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 a neurologist experienced in treating SMA;
B. The diagnosis of SMA has been made by a neurologist with expertise in diagnosing SMA. For purposes of this guideline, neurologist refers to a board-certified neurologist, preferably specializing in pediatric neurology;
C. Genetic testing has been performed and confirmed biallelic mutations in the *survival motor neuron1(SMN1)* gene;
D. The individual does not have advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence*);
E. Testing for anti-AAV9 antibodies has been performed and confirmed an anti-AAV9 antibody titer of ≤ 1:50;
F. 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);
G. 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;
H. A description of the benefits, risks and treatment expectations has been provided to the parent or guardian;
I. A 30 day corticosteroid regimen equivalent to oral prednisolone at 1 mg/kg/day will be initiated 24 hours prior to infusion; and
J. 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 21 or more days in the absence of an acute reversible event.

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
C9399	Unclassified drugs or biologicals
J3590	Unclassified biologics

DEFINITIONS
1. **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.

2. **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.