PROVIDER POLICIES & PROCEDURES

ZOLGENSMA® (ONASEMNOGENE ABEPARVOVEC-XIOI)

The primary purpose of this document is to assist providers enrolled in the Connecticut Medical Assistance Program (CMAP) with the information needed to support a medical necessity determination for Zolgensma (onasemnogene abeparvovec-xioi) therapy. By clarifying the information needed for prior authorization of services, HUSKY Health hopes to facilitate timely review of requests so that individuals obtain the medically necessary care they need as quickly as possible.

Zolgensma is an adeno-associated virus vector-based gene therapy indicated for the treatment of infants and children less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the *survival motor neuron 1(SMN1)* gene. Zolgensma is given as a one-time intravenous infusion.

CLINICAL GUIDELINE

Coverage guidelines for Zolgensma are made in accordance with the Department of Social Services (DSS) Definition of Medical Necessity. The following criteria are guidelines *only*. Coverage determinations are based on an assessment of the individual and their unique clinical needs. If the guidelines conflict with the definition of Medical Necessity, the definition of Medical Necessity shall prevail.

Zolgensma may be considered medically necessary as an intravenous infusion for infants that have reached full-term gestational age and children under 2 years of age with SMA when the following criteria are met:

A. Zolgensma has been ordered by or in consultation with a physician specializing in the management of SMA or other neuromuscular disorders; **AND**
B. The individual has not been previously treated with Zolgensma; **AND**
C. If the individual is currently on other SMA gene-based therapy (e.g., Spinraza® [nusinersen] or EVRYSDI® [risdiplam]), it will be discontinued prior to administration of Zolgensma; **AND**
D. The diagnosis of SMA has been made by or in consultation with a physician with expertise in diagnosing SMA; **AND**
E. Genetic testing has been performed and confirmed biallelic mutations in the *survival motor neuron1(SMN1)* gene; **AND**
F. The individual does not have advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence*); **AND**
G. Testing for anti-AAV9 antibodies has been performed and confirmed an anti-AAV9 antibody titer of ≤ 1:50; **AND**
H. Liver function has been evaluated and will continue to be monitored for at least 3 months post infusion by clinical exam and an analysis of hepatic aminotransferases [aspartate aminotransferase (AST), alanine aminotransferase (ALT)], total bilirubin, and prothrombin time (PT); **AND**
I. Testing to obtain a baseline platelet count, and cardiac troponin-I has been performed and will continue to be performed on a regular basis for at least 3 months post infusion; **AND**

J. A description of the benefits, risks and treatment expectations has been provided to the parent or guardian; **AND**
K. Zolgensma administration will follow the current FDA Zolgensma labeling for dosing protocol.

*Permanent ventilator dependence is defined as requiring invasive ventilation (tracheostomy) or respiratory assistance for 16 or more hours per day (including noninvasive ventilator support) continuously for 14 or more days in the absence of an acute reversible event, excluding perioperative ventilation.

Repeat administration of Zolgensma has not been clinically evaluated and is therefore considered investigational and not medically necessary.

NOTE: EPSDT Special Provision
Early and Periodic Screening, Diagnosis, and Treatment (EPSDT) is a federal Medicaid requirement that requires the Connecticut Medical Assistance Program (CMAP) to cover services, products, or procedures for Medicaid enrollees under 21 years of age where the service or good is medically necessary health care to correct or ameliorate a defect, physical or mental illness, or a condition identified through a screening examination. The applicable definition of medical necessity is set forth in Conn. Gen. Stat. Section 17b-259b (2011) [ref. CMAP Provider Bulletin PB 2011-36].

PROCEDURE
Prior authorization Zolgensma is required. Requests for coverage of Zolgensma will be reviewed in accordance with procedures in place for reviewing requests for gene therapy. Coverage determinations will be based upon a review of requested and/or submitted case-specific information.

The following information is needed to review requests for Zolgensma:
1. Fully completed State of Connecticut, Department of Social Services HUSKY Health Program Zolgensma® (onasemnogene abeparvovec-xioi) Prior Authorization Request form (to include physician's order and signature);
2. Clinical information from the treating neurologist supporting the medical necessity of the treatment as outlined in the *Clinical Guideline* section of this policy; and
3. Other information as requested.

EFFECTIVE DATE
This Policy is effective for prior authorization requests for Zolgensma for HUSKY Health Program individuals on or after August 1, 2019.

LIMITATIONS
N/A

CODES

Code	Description
J3399	Injection, onasemnogene abeparvovec-xioi, per treatment, up to 5x10

DEFINITIONS

1. **HUSKY A**: Connecticut children and their parents or a relative caregiver; and pregnant women may qualify for HUSKY A (also known as Medicaid). Income limits apply.
2. **HUSKY B**: Uninsured children under the age of 19 in higher income households may be eligible for HUSKY B (also known as the Children's Health Insurance Program) depending on their family income level. Family cost-sharing may apply.
3. **HUSKY C**: Connecticut residents who are age 65 or older or residents who are ages 18-64 and who are blind, or have another disability, may qualify for Medicaid coverage under HUSKY C (this includes Medicaid for Employees with Disabilities (MED-Connect), if working). Income and asset limits apply.
4. **HUSKY D**: Connecticut residents who are ages 19-64 without dependent children and who: (1) do not qualify for HUSKY A; (2) do not receive Medicare; and (3) are not pregnant, may qualify for HUSKY D (also known as Medicaid for the Lowest-Income populations).
5. **HUSKY Health Program**: The HUSKY A, HUSKY B, HUSKY C, HUSKY D and HUSKY Limited Benefit programs, collectively.
6. **HUSKY Limited Benefit Program or HUSKY, LBP**: Connecticut's implementation of limited health insurance coverage under Medicaid for individuals with tuberculosis or for family planning purposes and such coverage is substantially less than the full Medicaid coverage.
7. **Medically Necessary or Medical Necessity**: (as defined in Connecticut General Statutes § 17b-259b) Those health services required to prevent, identify, diagnose, treat, rehabilitate or ameliorate an individual's medical condition, including mental illness, or its effects, in order to attain or maintain the individual's achievable health and independent functioning provided such services are: (1) Consistent with generally-accepted standards of medical practice that are defined as standards that are based on (A) credible scientific evidence published in peer-reviewed medical literature that is generally recognized by the relevant medical community, (B) recommendations of a physician-specialty society, (C) the views of physicians practicing in relevant clinical areas, and (D) any other relevant factors; (2) clinically appropriate in terms of type, frequency, timing, site, extent and duration and considered effective for the individual's illness, injury or disease; (3) not primarily for the convenience of the individual, the individual's health care provider or other health care providers; (4) not more costly than an alternative service or sequence of services at least as likely to produce equivalent therapeutic or diagnostic results as to the diagnosis or treatment of the individual's illness, injury or disease; and (5) based on an assessment of the individual and his or her medical condition.
8. **Prior Authorization**: A process for approving covered services prior to the delivery of the service or initiation of the plan of care based on a determination by CHNCT as to whether the requested service is medically necessary.

RESOURCES AND REFERENCES:

- Al-Zaidy S, Pickard AS, Lotha K, Alfano LN, Lowes L, Paul G, et al. Health outcomes in spinal muscular atrophy type 1 follow AVXS-101 gene replacement therapy. Pediatr Pulmonol. 2018. https://doi.org/10.1002/ppul.24203
- Gene Transfer Clinical Trial for Spinal Muscular Atrophy Type 1. Retrieved from https://clinicaltrials.gov (Identification No. NCT02122952)
- Study of Intrathecal Administration of AVXS-101 for Spinal Muscular Atrophy (STRONG). Retrieved from https://clinicaltrials.gov (Identification No. NCT03381729)
- Zolgensma Full Prescribing Information. Bannockburn, IL: AveXis, Inc.; 2019. Available at: https://www.avexis.com/content/pdf/prescribing_information.pdf**.** Accessed on June 5, 2019.

- Zolgensma Treatment Guide for Healthcare Providers. Bannockburn, IL: AveXis, Inc.; 2019. Available at: https://www.zolgensma.com/. Accessed on June 5, 2019.

PUBLICATION HISTORY

Date		Action Taken
Original Publication		Approved at the June 12, 2019 Medical Reviewer meeting. Approved by the CHNCT Clinical Quality Subcommittee on June 19, 2019. Approved by DSS on June 21, 2019.
Update	June 2020	Added criteria related to previous gene therapy/transitioning from one therapy to another. Removed criteria pertaining to treatment with steroids. Change approved at the May 13, 2020 Medical Reviewer meeting. Change approved by the CHNCT Clinical Quality Subcommittee on June 15, 2020. Approved by DSS on June 19, 2020.
Update	June 2021	Revised criteria to add the following: Individual is not on concurrent gene based therapy for SMA. Updated coding. Changed neurologist to a physician with expertise in diagnosing and treating neuromuscular conditions. Changes approved at the April 14, 2021 CHNCT Medical Reviewer meeting. Reviewed and approved at the June 21, 2021 CHNCT Clinical Quality Subcommittee meeting. Approved by DSS on June 28, 2021.
Update	June 2022	Updated Clinical Guideline section. Clarified language Letter C – individuals not currently on other SMA gene-based therapy. Updated definition of permanent ventilator dependence to match FDA labeling for Zolgensma. Changes approved at the May 11, 2022 CHNCT Medical Reviewer Meeting. Changes approved by the CHNCT Clinical Quality Subcommittee on June 20, 2022. Approved by DSS on July 5, 2022.
Reviewed	June 2023	Reviewed and approved without changes at the May 10, 2023, CHNCT Medical Reviewer Meeting. Approved by the CHNCT Clinical Quality Subcommittee on June 19, 2023. Approved by DSS on June 28, 2023.

Reviewed	May 2024	Reviewed and approved without changes at the May 22, 2024 CHNCT Medical Reviewer Meeting. Approved by the CHNCT Clinical Quality Subcommittee on June 17, 2024. Approved by DSS on June 26, 2024.