Onasemnogene Abeparvovec-xioi (Zolgensma®)

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the medical policy and a health plan or government program (e.g., TennCare), the express terms of the health plan or government program will govern.

POLICY

INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

Onasemnogene abeparvovec-xioi (Zolgensma) is an adeno 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.

All other indications are considered investigational.

PRESCRIBER SPECIALITIES

This medication must be prescribed by or in consultation with a neurologist or a physician who specializes in the treatment of spinal muscular atrophy.

COVERAGE CRITERIA

Note: Submission of medical records related to the medical necessity criteria is **REQUIRED** on all requests for authorizations. Records will be reviewed at the time of submission. Please provide documentation via direct upload through the PA web portal or by fax.

Onasemnogene abeparvovec-xioi is considered **medically appropriate** if **ALL** of the following conditions are met:

- Diagnosis of spinal muscular atrophy based on the results of SMA newborn screening
- Diagnosis of spinal muscular atrophy confirmed by genetic testing demonstrating bi-allelic mutations in the survival motor neuron 1 (*SMN1*) gene as stated below
  - deletion of both copies of the *SMN1* gene **OR**
  - compound heterozygous mutations of the *SMN1* gene (defined below):
    - pathogenic variant(s) in both copies of the *SMN1* gene
    - pathogenic variant in 1 copy and deletion of the second copy of the *SMN1*gene.
- Documentation of a genetic test confirms no more than **3 copies** of the *SMN2* gene.
- The patient is less than 2 years of age at the time of infusion of onasemnogene abeparvovec- xioi.
- Documentation of baseline laboratory assessments such as AST, ALT, total bilirubin, and prothrombin time.
- The patient does not have advanced spinal muscular atrophy (e.g., complete paralysis of limbs, permanent ventilator dependence).
- Baseline anti- adeno-associated virus serotype 9 (AAV9) antibody titers <1:50

- Patient must not have previously been administered onasemnogene abeparvovec-xioi.
- Will not be used in combination with other agents for SMA (e.g., Risdiplam, Nusinersen, etc.).

CONTINUATION OF THERAPY

Onasemnogene abeparvovec-xioi is **NOT** considered **medically appropriate** for renewal.

Note: Please see Additional Information

LENGTH OF AUTHORIZATION

Coverage will be provided as a one-time infusion and **may not** be renewed.

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

REFERENCES

1. BlueCross BlueShield Association. Evidence Positioning System. (8:2022). *Treatment for Spinal Muscular Atrophy* (5.01.28). Retrieved October , 2022 from https://www.evidencepositioningsystem.com/. (54 articles and/or guidelines reviewed)
2. MICROMEDEX Healthcare Series. Drugdex Evaluations. (2024, August). *Onasemnogene abeparvovec-xioi*. Retrieved January 9, 2025 from MICROMEDEX Healthcare Series.
3. Prior, T.W., Finanger, E. (2000, February [Updated 2024, September]). *Spinal Muscular Atrophy*. In: Adam, M. P., Ardinger, H. H., Pagon, R. A., et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2019. Bookshelf URL: https://www.ncbi.nlm.nih.gov/books/.
4. Zolgensma [package insert]. Bannockburn, IL; AveXis, Inc., July 2024. Retrieved January, 2025.

EFFECTIVE DATE 5/31/2025

ID_BT