Mass General Brigham
Health Plan

Medical Policy
Zolgensma (Onasemnogene Abeparvovec)

Policy Number: 060

	Commercial and Qualified Health Plans	MassHealth	Medicare Advantage
Authorization required	X	X	X
Authorization not required			

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; 1.1×10^{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.

MassHealth Variation
Mass General Brigham Health Plan uses the MassHealth Drug List for coverage determinations for members of the MGB ACO. Criteria for Zolgensma are found in Table 76: Neuromuscular Agents – Duchenne Muscular Dystrophy and Spinal Muscular Atrophy.

Medicare Variation
Mass General Brigham Health Plan uses guidance from the Centers for Medicare and Medicaid Services (CMS) for coverage determinations for its Medicare Advantage plan members. National Coverage Determinations (NCDs), Local Coverage Determinations (LCDs), Local Coverage Articles (LCAs) and documentation included in

the Medicare manuals are the basis for coverage determinations. When there is no guidance from CMS for the requested service, Mass General Brigham Health Plan's medical policies are used for coverage determinations. **At the time of Mass General Brigham Health Plan's most recent policy review, CMS had no NCD or LCD for onasemnogene abeparvovec.**

Codes

The following codes are included below for informational purposes only. Inclusion of a code does not constitute or imply coverage or reimbursement.

This list of codes applies to commercial and MassHealth plans only.

Authorized Code	Code Description
J3399	Injection, Onasemnogene abeparvovec-xioi, per treatment, up to 5×10^{15} vector genomes

Effective
October 2024: Annual update. Clarified Medicare variation language.
September 2024: Ad hoc update. MassHealth variation added.
October 2023: Annual update. Medicare Advantage added to table.
October 2022: Annual update. References updated.
October 2021: Annual update. References updated.
October 2020: Annual update. References updated.
July 2020: Added code.
April 1, 2020: Updated table and added Criteria section to reflect MassHealth coverage.
December 2019: Effective date.

References
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