

PA DEPARTMENT OF HUMAN SERVICES  
MAAC BRIEFING DOCUMENT  
CASGEVY (EXAGAMGLOGENE AUTOTEMCEL)

**Proposed Effective Date:** November 10, 2025

Revisions are noted with a ~~strikethrough~~ for deletions and **bold and underline** for additions.

**I. Requirements for Prior Authorization of Casgevy (exagamglogene autotemcel)**

A. Prescriptions That Require Prior Authorization

All prescriptions for Casgevy (exagamglogene autotemcel) must be prior authorized.

B. Revisions to Review of Documentation for Medical Necessity

In evaluating a request for prior authorization of a prescription for Casgevy (exagamglogene autotemcel), the determination of whether the requested prescription is medically necessary will take into account whether the beneficiary:

1. Is prescribed Casgevy (exagamglogene autotemcel) for an indication that is included in the U.S. Food and Drug Administration (FDA)-approved package labeling; **AND**
2. Is age-appropriate according to FDA-approved package labeling; **AND**
3. Is prescribed a dose and number of treatments that are consistent with FDA-approved package labeling; **AND**
4. Is prescribed Casgevy (exagamglogene autotemcel) by a specialist at an authorized treatment center for Casgevy (exagamglogene autotemcel); **AND**
5. Does not have a contraindication to the prescribed drug; **AND**
6. ~~Is not a prior recipient of gene therapy or an allogeneic hematopoietic stem cell transplant~~  
**Is clinically stable for transplantation based on the prescriber's assessment;** **AND**
7. **One** of the following:
  - a. For treatment of sickle cell disease, **both** of the following:
    - i. Has sickle cell disease with a  ~~$\beta$ S/ $\beta$ S,  $\beta$ S/ $\beta$ 0, or  $\beta$ S/ $\beta$ +~~ genotype **confirmatory genetic testing**
    - ii. **One** of the following:
      - a) Has a history of vaso-occlusive episodes (e.g., pain crises, acute chest syndrome, splenic sequestration, priapism) that required a medical facility visit (e.g., emergency department, hospital)
      - b) Is currently receiving chronic transfusion therapy for recurrent vaso-occlusive episodes

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b. For treatment of transfusion-dependent  $\beta$ -thalassemia, **both** of the following:

- i. Has genetic testing confirming diagnosis of  $\beta$ -thalassemia
- ii. Has a history of at least 100 mL/kg/year or 8 transfusion episodes/year of packed red blood cell transfusions in the prior 2 years.

NOTE: If the beneficiary does not meet the clinical review guidelines listed above but, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the beneficiary, the request for prior authorization will be approved.

C. Clinical Review Process

Prior authorization personnel will review the request for prior authorization and apply the clinical guidelines in Section B. above to assess the medical necessity of a prescription for Casgevy (exagamglogene autotemcel). If the guidelines in Section B. are met, the reviewer will prior authorize the prescription. If the guidelines are not met, the prior authorization request will be referred to a physician reviewer for a medical necessity determination. Such a request for prior authorization will be approved when, in the professional judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the beneficiary.

D. Dose and Duration of Therapy

Requests for prior authorization of Casgevy (exagamglogene autotemcel) will be approved for 18 months.

E. References

1. Casgevy [prescribing information]. Boston, MA: Vertex Pharmaceuticals Incorporated; January 2024.
2. The National Institutes of Health – National Heart, Lung, and Blood Institute Evidence-Based Management of Sickle Cell Disease, Expert Panel Report, 2014. Available at: [https://www.nhlbi.nih.gov/sites/default/files/media/docs/sickle-cell-disease-report%20020816\\_0.pdf](https://www.nhlbi.nih.gov/sites/default/files/media/docs/sickle-cell-disease-report%20020816_0.pdf). Accessed March 2024.
3. Cappellini MD, Farmakis D, Porter J, Taher A, eds. 2021 Guidelines for the Management of Transfusion Dependent Thalassaemia (TDT). 4th ed. Thalassaemia International Federation (TIF). Available at: <https://thalassaemia.org.cy/>. Accessed March 2024.
4. Frangoul H, Altshuler D, Cappellini MD, et al. CRISPR-Cas9 gene editing for sickle cell disease and  $\beta$ -thalassemia. N Engl J Med. 2021;384:252-260.
5. Connor RF, Fosmarin AG, Tirnauer JS. What's new in hematology. UpToDate [internet database]. Waltham, MA: UpToDate Inc. Updated February 29, 2024. Accessed March 18, 2024.
6. Fitzjugh C. Investigational therapies for sickle cell disease. UpToDate [internet database]. DeBaun MR, Tirnauer JS, eds. Waltham, MA: UpToDate Inc. Updated December 22, 2023. Accessed March 15, 2024.