clinical outcomes including, at a minimum, overall survival, allogeneic HSCT-free survival, and infectious outcomes, as well as biomarker changes (e.g., neutrophil CD18 expression, CD11a expression) in: 1) all treated patients with severe LAD-I currently enrolled in Study RP-L201-0121-LTFU with each patient followed to at least 10 years of age; and 2) at least 4 newly treated pediatric patients with *ITGB2*-associated, severe LAD-I who are ≤1 year of age at the time of marnetegragene autotemcel administration with each patient followed to at least 2 years of age. All endpoints should be well defined, and data collected systematically and consistently considering potential confounding variables to enable data interpretation, e.g., antibiotic use in relation to incidence of serious infections, etc. Biomarker assessments should be based on appropriately validated bioanalytical assays (as assessed in PMR 2). All analyses should include comparisons to a suitable comparator for purposes of verifying and describing the clinical benefit of marnetegragene autotemcel in *ITGB2*-associated severe LAD-I.

   Final Protocol Submission: June 30, 2026

   Interim Study Report Submission: October 31, 2030

   Study Completion: December 31, 2033

   Final Study Report Submission: June 30, 2034

2. Submit data from supplemental validation studies performed on the LAD-1 Flow Cytometry assay described in SOP-778817. This validation is needed to enable data interpretation of your confirmatory study and specifically evaluate the performance of the CD18 (6p7) and CD11a assays throughout the complete analytical range, including low cell surface expression levels, and should include

assessments of repeatability, linearity, accuracy, intermediate precision, and specificity.

Final Protocol Submission: July 31, 2026

Study Completion: December 31, 2026

Final Study Report Submission: February 27, 2027

We expect you to complete design, initiation, accrual, completion, and reporting of these studies within the framework described in your letter of March 25, 2026.

Please submit the protocol to your IND 18485, with a cross-reference letter to this BLA, STN BL 125806 explaining that these protocols were submitted to the IND. Please refer to the sequential number for each study and the submission number as shown in this letter.

You must conduct these studies with due diligence. If required postmarketing studies fail to verify that clinical benefit is conferred by marnetegragene autotemcel, or are not conducted with due diligence, including with respect to the conditions set forth below, we may withdraw this approval.

You must submit reports of the progress of each study listed above as required under section 506(c) of the FDCA to this BLA 180 days after the date of approval of this BLA and approximately every 180 days thereafter (see section 506B(a)(2) of the FDCA) (hereinafter “180-day reports”).

You are required to submit two 180-day reports per year for each open study or clinical trial required under 506(c) of the FDCA. The initial report will be a standalone submission and the subsequent report will be combined with your application’s annual status report required under section 506B(a)(1) of the FDCA and 21 CFR 601.70. The standalone 180-day report will be due 180 days after the date of approval (with a 60-day grace period). Submit the subsequent 180-day report with your application’s annual status report. Submit both of these 180-day reports each year until the final report for the corresponding study or clinical trial is submitted.

Your 180-day report must include the information listed in 21 CFR 601.70(b). FDA recommends that you use form FDA 3989 PMR/PMC Annual Status Report for Drugs and Biologics, to submit your 180-day reports. Form FDA 3989, along with instructions for completing this form, is available on the FDA Forms web page at https://www.fda.gov/about-fda/reports-manuals-forms/forms.

Your 180-day reports, including both the standalone 180-day report submitted 180 days after the date of approval and the 180-day report submitted with your annual status report, must be clearly designated as **180-Day AA PMR Progress Report.**

FDA will consider the submission of your annual status report under section 506B(a)(1) of the FDCA and 21 CFR 601.70, in addition to the submission of reports 180 days after the date of approval each year (subject to a 60-day grace period), to satisfy the periodic reporting requirement under section 506B(a)(2) of the FDCA. You are also required to submit information related to your confirmatory trial as part of your annual reporting requirement under section 506B(a)(1) of the FDCA until the FDA notifies you, in writing, that the Agency concurs that the study requirement has been fulfilled or that the study either is no longer feasible or would no longer provide useful information.

Label your annual report as an **Annual Status Report of Postmarketing Requirements/Commitments** and submit it to the FDA each year within 60 calendar days of the anniversary date of this letter until all Postmarketing Requirements and 506B Commitments are fulfilled or released.

Please submit final study report as a supplement to this BLA, STN BLA 125806. For administrative purposes, all submissions related to this postmarketing study requirement must be clearly designated as "**Subpart E Postmarketing Study Requirements**."

MANUFACTURING LOCATIONS

Under this license, you are approved to manufacture marnetegragene autotemcel drug substance and drug product at (b) (4) . facility located at (b) (4) , and lentiviral vector LV-RP-L201 at (b) (4) located at (b) (4) .

You may label your product with the proprietary name KRESLADI and market it in bags formulated at a concentration between 0.34 - 6.1 x 10^6 viable cells/mL (0.32 - 6.1 x 10^6 CD34+ cells/mL) in one or two bags, where each bag contains 30 mL of formulated drug product. The minimum recommended dose is 2.8 x 10^6 $CD34^+$ cells/kg.

ADVISORY COMMITTEE

We did not refer your application to the Cellular, Tissue, and Gene Therapies Advisory Committee because our review of information submitted in your BLA, including the clinical study design and trial results, did not raise concerns or controversial issues which would have benefitted from an advisory committee discussion.

DATING PERIOD

The dating period for marnetegragene autotemcel shall be six months from the date of manufacture when stored at ≤ -150 °C. The date of manufacture shall be defined as the date of final formulation of the drug product. Following the final formulation, no reprocessing/reworking is allowed without prior approval from the Agency. The dating period for your the LV-RP-L201 lentiviral vector shall be (b) (4) when stored at (b) (4) We have approved the stability protocol(s) in your license application for the

purpose of extending the expiration dating period of the LV-RP-L201 lentiviral vector and drug product KRESLADI under 21 CFR 601.12.

FDA LOT RELEASE

You are not currently required to submit samples or protocols of future lots of marnetegragene autotemcel to the Center for Biologics Evaluation and Research (CBER) for release by the Director, CBER, under 21 CFR 610.2(a). We will continue to monitor compliance with 21 CFR 610.1 requiring completion of tests for conformity with standards applicable to each product prior to release of each lot.

BIOLOGICAL PRODUCT DEVIATIONS

You must submit reports of biological product deviations under 21 CFR 600.14. You should identify and investigate all manufacturing deviations promptly, including those associated with processing, testing, packaging, labeling, storage, holding and distribution. If the deviation involves a distributed product, may affect the safety, purity, or potency of the product, and meets the other criteria in the regulation, you must submit a report on FORM FDA 3486 to the Director, Office of Compliance and Biologics Quality, electronically through the eBPDR web application or at the address below. Links for the instructions on completing the electronic form (eBPDR) may be found on CBER's web site at https://www.fda.gov/vaccines-blood-biologics/report-problem-center-biologics-evaluation-research/biological-product-deviations.

Food and Drug Administration
Center for Biologics Evaluation and Research
Document Control Center
10903 New Hampshire Ave.
WO71-G112
Silver Spring, MD 20993-0002

MANUFACTURING CHANGES

You must submit information to your BLA for our review and written approval under 21 CFR 601.12 for any changes in, including but not limited to, the manufacturing, testing, packaging or labeling of marnetegragene autotemcel, or in the manufacturing facilities.

LABELING

We hereby approve the draft content of labeling including Package Insert, submitted under amendment 122, dated March 25, 2026, and the draft carton and container labels submitted under amendment 116, dated March 23, 2026.

CONTENT OF LABELING

As soon as possible, but no later than 14 days from the date of this letter, please submit the final content of labeling (21 CFR 601.14) in Structured Product Labeling (SPL) format via the FDA automated drug registration and listing system, (eLIST) as described at https://www.fda.gov/industry/fda-data-standards-advisory-board/structured-product-labeling-resources. Content of labeling must be identical to the Package Insert submitted on March 25, 2026. Information on submitting SPL files using eLIST may be found in the guidance for industry *SPL Standard for Content of Labeling Technical Qs and As* at https://www.fda.gov/regulatory-information/search-fda-guidance-documents/spl-standard-content-labeling-technical-qs.

The SPL will be accessible via publicly available labeling repositories.

PACKAGE AND CONTAINER LABELS

Please electronically submit final printed package and container labels identical to the package and container labels submitted on March 23, 2026, according to the guidance for industry *Providing Regulatory Submissions in Electronic Format — Certain Human Pharmaceutical Product Applications and Related Submissions Using the eCTD Specifications* at https://www.fda.gov/regulatory-information/search-fda-guidance-documents/providing-regulatory-submissions-electronic-format-certain-human-pharmaceutical-product-applications.

All final labeling should be submitted as Product Correspondence to this BLA, STN BL 125806 at the time of use and include implementation information on Form FDA 356h.

PROMOTIONAL MATERIALS

Please note that the accelerated approval regulation concerning promotional materials (21 CFR 601.45) stipulates that all advertising and promotional labeling items that you wish to distribute in the first 120 days following approval, must have been received by FDA prior to the approval date. After approval, promotional items intended for dissemination after the first 120 days following approval must be submitted to the FDA at least 30 days prior to the anticipated distribution date. Please submit draft materials with a cover letter noting that the items are for accelerated approval, and an accompanying FORM FDA 2253 to the Advertising and Promotional Labeling Branch at the following address:

Food and Drug Administration
Center for Biologics Evaluation and Research
Document Control Center
10903 New Hampshire Ave.
WO71-G112
Silver Spring, MD 20993-0002

You must submit copies of your final advertisement and promotional labeling at the time of initial dissemination or publication, accompanied by FORM FDA 2253 (21 CFR 601.12(f)(4)).

Alternatively, you may submit promotional materials for accelerated approval products electronically in eCTD format. For more information about submitting promotional materials in eCTD format, see the draft guidance for industry *Providing Regulatory Submissions in Electronic and Non-Electronic Format—Promotional Labeling and Advertising Materials for Human Prescription Drugs* at https://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM443702.pdf.

All promotional claims must be consistent with and not contrary to approved labeling. You should not make a comparative promotional claim or claim of superiority over other products unless you have substantial evidence or substantial clinical experience to support such claims (21 CFR 202.1(e)(6)).

ADVERSE EVENT REPORTING

You must submit adverse experience reports in accordance with the adverse experience reporting requirements for licensed biological products (21 CFR 600.80) and you must submit distribution reports as described in 21 CFR 600.81. For information on adverse experience reporting, please refer to the guidance for industry *Providing Submissions in Electronic Format —Postmarketing Safety Reports* at https://www.fda.gov/regulatory-information/search-fda-guidance-documents/providing-submissions-electronic-format-postmarketing-safety-reports and FDA's Adverse Event reporting System website at http://www.fda.gov/Drugs/GuidanceComplianceRegulatoryInformation/Surveillance/AdverseDrugEffects/ucm115894.htm. For information on distribution reporting, please refer to the guidance for industry *Electronic Submission of Lot Distribution Reports* at http://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Post-MarketActivities/LotReleases/ucm061966.htm.

RARE PEDIATRIC DISEASE PRIORITY REVIEW VOUCHER

We also inform you that you have been granted a rare pediatric disease priority review voucher (PRV), as provided under section 529 of the FDCA. This PRV has been assigned a tracking number, PRV BLA 125806. All correspondences related to this voucher should refer to this tracking number.

This voucher entitles you to designate a single human drug application submitted under section 505(b)(1) of the FDCA or a single biologic application submitted under section 351 of the Public Health Service Act as qualifying for a priority review. Such an application would not have to meet any other requirements for a priority review. The list below describes the sponsor responsibilities and the parameters for using and transferring a rare pediatric disease priority review voucher.

- The sponsor who redeems the PRV must notify FDA of its intent to submit an application with a PRV at least 90 days before submission of the application and must include the date the sponsor intends to submit the application. This notification should be prominently marked, "**Notification of Intent to Submit an Application with a Rare Pediatric Disease Priority Review Voucher**."

- This PRV may be transferred, including by sale, by you to another sponsor of a human drug or biologic application. There is no limit on the number of times that the PRV may be transferred, but each person to whom the PRV is transferred must notify FDA of the change in ownership of the voucher not later than 30 days after the transfer. If you retain and redeem this PRV, you should refer to this letter as an official record of the voucher. If the PRV is transferred, the sponsor to whom the PRV has been transferred should include a copy of this letter (which will be posted on our website as are all approval letters) and proof that the PRV was transferred.

- FDA may revoke the PRV if the rare pediatric disease product for which the PRV was awarded is not marketed in the U.S. within 1 year following the date of approval.

- The sponsor of an approved rare pediatric disease product application who is awarded a PRV must submit a report to FDA no later than 5 years after approval that addresses, for each of the first 4 post-approval years:
    - the estimated population in the U.S. suffering from the rare pediatric disease for which the product was approved (both the entire population and the population aged 0 through 18 years),
    - the estimated demand in the U.S. for the product, and
    - the actual amount of product distributed in the U.S.

You may also review the requirements related to this program by visiting FDA's Rare Pediatric Disease PRV Program webpage available at https://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/RarePediatricDiseasePriorityVoucherProgram/default.htm.

PEDIATRIC REQUIREMENTS

Under the Pediatric Research Equity Act (PREA) (21 U.S.C. 355c), all applications for new active ingredients, new indications, new dosage forms, new dosing regimens, or new routes of administration are required to contain an assessment of the safety and effectiveness of the product for the claimed indication in pediatric patients unless this requirement is waived, deferred, or inapplicable.

Because the biological product for this indication has an orphan drug designation, you are exempt from this requirement.

POSTMARKETING REQUIREMENTS UNDER SECTION 505(o)

Section 505(o) of the Federal Food, Drug, and Cosmetic Act (FDCA) authorizes FDA to require holders of approved drug and biological product applications to conduct postmarketing studies and clinical trials for certain purposes, if FDA makes certain findings required by the statute (section 505(o)(3)(A), 21 U.S.C. 355(o)(3)(A)).

We have determined that an analysis of spontaneous postmarketing adverse events reported under section 505(k)(1) of the FDCA will not be sufficient to identify a serious risk of secondary malignancies after administration of marnetegragene autotemcel.

Furthermore, the pharmacovigilance system that FDA is required to maintain under section 505(k)(3) of the FDCA is not sufficient to assess this serious risk.

Therefore, based on appropriate scientific data, we have determined that you are required to conduct the following studies:

3. Submit analyses of safety data from a postmarketing, prospective, longitudinal, observational study assessing and characterizing the long-term safety risks of marnetegragene autotemcel in patients with severe LAD-I including the risk of secondary malignancies. The study will enroll a minimum of 10 patients with severe LAD-I who receive marnetegragene autotemcel. Safety data will be collected for each patient for at least 15 years after product administration.

   We acknowledge the timetable you submitted on March 23, 2026, which states that you will conduct this study according to the following schedule:

   Final Protocol Submission: June 30, 2026

   Study Completion Date: June 30, 2047

   Final Report Submission: December 31, 2047

4. An adequate leachables safety assessment for the KRESLADI drug product (DP) through its manufacturing process, storage, and in-use conditions. This assessment must include the following:
   a. Assessment of both organic and elemental extractables from the high-risk for leachables manufacturing/storage components of final DP (i.e. cumulative leachables in DP).
   b. The leachables study can be conducted by simulating the DP manufacturing process from the step with high-risk for leachables component (b) (4)

      performed using maximal hold times and temperatures through the

process, product freezing, shelf-life storage, thawing, and in-use processing.

c. A full toxicological risk assessment for the identified leachables. Since the drug product is specifically intended for pediatric use, the permitted daily exposure (PDE) or comparator values should be calculated using the worst-case body weight assumption (e.g., 5-kg) for pediatric subjects administered the LV-RP-L201 drug product for the leachables toxicological risk assessment.

We acknowledge the timetable you submitted on March 25, 2026, which states that you will conduct this study according to the following schedule:

Final Protocol Submission: June 30, 2026

Study Completion Date: December 31, 2026

Final Report Submission: May 31, 2027

Please submit the protocols to your IND 18485, with a cross-reference letter to this BLA, STN BL 125806 explaining that these protocols were submitted to the IND. Please refer to the sequential number for each study/clinical trial and the submission number as shown in this letter.

Please submit final study reports to the BLA. If the information in the final study report supports a change in the labeling, the final study report must be submitted as a supplement to this BLA, STN BL 125806. For administrative purposes, all submissions related to these postmarketing studies required under section 505(o) must be submitted to this BLA and be clearly designated as:

- **Required Postmarketing Correspondence Status Update under Section 505(o)**
- **Supplement contains Required Postmarketing Final Report under Section 505(o)**

Section 505(o)(3)(E)(ii) of the FDCA requires you to report periodically on the status of any study or clinical trial required under this section. This section also requires you to periodically report to FDA on the status of any study or clinical trial otherwise undertaken to investigate a safety issue. In addition, section 506B of the FDCA and 21 CFR 601.70 require you to report annually on the status of any postmarketing commitments or required studies or clinical trials.

You must describe the status in an annual report on postmarketing studies for this product. Label your annual report as an **Annual Status Report of Postmarketing Requirements/Commitments** and submit it to the FDA each year within 60 calendar days of the anniversary date of this letter until all Requirements and Commitments

subject to the reporting requirements of section 506B of the FDCA are fulfilled or released. The status report for each study should include:

- the sequential number for each study as shown in this letter;
- information to identify and describe the postmarketing requirement;
- the original milestone schedule for the requirement;
- the revised milestone schedule for the requirement, if appropriate;
- the current status of the requirement (i.e., pending, ongoing, delayed, terminated, or submitted); and,
- an explanation of the status for the study or clinical trial. The explanation should include how the study is progressing in reference to the original projected schedule, including, the patient accrual rate (i.e., number enrolled to date and the total planned enrollment).

As described in 21 CFR 601.70(e), we may publicly disclose information regarding these postmarketing studies on our website at https://www.fda.gov/Drugs/GuidanceComplianceRegulatoryInformation/Post-marketingPhaseIVCommitments/default.htm.

We will consider the submission of your annual report under section 506B of the FDCA and 21 CFR 601.70 to satisfy the periodic reporting requirement under section 505(o)(3)(E)(ii) provided that you include the elements listed in section 505(o) and 21 CFR 601.70. We remind you that to comply with section 505(o), your annual report must also include a report on the status of any study or clinical trial otherwise undertaken to investigate a safety issue. Failure to periodically report on the status of studies or clinical trials required under section 505(o) may be a violation of FDCA section 505(o)(3)(E)(ii) and could result in regulatory action.

POSTMARKETING COMMITMENTS NOT SUBJECT TO THE REPORTING REQUIREMENTS UNDER SECTION 506B

We acknowledge your written commitments as described in your correspondence of March 19, 2026, as outlined below:

5. Rocket Pharmaceuticals, Inc. commits to conducting an additional in-use stability study to support in-use conditions for KRESLADI from the start of thaw to completion of infusion as described in the approved USPI. The final study report will be submitted as a “Postmarketing Commitment – In-use Stability Final Study Report” by September 30, 2026, and revise the hold duration in the USPI as supported by the in-use stability data.

   Final Study Report Submission: September 30, 2026

6. Rocket Pharmaceuticals, Inc. commits to provide a risk assessment and perform additional studies to evaluate the impact of changes to (b) (4) . The final

study report will be submitted as a “Postmarketing Commitment – Final Study Report” by March 31, 2027.

Final Study Report Submission: March 31, 2027

7. Rocket Pharmaceuticals, Inc. commits to (b) (4) The final report will be submitted as a “Postmarketing Commitment – Final Study Report” by March 31, 2027.

   Final Study Report Submission: March 31, 2027

8. Rocket Pharmaceuticals, Inc. commits to perform a prospective revalidation of the KRESLADI drug product potency assay by (b) (4) The final validation study report will be submitted as a “Postmarketing Commitment – Final Study Report” by March 31, 2027.

   Final Study Report Submission: March 31, 2027

9. Rocket Pharmaceuticals, Inc. commits to conduct (b) (4) The final study report will be submitted as a “Postmarketing Commitment – Final Study Report” by September 30, 2026.

   Final Study Report Submission: September 30, 2026

10. Rocket Pharmaceuticals, Inc. commits to perform supplemental validation of the (b) (4) . The final validation study report will be submitted as a “Postmarketing Commitment – Final Study Report” by March 31, 2027.

    Final Study Report Submission: March 31, 2027

11. Rocket Pharmaceuticals, Inc. commits to conduct additional studies to define the (b) (4) . The final study report will be submitted as a “Postmarketing Commitment – Final Study Report” by September 30, 2026.

    Final Study Report Submission: September 30, 2026

12. Rocket Pharmaceuticals, Inc. commits to conduct additional studies to evaluate whether assay (b) (4) . The final study report will be submitted as a "Postmarketing Commitment – Final Study Report" by March 31, 2027.

    Final Study Report Submission: March 31, 2027

13. Rocket Pharmaceuticals, Inc. commits to submit the Insertion Site Analysis (ISA) assay protocol and validation report. The final study report will be submitted as a "Postmarketing Commitment – Final Study Report" by March 31, 2027.

    Final Study Report Submission: March 31, 2027

14. Rocket Pharmaceuticals Inc. commits to updating Section 14.4 of SOP-778817, which describes the "LAD-1 Flow Cytometry" assay used throughout the RP-L201-0318 clinical study to evaluate the expression of CD18, CD11a, and CD11b on neutrophils from treated patients. The updated Section 14.4 will describe (b) (4) similar to experiments performed as part of the method validation. The final study protocol will be submitted as a "Postmarketing Commitment – Final Study Protocol" by August 31, 2026.

    Final Study Protocol Submission: August 31, 2026

15. Rocket Pharmaceuticals, Inc. commits to reassessing the acceptance criterion for the (b) (4) assay performed as part of release of the LV-RP-L201 lentiviral vector after (b) (4) additional LV-RP-L201 lots are manufactured and used to generate commercial KRESLADI DP. The final study report will be submitted as a "Postmarketing Commitment – Final Study Report" by December 31, 2028.

    Final Study Report Submission: December 31, 2028

16. Rocket Pharmaceuticals, Inc. commits to implement storage and shipping of KRESLADI sterility samples at (b) (4) conduct a (b) (4) hold time study for DP lot release and provide data to support (b) (4) sample testing with current validated sterility test method. The final study report will be submitted as a "Postmarketing Commitment" by March 31, 2027.

    Final Study Report Submission: March 31, 2027

17. Rocket Pharmaceuticals, Inc. commits to conduct an additional shipping validation study, under worst-case conditions, with container closure integrity testing (CCIT) of the (b) (4) performed post-shipping. CCIT will be performed via the (b) (4) method. The validation study report will be submitted as a "Postmarketing Commitment – Final Study Report" by August 30, 2026.

    Final Study Report Submission: August 30, 2026

We request that you submit information concerning nonclinical and chemistry, manufacturing, and control postmarketing commitments and final reports to this BLA, STN BLA 125806. Please refer to the sequential number for each commitment.

Please use the following designators to prominently label all submissions, including supplements, relating to these postmarketing study commitments as appropriate:

- **Postmarketing Commitment – Correspondence Status Update**
- **Postmarketing Commitment – Final Study Report**
- **Supplement contains Postmarketing Commitment Final Study Report**

For each postmarketing commitment not subject to the reporting requirements of 21 CFR 601.70, you may report the status to FDA as a **Postmarketing Study Commitment – Correspondence Status Update**. The status report for each commitment should include:

- the sequential number for each study as shown in this letter;
- the submission number associated with this letter;
- describe what has been accomplished to fulfill the non-section 506B PMC; and,
- summarize any data collected or issues with fulfilling the non-section 506B PMC.

When you have fulfilled your commitment, submit your final report as **Postmarketing Commitment – Final Study Report** or **Supplement contains Postmarketing Commitment Final Study Report**.

POST APPROVAL FEEDBACK MEETING

New biological products qualify for a post approval feedback meeting. Such meetings are used to discuss the quality of the application and to evaluate the communication process during drug development and marketing application review. The purpose is to learn from successful aspects of the review process and to identify areas that could benefit from improvement. If you would like to have such a meeting with us, please contact the Regulatory Project Manager for this application.

Sincerely,

Melissa Mendoza, JD
Director
Office of Compliance and Biologics Quality
Center for Biologics
Evaluation and Research

Asha Das, MD
Director
Office of Clinical Evaluation
Office of Therapeutic Products
Center for Biologics
Evaluation and Research