Geisinger

POLICIES AND PROCEDURE MANUAL

Policy: MBP 282.0

Section: Medical Benefit Pharmaceutical Policy

Subject: Skysona (elivaldogene autotemcel)

I. Policy:
Skysona (elivaldogene autotemcel)

II. Purpose/Objective:
To provide a policy of coverage regarding Skysona (elivaldogene autotemcel)

III. Responsibility:
A. Medical Directors
B. Medical Management
C. Pharmacy Department

IV. Required Definitions
1. Attachment – a supporting document that is developed and maintained by the policy writer or department requiring/authoring the policy.
2. Exhibit – a supporting document developed and maintained in a department other than
3. the department requiring/authoring the policy.
4. Devised – the date the policy was implemented.
5. Revised – the date of every revision to the policy, including typographical and grammatical changes.
6. Reviewed – the date documenting the annual review if the policy has no revisions necessary.

V. Additional Definitions
Medical Necessity or Medically Necessary means Covered Services rendered by a Health Care Provider that the Plan determines are:

a. appropriate for the symptoms and diagnosis or treatment of the Member's condition, illness, disease or injury;
b. provided for the diagnosis and the direct care and treatment of the Member's condition, illness disease or injury;
c. in accordance with current standards good medical treatment practiced by the general medical community;
d. not primarily for the convenience of the Member, or the Member's Health Care Provider; and
e. the most appropriate source or level of service that can safely be provided to the Member. When applied to hospitalization, this further means that the Member requires acute care as an inpatient due to the nature of the services rendered or the Member's condition, and the Member cannot receive safe or adequate care as an outpatient

Medicaid Business Segment
Medically Necessary — A service, item, procedure, or level of care compensable under the Medical Assistance program that is necessary for the proper treatment or management of an illness, injury, or disability is one that:

i. Will, or is reasonably expected to, prevent the onset of an illness, condition, injury or disability.
ii. Will, or is reasonably expected to, reduce or ameliorate the physical, mental or developmental effects of an illness, condition, injury or disability.
iii. Will assist the Member to achieve or maintain maximum functional capacity in performing daily activities, taking into account both the functional capacity of the Member and those functional capacities that are appropriate for Members of the same age.

DESCRIPTION:
Skysona (elivaldogene autotemcel) is a gene therapy consisting of autologous CD34+ hematopoietic cells transduced with the Lenti-D lentiviral vector to add functional copies of the ABCD1 cDNA into the hematopoietic stem cells. After elivaldogene autotemcel infusion, transduced CD34+ hematopoietic stem cells engraft in the bone marrow and differentiate to produce various cell types, including monocytes (CD14+) capable of producing functional ALDP. Functional ALDP participates in the local degradation of very long chain fatty acids, which slows or possibly prevents further demyelination and inflammation.

CRITERIA FOR USE: Requires Prior Authorization by Medical Director or Designee

Skysona (elivaldogene autotemcel) will be considered medically necessary for all lines of business when ALL of the following criteria are met:

- Prescription written by a hematologist, neurologist and/or stem cell transplant specialist **AND**
- Medical record documentation that the patient is a male based on assigned sex at birth, age greater than or equal to 4 years and less than or equal to 17 years **AND**
- Medical record documentation of a diagnosis of adrenoleukodystrophy (ALD) confirmed by BOTH of the following:
    - Medical record documentation of the presence of a mutation (variant) in the adenosine triphosphate binding cassette, sub family D member 1 (ABCD1) gene confirmed by genetic testing, **AND**
    - Medical record documentation of elevated plasma concentrations of very long chain fatty acids (VLCFA) levels

  AND
- Medical record documentation that the patient has early, active cerebral disease (Cerebral adrenoleukodystrophy (CALD)) as evidenced by ALL of the following:
    - Central radiographic review of Brain MRI demonstrating BOTH of the following:
        - Loes score between 0.5 and 9 (inclusive) on the 34-point scale **AND**
        - Gadolinium enhancement on MRI of demyelinating lesions

      AND
    - Neurologic function score (NFS) of less than or equal to 1

  AND
- Medical record documentation that the member has not had a prior hematopoietic stem cell transplant **AND**
- Medical record documentation the member is a candidate for an allogenic hematopoietic stem cell transplant but ineligible due to absence of Human Leukocyte Antigen (HLA)-matched family donor* **AND**
- Medical record documentation that the member has not received Skysona, or any other gene therapy previously **AND**
- Medical record documentation that the member has a negative serology test for Human Immunodeficiency Virus (HIV) **AND**
- Medical record documentation that the member will have treatment administered at a Skysona Qualified Treatment Center

*Note to reviewer: The package insert recommends confirming that hematopoietic stem cell transplantation (HSCT) is appropriate prior to Skysona since patients will be going through similar steps (mobilization, apheresis, and myeloablation) required for a HSCT. However, the ALD-102 clinical trial excluded patients who had a known and available HLA-matched family donor. Considering that HSCT has been available for longer and has more evidence supporting its use, it may be appropriate to require HSCT as an alternate to Skysona. While it is possible for patients to have a matched unrelated donor, outcomes are best with matched related donors.

AUTHORIZATION DURATION: One (1) time approval per lifetime; Requests for authorizations exceeding these limits will require the following medical record documentation of peer-reviewed literature citing well-designed clinical trials to indicate that the member's healthcare outcome will be improved by dosing beyond the FDA-approved treatment duration.

Note: For Medicaid (GHP Family), any requests for services that do not meet criteria set in the PARP will be evaluated on a case-by-case basis.

LINE OF BUSINESS:
Eligibility and contract specific benefit limitations and/or exclusions will apply. Coverage statements found in the line of business specific benefit document will supersede this policy.

This policy will be revised as necessary and reviewed no less than annually**.**

Devised: 5/16/23

Revised:

Reviewed: