



Our STN: BL125787/375

**SUPPLEMENT APPROVAL**

March 18, 2026

Vertex Pharmaceuticals Inc  
Attention: Brett Richardson  
50 Northern Avenue  
Boston, MA 02210

Dear Brett Richardson:

We have approved your request received September 16, 2025, to supplement your Biologics License Application (BLA) submitted under section 351(a) of the Public Health Service Act for exagamglogene autotemcel (exa-cel) (CASGEVY™) to increase the number of consecutive days allowed for the collection of CD34+ hematopoietic stem and progenitor cells (HSPCs) for manufacturing purposes from 2 to up to 3 days which is reflected in changes to Section 2 DOSAGE AND ADMINISTRATION of the Prescribing Information (PI) label.

## **LABELING**

We hereby approve the draft content of labeling Package Insert submitted under 125787/375 amendment number 4 on March 10, 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 <http://www.fda.gov/ForIndustry/DataStandards/StructuredProductLabeling/default.htm>. Content of labeling must be identical to the Package Insert, submitted on March 10, 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 <http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM072392.pdf>.

The SPL will be accessible via publicly available labeling repositories.

All final labeling should be submitted as Product Correspondence to this BLA, STN BL 125787/0 at the time of use and include implementation information on Form FDA 356h.

## **ADVERTISING AND PROMOTIONAL LABELING**

You may submit two draft copies of the proposed introductory advertising and promotional labeling with 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 advertising and promotional labeling at the time of initial dissemination or publication, accompanied by Form FDA 2253 (21 CFR 601.12(f)(4)).

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)).

For each pending supplemental application for this BLA that includes proposed revised labeling, please submit an amendment to update the proposed revised labeling with the change approved today.

We will include information contained in the above-referenced supplement in your BLA file.

Sincerely,

Bindu George, MD  
Acting Director  
Division of Clinical Evaluation Hematology  
Office of Clinical Evaluation  
Office of Therapeutic Products  
Center for Biologics Evaluation and Research