Onasemnogene Abeparvovec (Zolgensma) – PA Criteria

HCPC: J3399

Zolgensma is a recombinant adeno-associated virus vector-based gene therapy that is indicated for the treatment of spinal muscular atrophy (SMA) in infants and children <2 years of age. It is covered by South Dakota Medicaid following prior authorization when the following criteria are met:

** 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 board-certified pediatric neurologist with expertise in the treatment of SMA
  - Documentation is provided (e.g., chart notes, laboratory values) confirming the mutation or deletion of genes in chromosome 5q resulting in **one** of the following:
    - Homozygous gene deletion or mutation of SMN1 gene (e.g., homozygous deletion of exon 7 at locus 5q13)
    - Compound heterozygous mutation of SMN1 gene (e.g., deletion of SMN1 exon 7 [allele 1] and mutation of SMN1 [allele 2])
  - Individual meets **one** of the following in regard to diagnosis of SMA:
    - Diagnosis of SMA by a board-certified pediatric neurologist with expertise in the diagnosis of SMA
    - Diagnosis of SMA based on the results of SMA newborn screening and submission of medical records (e.g., chart notes, laboratory values) confirming that patient has 3 copies or less of SMN2 gene
  - Individual is <2 years of age
  - For use in a neonatal patient born prematurely, the full-term gestational age has been reached
  - Individual is not dependent on **either** of the following:
    - Invasive ventilation or tracheostomy
    - Use of non-invasive ventilation beyond use for naps and nighttime sleep
  - Individual is not to receive routine concomitant SMN modifying therapy [e.g., Spinraza (nusinersen)]. Patient's medical record will be reviewed and any current authorizations for SMN modifying therapy will be terminated upon Zolgensma approval; patient access to subsequent SMN modifying therapy will be assessed according to respective coverage policy of concomitant agent
  - Individual does not have an elevated anti-adeno-associated virus serotype 9 (anti-AAV9) antibody titer above 1:50
  - Individual has LFTs less than 2X ULN determined by a certified lab
  - Provider attests that the patient, while under the care of the physician, will be assessed on the Hammersmith Functional Motor Scale Expanded (HFMSE) assessment (or the initial test used to establish baseline motor ability unless that test is no longer appropriate for the patient) during subsequent office visits while the patient is 2 to 3 years of age or older following exam scales during subsequent office visits
  - Individual has never received Zolgensma treatment in their lifetime
  - Approval duration: one dose or until individual reaches 2 years of age, whichever is first
- **Continuation of Therapy:** not authorized

Zolgensma is not covered for:

- The treatment of pre-symptomatic patients diagnosed by newborn screening who have more than 3 copies of the SMN2 gene.
- The treatment of symptomatic later-onset SMA older than 2 years of age
- SMA without chromosome 5q mutations or deletions.
- The routine combination treatment of SMA with concomitant SMN modifying therapy