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

Zolgensma is 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.

Limitations of Use:

- The safety and effectiveness of repeat administrations of Zolgensma have not been evaluated.
- The use of Zolgensma in patients with advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence) has not been evaluated.

All other indications are considered experimental / investigational and not medically necessary.

DOCUMENATION

Submission of the following information is necessary to initiate the prior authorization review:

- Genetic testing results demonstrating bi-allelic pathogenic variants in the survival motor neuron 1 (SMN1) gene.
- Medical records (e.g., chart notes and/or laboratory reports) documenting baseline liver function, platelet count, troponin I levels, and creatinine.
- Documentation of a genetic test confirms no more than **3 copies** of the *SMN2* gene

PRESCRIBER SPECIALITIES

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

COVERAGE CRITERIA

Spinal Muscular Atrophy

Authorization of one dose total may be granted for treatment of spinal muscular atrophy (SMA) **when all of the following** criteria are met:

- Member has a genetically confirmed diagnosis of SMA, with documentation of bi-allelic pathogenic variants in the survival motor neuron 1 (SMN1) gene (deletions or point mutations).
- Member has no more than 3 copies of SMN2.
- Member experienced onset of disease before 6 months of age.
- Member is less than 2 years of age.
- Member does not have advanced SMA, including but not limited to any of the following:
  - Complete paralysis of limbs
  - Invasive ventilatory support (tracheostomy)
  - Respiratory assistance for 16 or more hours per day (including non-invasive respiratory support) continuously for 14 or more days in the absence of acute reversible illness (excluding perioperative ventilation).
- Member has an anti-adeno-associated virus 9 (AAV9) antibody titer less than or equal to 1:50 as determined by Enzyme-linked Immunosorbent Assay (ELISA) binding immunoassay.
- Member does not have an active infectious process (e.g. viral, bacterial, or febrile illness) prior to treatment.
- Member does not have a serious concomitant illness (e.g., severe liver or kidney disease, symptomatic cardiomyopathy).
- Liver function, platelet count, troponin I, and creatinine levels have been assessed at baseline and will be monitored after Zolgensma administration as clinically appropriate.
- Member's vaccination status will be up to date prior to Zolgensma administration.
- If the member is on nusinersen (Spinraza) or risdiplam (Evrysdi), it will be discontinued prior to administration of the requested drug.
- Member has not received Zolgensma, Itvisma, or other gene therapy previously.

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

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EFFECTIVE DATE 6/30/2026

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