Medical Policy:

Skysona (elivaldogene autotemcel), suspension for IV infusion

POLICY NUMBER	LAST REVIEW	ORIGIN DATE
MG.MM.PH.375	January 5, 2024	February 9, 2023

The treating physician or primary care provider must submit to EmblemHealth, or ConnectiCare, as applicable (hereinafter jointly referred to as "EmblemHealth"), the clinical evidence that the member meets the criteria for the treatment or surgical procedure. Without this documentation and information, EmblemHealth will not be able to properly review the request preauthorization or post-payment review. The clinical review criteria expressed below reflects how EmblemHealth determines whether certain services or supplies are medically necessary. This clinical policy is not intended to pre-empt the judgment of the reviewing medical director or dictate to health care providers how to practice medicine. Health care providers are expected to exercise their medical judgment in rendering appropriate care. Health care providers are expected to exercise their medical judgment in rendering appropriate care.

EmblemHealth established the clinical review criteria based upon a review of currently available clinical information (including clinical outcome studies in the peer reviewed published medical literature, regulatory status of the technology, evidence-based guidelines of public health and health research agencies, evidence-based guidelines and positions of leading national health professional organizations, views of physicians practicing in relevant clinical areas, and other relevant factors). EmblemHealth expressly reserves the right to revise these conclusions as clinical information changes and welcomes further relevant information. Each benefit program defines which services are covered. The conclusion that a particular service or supply is medically necessary does not constitute a representation or warranty that this service or supply is covered and/or paid for by EmblemHealth, as some programs exclude coverage for services or supplies that EmblemHealth considers medically necessary.

If there is a discrepancy between this guideline and a member's benefits program, the benefits program will govern. Identification of selected brand names of devices, tests and procedures in a medical coverage policy is for reference only and is not an endorsement of any one device, test or procedure over another. In addition, coverage may be mandated by applicable legal requirements of a state, the Federal Government or the Centers for Medicare & Medicaid Services (CMS) for Medicare and Medicaid members. All coding and web site links are accurate at time of publication.

EmblemHealth may also use tools developed by third parties, such as the MCG™ Care Guidelines, to assist us in administering health benefits. The MCG™ Care Guidelines are intended to be used in connection with the independent professional medical judgment of a qualified health care provider and do not constitute the practice of medicine or medical advice. EmblemHealth Services Company, LLC, has adopted this policy in providing management, administrative and other services to EmblemHealth Plan, Inc., EmblemHealth Insurance Company, EmblemHealth Services Company, LLC, and Health Insurance Plan of Greater New York (HIP) related to health benefit plans offered by these entities. ConnectiCare, an EmblemHealth company, has also adopted this policy. All of the aforementioned entities are affiliated companies under common control of EmblemHealth Inc.

Definitions

Skysona is indicated to slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active cerebral adrenoleukodystrophy (CALD). Early, active cerebral adrenoleukodystrophy refers to asymptomatic or mildly symptomatic (neurologic function score, NFS ≤ 1) boys who have gadolinium enhancement on brain magnetic resonance imaging (MRI) and Loes scores of 0.5-9

Length of Authorization

Once per Lifetime

Dosing Limits [Medical Benefit]

The single dose is given intravenously which contains a minimum of 5.0 x 106 CD34+ cells/kg of body weight in which body weight is based on patient weight prior to first apheresis.

Guideline

1. **Cerebral Adrenoleukodystrophy.** Approve a one-time (lifetime) dose if the patient meets the following criteria (A, B, C, D, E, F, G, H, I, J, K, L, M, N, O, P, Q, R, S, and T).
   A. Patient is a male; **AND**
   B. Patient is ≥ 4 and < 18 years of age; **AND**
   C. Patient has early, active cerebral adrenoleukodystrophy as demonstrated by meeting the following (i, ii, and iii):
      i. Patient has a neurologic function score ≤ 1 **[documentation required]**; **AND**
      ii. Patient has gadolinium enhancement on brain magnetic resonance imaging (MRI) **[documentation required]**; **AND**
      iii. Patient has a Loes score between 0.5 and 9 **[documentation required]**; **AND**
   D. Patient has a confirmed mutation in the adenosine triphosphate binding cassette, sub family D member 1 (*ABCD1*) gene **[documentation required]**; **AND**
   E. Patient has elevated very long chain fatty acid levels according to the standard reference values of the laboratory **[documentation required]**; **AND**
   F. Patient does not have a Human Leukocyte Antigen (HLA)-matched family donor **[documentation required]**; **AND**
   G. According to the prescribing physician, the patient is able to undergo monitoring by magnetic resonance imaging; **AND**
   H. Patient does not currently have an active bacterial, viral, fungal, or parasitic infection as determined by the prescribing physician; **AND**
   I. Patient does not have any of the following (i and ii):
      i. Prior or current hematologic malignancy or myeloproliferative disorder; **AND**
      ii. Familial cancer syndrome or a history of such in his immediate family; **AND**
   J. According to the prescribing physician, hematopoietic stem cell transplantation is appropriate for the patient; **AND**
   K. Patient has adequate hepatic function defined by meeting the following (i, ii, and iii):
      i. Aspartate aminotransferase values are normal or ≤ 2.5 times the upper limit of normal **[documentation required]**; **AND**
      ii. Alanine aminotransferase values are normal or ≤ 2.5 times the upper limit of normal **[documentation required]**; **AND**
      iii. Total bilirubin values are normal or ≤ 3.0 mg/dL **[documentation required]**; **AND**
   L. Patient has adequate renal function as defined by meeting the following (i or ii);
      i. Estimated creatinine clearance is ≥ 50 mL/min; **OR**
      ii. Estimated glomerular filtration rate is ≥ 70 mL/minute/1.73 m^2; **AND**
   M. According to the prescribing physician, patient does not have evidence of cardiac compromise; **AND**
   N. Prior to collection of cells for manufacturing, patient screening is negative for the following (i, ii, iii, and iv):
      i. Hepatitis B virus **[documentation required]**; **AND**
      ii. Hepatitis C virus **[documentation required]**; **AND**
      iii. Human T-lymphotropic virus 1 and 2 **[documentation required]**; **AND**
      iv. Human immunodeficiency virus 1 and 2 **[documentation required]**; **AND**
   O. Prior to therapy, patient does not have evidence of hematological compromise as defined by meeting the following (i, ii, iii, and iv):
      i. Peripheral blood absolute neutrophil count ≥ 1,500 cells/mm^3 **[documentation required]**; **AND**
      ii. Platelet count ≥ 100,000 cells/mm^3 **[documentation required]**; **AND**
      iii. Hemoglobin ≥ 10 g/dL **[documentation required]**; **AND**
      iv. Patient does not have an uncorrected bleeding disorder; **AND**
   P. Patient meets the following (i, ii, iii, and iv):
      i. Patient will undergo mobilization, apheresis, myeloablative conditioning, and lymphodepletion; **AND**

ii. A granulocyte-colony stimulating factor product will be used for mobilization; **AND**
iii. Busulfan will be used for myeloablative conditioning; **AND**
iv. Cyclophosphamide or fludarabine will be used for lymphodepletion; **AND**

Q. Patient has received or is planning to receive prophylaxis for hepatic veno-occlusive disease/hepatic sinusoidal obstruction syndrome before conditioning; **AND**
Note: Examples of medications used include ursodeoxycholic acid or Defitelio (defibrotide intravenous infusion).

R. The prescribing physician confirms that the patient or his partner of childbearing potential will be using an effective method of contraception from the start of mobilization through at least 6 months after administration of Skysona; **AND**

S. Patient has not received Skysona in the past [verification in claims history required]; **AND**
Note: Verify through claims that the patient has not previously received Skysona AND, if no claim for Skysona is present, the prescribing physician confirms that the patient has not previously received Skysona

T. Medication is prescribed by a hematologist, a neurologist, and/or a stem cell transplant specialist

Limitations/Exclusions

1. Patient has a Full *ABCD1* Gene Deletion
2. Prior Hemoatopoietic Stem Cell Transplantation
3. Prior Receipt of Gene Therapy

Applicable Procedure Codes

Code	Description
J3590	Unclassified biologics
C9399	Unclassified drugs or biologicals

Applicable NDCs

Code	Description
73554-2111-01	Skysona (elivaldogene autotemcel) intravenous

ICD-10 Diagnoses

Code	Description
E71.520	Childhood cerebral X-linked adrenoleukodystrophy
E71.521	Adolescent X-linked adrenoleukodystrophy

Revision History

Company(ies)	DATE	REVISION
EmblemHealth & ConnectiCare	1/5/2024	Annual Review: Initial Criteria: Cerebral Adrenoleukodystrophy: The phrase “plans to” was changed to “will” to be more directive in the requirement that the patient undergoes mobilization, apheresis, myeloablative conditioning, and lymphodepletion. Added: “Patient has not received Skysona in the past [verification in claims history required]; AND” and all Limitation and Exclusion Criteria

EmblemHealth & ConnectiCare	02/09/2023	New Policy

References

1. Product Information: SKYSONA® intravenous suspension, elivaldogene autotemcel intravenous suspension. bluebird bio Inc (per FDA), Somerville, MA, 2022.