# Medical Policy

## Zolgensma (Onasemnogene Abeparvovec)

<table>
<thead>
<tr>
<th>Authorization required</th>
<th>Commercial and Qualified Health Plans</th>
<th>MassHealth</th>
</tr>
</thead>
<tbody>
<tr>
<td>Authorization not required</td>
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### Overview

Zolgensma is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene.

### Criteria (Commercial)

1. **Criteria for Approval** (The member must meet **all** of the following requirements):
   - Member has confirmed and symptomatic genetic diagnosis documented by bi-allelic mutations in the SMN1 gene AND three or less copies of SMN2 gene
   - Member has an anti-adeno-associated viral vector, serotype 9 (AAV9) antibody titer less than or equal to 1:50
   - Member is less than 2 years of age
   - Member has not previously received Zolgensma
   - Member does not have concomitant illness such as severe kidney or liver disease, active viral infection, or symptomatic cardiomyopathy
   - If the member is receiving treatment with Spinraza, that treatment will be discontinued

2. **Dosing and Administration**
   - Member will receive a single-dose Zolgensma intravenously infusion within accordance of the FDA approved labeling; $1.1 \times 10^{14}$ vector genomes (vg) per kilogram of body weight.

3. **Duration of Therapy**
   - Single-dose one-time intravenous infusion per lifetime

4. **Exclusions**
   - The member has advanced SMA as evidenced but not limited to complete paralysis of limbs, invasive ventilatory support (tracheostomy), or use of non-invasive respiratory support for more than 16 hours per day.

### Criteria (MassHealth)

For its MassHealth members AllWays Health Partners follows the Zolgensma clinical guidelines as set forth in MassHealth’s Drug Utilization Review Program.

1. **Criteria for Approval** (The member must meet **all** of the following requirements as outlined in documentation provided by the treating prescriber):
   - Appropriate diagnosis (Type 1, 2 or 3 SMA)
• A genetic test confirming the member has a diagnosis of bi-allelic mutation in the SMA1 gene (e.g. SMN1 homozygous gene deletion or mutation or compound heterozygous mutation)
• A genetic test confirming the member has two or three copies of the SMN2 gene
• Member has an anti-adeno-associated viral vector, serotype 9 (AAV9) antibody titer less than or equal to 1:50
• Member is less than 2 years of age

2. Dosing and Administration
• The prescriber is a neuromuscular specialist
• Member will receive a single-dose Zolgensma intravenously infusion within accordance of the FDA approved labeling; 1.1 x 10^{14} vector genomes (vg) per kilogram of body weight.

3. Duration of Therapy
• Single-dose one-time intravenous infusion per lifetime

4. Exclusions
• The member has advanced SMA as evidenced but not limited to complete paralysis of limbs, invasive ventilatory support (endotracheal tube or tracheotomy tube) or use of non-invasive respiratory assistance for at least 14 days for at least 16 hours per day.

CPT/HCPC Codes

<table>
<thead>
<tr>
<th>Authorized Code</th>
<th>Code Description</th>
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<td>J3399</td>
<td>Injection, Onasemnogene abeparvovec-xioi, per treatment, up to 5×10^{15} vector genomes</td>
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Effective
July 2020: Added code.
April 1, 2020: Updated table and added Criteria section to reflect MassHealth coverage.
December 2019: Effective date.

References


