

Zolgensma®

(onasemnogene abeparvovec-xioi)

Date of Origin: 07/24/19

Last Review Date: 05/26/2021

Effective Date: 06/01/2021

Dates Reviewed: 07/24/19, 05/26/21

Developed By: Medical Criteria Committee

I. Length of Authorization

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

II. Dosing Limits

A. Max Units [Medical Benefit]:

- 1 kit per lifetime (based on weight chart below)

III. Initial Approval Criteria

Spinal Muscular Atrophy (SMA)

- Submission of medical records (e.g. chart notes, laboratory values) confirming the following:
 - Onasemnogene abeparvovec-xioi (Zolgensma) is prescribed by or in consultation with a neurologist or neuromuscular specialist with expertise in the diagnosis and treatment of SMA; **AND**
 - Patient is less than 2 years of age; **AND**
 - Patient has a diagnosis of infantile-onset SMA with genetic confirmation of bi-allelic SMN1 mutations; **AND**
 - Patient does not have advanced SMA (e.g. complete paralysis of limbs, permanent ventilator dependence); **AND**
 - Patient must have a baseline anti-AAV9 antibody titer of $\leq 1:50$ measured by ELISA; **AND**
 - Patient does not have pre-existing hepatic insufficiency; **AND**
 - Onasemnogene abeparvovec-xioi (Zolgensma) will be administered intravenously per the FDA-approved label; **AND**
 - Onasemnogene abeparvovec-xioi (Zolgensma) will not be used in combination with nusinersen (Spinraza); **AND**
 - Patient has not previously received onasemnogene abeparvovec-xioi (Zolgensma) or another gene therapy for SMA; **AND**
 - Provider acknowledges that Moda may request documentation to support patient's progress [e.g. developmental milestones, objective measures (e.g. CHOP INTEND)]

IV. Renewal Criteria

Onasemnogene abeparvovec-xioi (Zolgensma) is a single lifetime dose and cannot be renewed.

V. Dosage/Administration

Indication	Dose
Infantile-onset SMA	<p><i>For single-dose intravenous infusion only.</i></p> <p><u>Preparing for Administration:</u> One day prior to Zolgensma infusion, begin administration of systemic corticosteroids equivalent to oral prednisone at 1 mg/kg of body weight per day for a total of 30 days</p> <p><u>Zolgensma Infusion:</u></p> <ul style="list-style-type: none">• 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 (see table below)

VI. Billing Code/Availability Information

Jcode:

- J3399 – Injection, onasemnogene abeparvovec-xioi, per treatment, up to 5×10^{14} vector genomes

NDC:

Zolgensma kit sizes:

Patient Weight (kg)	5.5 mL vial	8.3 mL vial	Total vials per kit	NDC
2.6 – 3.0	0	2	2	71894-120-02
3.1 – 3.5	2	1	3	71894-121-03
3.6 – 4.0	1	2	3	71894-122-03
4.1 – 4.5	0	3	3	71894-123-03
4.6 – 5.0	2	2	4	71894-124-04
5.1 – 5.5	1	3	4	71894-125-04
5.6 – 6.0	0	4	4	71894-126-04
6.1 – 6.5	2	3	5	71894-127-05
6.6 – 7.0	1	4	5	71894-128-05
7.1 – 7.5	0	5	5	71894-129-05
7.6 – 8.0	2	4	6	71894-130-06
8.1 – 8.5	1	5	6	71894-131-06
8.6 – 9.0	0	6	6	71894-132-06
9.1 – 9.5	2	5	7	71894-133-07
9.6 – 10.0	1	6	7	71894-134-07

10.1 – 10.5	0	7	7	71894-135-07
10.6 – 11.0	2	6	8	71894-136-08
11.1 – 11.5	1	7	8	71894-137-08
11.6 – 12.0	0	8	8	71894-138-08
12.1 – 12.5	2	7	9	71894-139-09
12.6 – 13.0	1	8	9	71894-140-09
13.1 – 13.5	0	9	9	71894-141-09

VII. References

1. Zolgensma [package insert]. Bannockburn, IL; AveXis, Inc., March 2021.
2. Mendell, JR, Al-Zaidy, S, Shell, R, et al. Single-Dose Gene-Replacement Therapy for Spinal Muscular Atrophy. *The New England journal of medicine*. 2017 Nov 2;377(18):1713-22. PMID: 29091557
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. Food and Drug Administration: BLA Clinical Review Memorandum for Zolgensma. Available from: <https://www.fda.gov/vaccines-blood-biologics/zolgensma>
5. UpToDate, Inc. Spinal Muscular Atrophy. UpToDate [Online Database]. Waltham, MA. Last updated February 11, 2019. Available from: <http://uptodate.com/home/index.html>.

Appendix 1 – Supplemental Information

Clinical classification of spinal muscular atrophy (SMA)

SMA Type	SMN2 Copies	Onset	Highest Achieved Motor Function	Life Expectancy
0	1	In utero	None (unable to sit or roll)	< 6 months
I (Werdnig-Hoffman disease)	2-3 (~80% have 2)	< 6 months	Unable to sit or roll unassisted	< 2 years
II (Dubowitz Syndrome)	3-4	6-18 months	Sit independently, unable to walk unassisted	> 2 years (70% alive at 25 years)
III (Kugelberg-Welander Disease)	3-4 (~80% have 4)	Early childhood to early adulthood (juvenile)	Stand and walk independently, but lose this ability as disease progresses	Adulthood (may have normal life expectancy)
IV	4-8	Adult (20-30 years), usually after 30	Ambulatory during adulthood, may experience mild muscle weakness	Adult (normal lifespan)

Appendix 2 – Covered Diagnosis Codes

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

Appendix 3 – 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) 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/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
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corporation (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 Corporation (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Cahaba Government Benefit Administrators, 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