

Onasemnogene abeparvovec (Zolgensma)

Notes:

- * Tracheostomy: surgically created hole in front of neck for placement of a tube into windpipe to help with breathing
- ^ Noninvasive ventilation refers to positive pressure ventilation delivered through a noninvasive interface (nasal mask, facemask, or nasal plugs)

Non-Formulary **onasemnogene abeparvovec (Zolgensma)** requires a clinical review. Appropriateness of therapy will be based on the following criteria:

Initiation (new start) criteria: Non-Formulary **onasemnogene abeparvovec (Zolgensma)** will be covered on the prescription drug benefit when the following criteria are met:

- Prescribed by a neurologist with specialty in neuromuscular disorders
- Patient is 6 months old or younger
- Patient has a confirmed diagnosis of 5q-autosomal recessive spinal muscular atrophy (SMA) with biallelic deletions or mutations in the SMN1 gene
- Patient has 1, 2 to 3 copies of the SMN2 gene
- Anti-AAV9 antibody titer $\leq 1:50$ within 14 days
- Patient has not had prior gene therapy for SMA
- Patient does not require permanent invasive ventilation or tracheostomy*
- Patient does not use noninvasive ventilation^ support OR uses noninvasive ventilation support for less than 10 hours per day
- Patient has been reviewed by the Kaiser Permanente Interregional Consultative Physician Panel, with recommendation to use medication