Zolgensma® (onasemnogene abeparvovec-xioi) (Intravenous)

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I. Length of Authorization

- Initial: Prior authorization validity will be provided initially for one dose.
- Renewal: Prior authorization validity may NOT be renewed.

II. Dosing Limits

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

- 1 billable unit (1 treatment of up to $5x10^{15}$ vector genomes)

III. Initial Approval Criteria

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.

Prior authorization validity is provided in the following conditions:

Spinal Muscular Atrophy (SMA) † Φ [1-12]

- Patient must be less than 2 years of age; **AND**
- Patient has a diagnosis of 5q spinal muscular atrophy confirmed by either bi-allelic deletion or dysfunctional point mutation of the *SMN1* (survival motor neuron 1) gene; **AND**
- One of the following:
    - Diagnosis of symptomatic SMA by a neurologist with expertise in the diagnosis of SMA; **OR**
    - Both of the following:
        - Diagnosis of SMA based on the results of SMA newborn screening; **AND**
        - Submission of medical records (e.g., chart notes, laboratory values) confirming that patient has 4 copies or less of SMN2 gene; **AND**

- Patient must have a baseline anti-AAV9 antibody titer of ≤ 1:50 measured using an enzyme-linked immunosorbent assay (ELISA); **AND**
- Baseline liver function will be assessed prior to initiating therapy and will continue to be monitored for at least 3 months after therapy, and at other times as clinically indicated; **AND**
- Used concomitantly with systemic corticosteroids (see dosage/administration below); **AND**
- Patient does not have advanced disease (e.g., complete limb paralysis, permanent ventilation support, etc.); **AND**
- Patient must not have previously received treatment with SMA gene therapy (e.g., onasemnogene abeparvovec-xioi, etc.); **AND**
- Will not be used in combination with other agents for SMA (e.g., nusinersen, risdiplam, etc.)

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

IV. Renewal Criteria [1]

- Duration of authorization has not been exceeded (refer to Section I)

V. Dosage/Administration [1]

Indication	Dose
Spinal Muscular Atrophy	**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
NOTE: • Zolgensma is shipped and delivered frozen at ≤ -60 °C (-76°F). Upon receipt, immediately place in a refrigerator at 2°C to 8°C (36°F to 46°F). Thaw prior to infusion. DO NOT RE-FREEZE. Must be used 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.	

VI. 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(s):

Zolgensma kits:

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		

VII. References

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Appendix A – Non-Quantitative Treatment Limitations (NQTL) Factor Checklist

Non-quantitative treatment limitations (NQTLs) refer to the methods, guidelines, standards of evidence, or other conditions that can restrict how long or to what extent benefits are provided under a health plan. These may include things like utilization review or prior authorization. The utilization management NQTL applies comparably, and not more stringently, to mental health/substance use disorder (MH/SUD) Medical Benefit Prescription Drugs and medical/surgical (M/S) Medical Benefit Prescription Drugs. The table below lists the factors that were considered in designing and applying prior authorization to this drug/drug group, and a summary of the conclusions that Prime's assessment led to for each.

Factor	Conclusion
Indication	Yes: Consider for PA
Safety and efficacy	Yes: Consider for PA
Potential for misuse/abuse	No: PA not a priority
Cost of drug	Yes: Consider for PA

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
G12.0	Infantile spinal muscular atrophy, type I [Werdnig-Hoffmann]
G12.1	Other inherited spinal muscular atrophy
G12.25	Progressive spinal muscle atrophy
G12.8	Other spinal muscular atrophies and related syndromes
G12.9	Spinal muscular atrophy, unspecified

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

The preceding information is intended for non-Medicare coverage determinations. 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 Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under Part B for drugs which may be self-administered. The following link may be used to search for NCD, LCD, or LCA documents: https://www.cms.gov/medicare-coverage-database/search.aspx. Additional indications, including any preceding information, may be applied 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		
Jurisdictio	**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 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
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA
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