

Medical Policy

Skysona (elivaldogene autotemcel)

Policy Number: 065

	Commercial and Qualified Health Plans	MassHealth	Medicare Advantage
Authorization Required	X	X	X
No Prior Authorization			

Skysona is an autologous hematopoietic stem cell (HSC)-based gene therapy indicated for the treatment of patients with confirmed early, active cerebral adrenoleukodystrophy (CALD).

Criteria

1. Criteria for Initial Approval

Authorization of a single treatment may be granted to members 4 to 17 years of age for treatment of early, active cerebral adrenoleukodystrophy (CALD) when **ALL** of the following criteria are met:

- A. Documentation of adrenoleukodystrophy as established by meeting BOTH of the following:
 - i. Elevated plasma very long chain fatty acid levels according to the standard reference values of the performing laboratory.
 - ii. The presence of a variant in the ABCD1 gene as detected by genetic testing.
- B. Documentation of early, active CALD as defined by meeting ALL of the following:
 - i. A Loes score between 0.5 and 9 (inclusive) on the 34-point scale.
 - ii. A Neurologic Function Score (NFS) less than or equal to 1.
 - iii. Gadolinium enhancement on MRI of demyelinating lesions.
- C. The member is eligible for a hematopoietic stem cell transplant (HSCT), but an HLA-matched family donor is not available. HLA matching with family members should be explored to optimally inform transplant planning.
- D. The member has not taken anti-retroviral medication(s) for at least one month prior to mobilization, OR the expected duration for elimination of the anti-retroviral medication(s) and until all cycles of apheresis are completed.
- E. The member has not received a vaccination during the 6 weeks preceding the start of myeloablative conditioning, and until hematological recovery following treatment with Skysona.
- F. The member does not test positive for HIV-1 and HIV-2, hepatitis B virus (HBV), or hepatitis C (HCV). and Human T-lymphotropic virus 1 & 2 (HTLV-1/HTLV-2) in accordance with clinical guidelines before collection of cells for manufacturing. Documentation of planned testing is required.
- G. The member has been evaluated for hepatic function to ensure HSC transplantation is appropriate as defined by one of the following:
 - i. Alanine transaminase (ALT) value less than 2.5x ULN, or
 - ii. Aspartate transaminase (AST) value less than 2.5x the upper limit of normal (ULN), or
 - iii. Total bilirubin value less than 3.0 milligram per deciliter (mg/dL), except if there is a diagnosis of Gilbert's Syndrome and the member is otherwise stable.
- H. The member does not have any current malignancies.
- I. The member has not received Skysona or any other gene therapy.

2. Dosing and Administration
 - The recommended dose is a single dose, given intravenously, containing a minimum of 5.0×10^6 CD34+ cells/kg of body weight in which body weight is based on individual's weight prior to first apheresis.
 - Full myeloablative and lymphodepleting conditioning must be administered before infusion of Skysona.
3. Duration of Therapy
 - Single treatment course
 - The member should receive seizure, hepatic veno-occlusive disease, anti-fungal, and antibiotic prophylaxis as needed.
 - Additional courses of therapy are considered experimental/investigational.
4. Facility Criteria
 - The medication is prescribed by a hematologist, a neurologist, and/or a stem cell transplant specialist
 - The treatment will be administered at a Skysona Qualified Treatment Center.

Medicare Variation

Mass General Brigham Health Plan uses guidance from the Centers for Medicare and Medicaid Services (CMS) for coverage determinations for its Medicare Advantage plan members. National Coverage Determinations (NCDs), Local Coverage Determinations (LCDs), Local Coverage Articles (LCAs) and documentation included in the Medicare manuals are the basis for coverage determinations. When there is no guidance from CMS for the requested service, Mass General Brigham Health Plan's medical policies are used for coverage determinations.

At the time of Mass General Brigham Health Plan's most recent policy review, Medicare has an NCD for Chimeric Antigen Receptor (CAR) T-cell Therapy (110.24).

CPT/HCPC Codes

Authorized CPT/HCPCS Codes	Code Description
No specific code	

Effective

May 2023: Effective Date.

References

Bluebird bio, Inc. bluebird bio receives FDA accelerated approval for Skysona gene therapy for early, active cerebral adrenoleukodystrophy (CALD). Press Release. Somerville, MA: bluebird bio; September 16, 2022a

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