Zolgensma® (onasemnogene abeparvovec-xioi)
(Intravenous)

Effective Date: 8/14/2019
Review date: 10/17/2019, 10/5/2020, 7/22/2021, 4/7/2022, 2/23/2023, 12/07/2023, 01/04/2024, 03/12/2025, 3/10/2026

Scope: Medicaid, Commercial, Medicare

I. Length of Authorization

Coverage will be provided for one dose and may not be renewed.

II. Dosing Limits

A. Max Units (per dose and over time) [HCPCS Unit]:

- 1 kit (based on weight chart below)

III. Summary of Evidence

Zolgensma (onasemnogene abeparvovec) is an adeno-associated virus (AAV) 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. Clinical trials, including the START trial, have demonstrated that Zolgensma significantly improves motor function and survival in infants with SMA. Notably, many treated infants achieved motor milestones such as sitting unassisted, standing, and walking, milestones that are typically not achieved in the natural course of the disease without treatment. Zolgensma-treated patients showed a significant reduction in the need for permanent ventilation or tracheostomy and a significant increase in event-free survival compared to untreated infants. Common adverse events include elevated liver enzymes and transient thrombocytopenia.

IV. Initial Approval Criteria [1-7]

- Submission of supporting clinical documentation (including but not limited to medical records, chart notes, lab results, and confirmatory diagnostics) related to the medical necessity criteria is REQUIRED on all requests for authorizations. Records will be reviewed at the time of submission as part of the evaluation of this request. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e., genetic, and mutational testing) supporting initiation when applicable. Please provide documentation via direct upload through the PA web portal or by fax**.** Failure to submit the medical records may result in the denial of the request due to inability to establish medical necessity in accordance with policy guidelines.

Coverage is provided in the following conditions:

Spinal Muscular Atrophy (SMA) † Φ

- Member must be less than 2 years of age; **AND**
- Member has a diagnosis* of 5q spinal muscular atrophy confirmed by either bi-allelic deletion or dysfunctional point mutation of the *SMN1* gene; **AND**
- Member must have SMA phenotype 1 or 2; **AND**
- Member has ≤ 3 copies of the SMN2 gene (Note: Members with >3 copies of the SMN2 gene will be reviewed on a case-by-case basis); **AND**
- Member must have a baseline anti-AAV9 antibody titer of ≤ 1:50 measured using an enzyme-linked immunosorbent assay (ELISA); **AND**
- Member is clinically stable in their overall baseline health status (e.g., hydration and nutritional status, respiratory status, etc.) prior to administration; **AND**
- Member does not have an active infection, including clinically important localized infections; **AND**
- Prescriber submits baseline documentation of baseline AST, ALT, total bilirubin, and prothrombin time lab values prior to and subsequent to therapy for at least 3 months; **AND**
- Baseline platelet counts will be assessed prior to initiating therapy and will continue to be monitored on a regular basis (i.e., at least weekly for the first month and as clinically indicated until platelet counts return to baseline); **AND**
- Documentation that member is up to date with all vaccinations (including seasonal prophylaxis against respiratory syncytial virus (RSV), in accordance with current vaccination guidelines, prior to initiating therapy; **AND**
- Used concomitantly with systemic corticosteroids (see dosage/administration below); **AND**
- Member will be considered for cardiac evaluation based on clinical presentation; **AND**
- Member does not have advanced disease (e.g., complete limb paralysis, permanent ventilation support, etc.); **AND**
- Member will not use in combination with other agents for SMA (e.g., nusinersen, risdiplam, etc.). Member's medical record will be reviewed and any current authorizations for other agents for SMA will be terminated upon Zolgensma approval; **AND**
- Member must have diagnosis of Type 1 SMA by a board-certified pediatric neurologist; **AND**
- Member has clinical signs and symptoms from birth and up to 6 months of age, unless the diagnosis is confirmed by genetic testing and/or newborn screening and the member is asymptomatic from birth and up to 6 months of age; **AND**
- For use in a neonatal member born prematurely, the full-term gestational age has been reached; **AND**
- Member must weigh between 2.6 kg and 21.0 kg; **AND**

- Submission of medical records (chart notes, laboratory testing) confirming the member's most recent **CHOP INTEND** score is greater than or equal to 40; **AND**
- Member will receive Zolgensma (onasemnogene abeparvovec-xioi) intravenously within accordance of the United States Food and Drug Administration approved labeling; **AND**
- Member has not been treated in the past with onasemnogene abeparvovec (e.g. Zolgensma, Itvisma, etc); **AND**
- Zolgensma (onasemnogene abeparvovec-xioi) will be administered at an authorized treatment center; **AND**
- Medicare members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements

SMA phenotype 1 (aka Werdnig-Hoffman disease) has a natural history characterized by onset of symptoms (i.e. severe weakness) prior to 6 months of age, inability to sit without support, and an average life span of less than 2 years (in patients without prior therapy to increase SMN protein). Deficiency of SMN protein, due to homozygous deletion/mutation in the *SMN1* gene, results in loss of motor neurons in the spinal cord and brain stem manifesting clinically as atrophy and weakness. Copy number of the *SMN2* gene, which produces approximated 5-10% functional SMN protein, are positively correlated with milder phenotype.

- Approximately 80% of patients with SMA1 have 1 or 2 copies of the *SMN2* gene; approximately 20% have 3 copies (estimated percentages vary)
- The c.859G>C single base substitution modification in exon 7 of the SMN2 gene is predictive of a milder phenotype

Onasemnogene abeparvovec-xioi is a recombinant self-complementary AAV9 containing a transgene encoding the human survival motor neuron (SMN) protein.

† FDA Approved Indication(s); ‡ Compendium Recommended Indication(s); Φ Orphan Drug

V. Renewal Criteria

Coverage cannot be renewed. Approval is for one kit once per lifetime

VI. Dosage/Administration

Indication	Dose
SMA1	**For single-dose intravenous infusion only.** Preparing for Administration: • One day prior to Zolgensma infusion, begin administration of systemic corticosteroids equivalent to oral prednisolone at 1 mg/kg of body weight per day for a total of 30 days Zolgensma Infusion: • Administer as a single-dose intravenous infusion through a venous catheter

	• Administer as a slow infusion over 60 minutes • The recommended dose of Zolgensma is 1.1×10^{14} vector genomes per kilogram (vg/kg) of body weight

- Zolgensma is shipped frozen at ≤ -60 °C. Thaw prior to infusion. Store refrigerated. Must use within 14 days of receipt.
- Zolgensma is an adeno-associated virus vector-based gene therapy. Follow precautions for viral vector shedding for one month after the infusion

VII. Billing Code/Availability Information

HCPCS code:

- J3399 – Injection, onasemnogene abeparvovec-xioi, per treatment, up to $5x10^{15}$ vector genomes: 1 billable unit = 1 treatment, up to $5x10^{15}$ vector genomes

NDC:

Zolgensma kit sizes:

Patient Weight (kg)	NDC	Patient Weight (kg)	NDC
2.6 – 3.0	71894-0120-xx	12.1 – 12.5	71894-0139-xx
3.1 – 3.5	71894-0121-xx	12.6 – 13.0	71894-0140-xx
3.6 – 4.0	71894-0122-xx	13.1 – 13.5	71894-0141-xx
4.1 – 4.5	71894-0123-xx	13.6 – 14.0	71894-0142-xx
4.6 – 5.0	71894-0124-xx	14.1 – 14.5	71894-0143-xx
5.1 – 5.5	71894-0125-xx	14.6 – 15.0	71894-0144-xx
5.6 – 6.0	71894-0126-xx	15.1 – 15.5	71894-0145-xx
6.1 – 6.5	71894-0127-xx	15.6 – 16.0	71894-0146-xx
6.6 – 7.0	71894-0128-xx	16.1 – 16.5	71894-0147-xx
7.1 – 7.5	71894-0129-xx	16.6 – 17.0	71894-0148-xx
7.6 – 8.0	71894-0130-xx	17.1 – 17.5	71894-0149-xx
8.1 – 8.5	71894-0131-xx	17.6 – 18.0	71894-0150-xx
8.6 – 9.0	71894-0132-xx	18.1 – 18.5	71894-0151-xx
9.1 – 9.5	71894-0133-xx	18.6 – 19.0	71894-0152-xx
9.6 – 10.0	71894-0134-xx	19.1 – 19.5	71894-0153-xx
10.1 – 10.5	71894-0135-xx	19.6 – 20.0	71894-0154-xx
10.6 – 11.0	71894-0136-xx	20.1 – 20.5	71894-0155-xx
11.1 – 11.5	71894-0137-xx	20.6 – 21.0	71894-0156-xx
11.6 – 12.0	71894-0138-xx		

VIII. References

1. Zolgensma [package insert]. Bannockburn, IL; AveXis, Inc., February 2025. Accessed January 2026.
2. Mendell JR, Al-Zaidy S, Shell R. Single-dose gene-replacement therapy for spinal muscular atrophy. N Engl J Med. 2017;377(18):1713-1722. doi: 10.1056/NEJMoa1706198.
3. Wang CH, Finkel RS, Bertini ES, et al. Consensus statement for standard of care in spinal muscular atrophy. J Child Neurol. 2007 Aug;22(8):1027-49.
4. Prior TW, Finanger E. Spinal muscular atrophy. GeneReviews. www.ncbi.nlm.nih.gov/books/NBK1352/ (Accessed on June 10, 2019)
5. Dabbous O, Maru B, Jansen JP, et al. Survival, Motor Function, and Motor Milestones: Comparison of AVXS-101 Relative to Nusinersen for the Treatment of Infants with Spinal Muscular Atrophy Type 1. Adv Ther. 2019 May;36(5):1164-1176.
6. Al-Zaidy S, Pickard AS, Kotha K, et al. Health outcomes in spinal muscular atrophy type 1 following AVXS-101 gene replacement therapy. Pediatr Pulmonol. 2019 Feb;54(2):179-185.
7. Al-Zaidy SA, Kolb SJ, Lowes L, et al. AVXS-101 (Onasemnogene Abeparvovec) for SMA1: Comparative Study with a Prospective Natural History Cohort. J Neuromuscul Dis. 2019;6(3):307-317. doi: 10.3233/JND-190403.

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
G12.0	Infantile spinal muscular atrophy, type I [Werdnig-Hoffmann]

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determination (NCD), Local Coverage Articles (LCAs) and Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. They can be found at: http://www.cms.gov/medicare-coverage-database/search/advanced-search.aspx. Additional indications may be covered at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCA/LCD): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA, LLC
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA, LLC
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC

Policy Rationale:

Zolgensma was reviewed by the Neighborhood Health Plan of Rhode Island Pharmacy & Therapeutics (P&T) Committee. Neighborhood adopted the following clinical coverage criteria to ensure that its members use Zolgensma according to Food and Drug Administration (FDA) approved labeling and/or relevant clinical literature. Neighborhood worked with network prescribers and pharmacists to draft these criteria. These criteria will help ensure its members are using this drug for a medically accepted indication, while minimizing the risk for adverse effects and ensuring more cost-effective options are used first, if applicable and appropriate. For Medicare members, these coverage criteria will only apply in the absence of National Coverage Determination (NCD) or Local Coverage Determination (LCD) criteria. Neighborhood will give individual consideration to each request it reviews based on the information submitted by the prescriber and other information available to the plan.