ZOLGENSMA® (onasemnogene abeparvovec-xioi)

LENGTH OF AUTHORIZATION: ONE TIME SINGLE DOSE

REVIEW CRITERIA (all labs and supporting documentation must be submitted):

- Patient must be < 2 years of age.
- Patient has diagnosis of Spinal Muscular Atrophy (SMA) based on gene mutation analysis including ALL of the following:
  - Bi-allelic SMN1 mutations (deletion or point mutations) in the survival motor neuron 1 gene and
  - Anti-AAV9 antibody titers of ≤ 1:50, measured by ELISA binding immunoassay.
- Prescribed by or in consultation with a pediatric neuromuscular specialist or a neurologist specializing in SMA.
- Patient must reach full gestational age (administration to premature neonates is not recommended).
- Documentation of baseline laboratory tests of the following to ensure no renal impairment, hepatic impairment or hematologic impairment is present:
  - Platelet count within normal limits
  - Troponin-1 within normal limits
  - Alanine aminotransferase/Aspartate aminotransferase (< 2x upper limit of normal)
  - Total bilirubin within normal limits
  - Prothrombin time within normal limits
- Continuation of monitoring liver function tests, platelet count, and troponin-1 for at least 3 months post infusion.
- Documentation of one of the following for baseline motor ability:
  - Children’s Hospital of Philadelphia Infant Test of Neuromuscular Disorder score (CHOP INTEND)
  - Hammersmith Infant Neurological Exam (HINE) (infant to early childhood)
  - Hammersmith Functional Motor Scale Expanded (HFMSE)
  - Upper Limb Module Test (ULM) (non-ambulatory)
- Patients must not have advanced SMA (e.g. complete paralysis of limbs, *permanent ventilator dependent).
- Verify the patient does not have a contraindication or intolerance to corticosteroid therapy.

- Concomitant therapy will be implemented with systemic corticosteroids equivalent to oral prednisolone 1mg/kg/day for 30 days, starting one day prior to administration of Zolgensma.

**DOSSING AND ADMINISTRATION:**

- Recommended dose is $1.1 \times 10^{14}$ vector genomes per kilogram (vg/kg) of body weight intravenously over 60 minutes.

*Permanent ventilation was defined as requiring invasive ventilation (tracheostomy), or respiratory assistance for 16 or more hours per day (including noninvasive ventilatory support) continuously for 14 or more days in the absence of an acute reversible illness, excluding perioperative ventilation.*