Elivaldogene autotemcel (Skysona) – PA Criteria

HCPC: J3387

Elivaldogene autotemcel (Skysona) is an autologous cellular gene therapy indicated for the treatment of cerebral adrenoleukodystrophy in male patients 4-17 years of age. It is covered by South Dakota Medicaid following prior authorization when the patient meets the following criteria:

** All requests under this policy require SD medical director review **in addition** to meeting specified criteria below **

- **Initial Therapy (must meet all):**
  - Therapy is prescribed by a neurologist or transplant specialist
  - Individual is a biologic male and between the ages of 4-17 years
  - Individual has a diagnosis of cerebral adrenoleukodystrophy confirmed by **both** of the following:
    - Genetic confirmation of the *ABCD1* mutation
    - Elevated levels of very long chain fatty acids (VLCFA)
  - Individual has early, active disease as evidenced by a Loes score of ≥0.5- ≤0.9 and gadolinium enhancement of demyelinating lesions on MRI
  - Documentation is submitted indicating neurologic function score (NFS) ≤1
  - Individual is not currently taking statins or other dietary regimens to lower VLCFA levels
  - Attestation from provider that member is clinically stable and able to undergo gene therapy
  - Screening has been done within the last 6 months and documentation is provided indicating that the individual is negative for the following active diseases: HIV, Hepatitis B, and Hepatitis C
  - Individual does not have a known, human leukocyte antigen (HLA)-matched donor
  - Individual does not have renal compromise as evidenced by abnormal renal function (actual or calculated creatinine clearance < 50 ml/min
  - Individual does not have cardiac compromise as evidenced by left ventricular ejection fraction <40 percent (%)
  - Individual has not previously received a hematopoietic stem cell transplant or prior gene therapy
  - Individual does not have advanced liver disease (defined as AST >2.5x upper limit of normal (ULN), ALT > 2.5x ULN, or total bilirubin value > 3.0 milligram per deciliter (mg/dL), except if there is a diagnosis of Gilbert's Syndrome and the participant is otherwise stable)
  - If female, documentation of negative pregnancy test must be submitted prior to therapy
  - Male and female members of reproductive potential must use an effective method of contraception at start of treatment and at least 6 months following Skysona administration
  - Approval duration: 1 dose
- **Continuation of Therapy: not authorized**