POLICY AND PROCEDURE

POLICY NAME: Onasemnogene Abeparvovec-xioi (Zolgensma®) Criteria	**POLICY ID:** TX.PHAR.79
BUSINESS UNIT: Superior HealthPlan	**FUNCTIONAL AREA:** Pharmacy
EFFECTIVE DATE: 10/01/19	**PRODUCT(S):** STAR, STAR Plus, STAR Kids, STAR Health, CHIP
REVIEWED/REVISED DATE: 02/19/20, 03/29/21, 06/25/21, 04/11/22, 9/16/22, 11/28/22, 01/23/23	
REGULATOR MOST RECENT APPROVAL DATE(S): N/A	

POLICY STATEMENT:
It is the policy of Superior HealthPlan (SHP) to follow state guidance for medical necessity review of onasemnogene abeparvovec-xioi (Zolgensma).

PURPOSE:
This medication is a pass through drug and should follow state guidance for medical necessity review for Medicaid/CHIP due to the manner in which it is reimbursed. All determinations will be performed by a Superior Medical Director. A pharmacy clinician will review the prior authorization request and make a recommendation to the Medical Director but will not make the ultimate determination on any case.

Additionally, this medication is a Precision Drug. Centene's Precision Drug Action Committee (PDAC) creates a standardized approach for Centene to manage Precision Drugs and the associated costs for their administration, prior to members presenting with a request for one of these agents. All Precision Drug requests or potential requests must be reported to the PDAC for tracking, regardless of whether agents are carved out, passed through, etc. All Precision Drug medical necessity determinations will be supported by PDAC UM recommendation, utilizing specialist input as directed and allowed by turnaround times.

In addition, Zolgensma (J3399) requests are limited to one approval per lifetime, by any provider. This includes if Zolgensma was previously approved with the unlisted procedure code J3590.

Description/Mechanism of Action:
Onasemnogene abeparvovec-xioi (Zolgensma) is a recombinant AAV9-based gene therapy designed to deliver a copy of the gene encoding the human survival motor neuron (SMN) protein. Spinal muscular atrophy (SMA) is caused by a bi-allelic mutation in the SMN1 gene, which results in insufficient SMN protein expression. Intravenous administration of onasemnogene abeparvovec-xioi resulting in cell transduction and expression of the SMN protein has been observed in 2 human cases.

Formulations:
Onasemnogene abeparvovec-xioi (Zolgensma): suspension for intravenous infusion, supplied as single-use vials. Zolgensma is provided in a kit containing 2 to 9 vials, as a combination of 2 vial fill volumes (either 5.5 mL or 8.3 mL). All vials have a nominal concentration of 2.0×10^{13} vector genomes (vg) per mL. Zolgensma is shipped frozen in 10 mL vials. Each vial of Zolgensma contains an extractable volume of not less than either 5.5 mL or 8.3 mL.

FDA Approved Indications:
Onasemnogene abeparvovec-xioi (Zolgensma) is a one-time infusion therapy indicated for the treatment of spinal muscular atrophy (SMA) with biallelic mutations in the survival motor neuron 1 (SMN1) gene in clients who are 24 months of age or younger.

SCOPE:
Superior HealthPlan Pharmacy Department, Medical Directors

DEFINITIONS:
PDAC = Precision Drug Action Committee
UM = Utilization Management

POLICY:
It is the policy of Superior HealthPlan (SHP) to follow state guidance for medical necessity review of onasemnogene abeparvovec-xioi (Zolgensma). This medication is a pass through drug and should follow state guidance for medical necessity review for Medicaid/CHIP due to the manner in which it is reimbursed. All determinations will be performed by a Superior Medical Director. A pharmacy clinician will review the prior authorization request and make a recommendation to the Medical Director but will not make the ultimate determination on any case

PROCEDURE:

I. Approval Criteria

A. Spinal Muscular Atrophy (SMA)

1. All prior authorization approvals or denials will be determined by a SHP Medical Director.
2. Medical necessity determinations will be supported by PDAC UM recommendation. The SHP pharmacy clinician will review the UM recommendation with the prior authorization request for clinical appropriateness and make a recommendation to the SHP Medical Director but will not make the ultimate determination on any case.
3. Member age is 24 months of age or younger.
4. Zolgensma will not be a benefit for clients with a tracheostomy or invasive ventilator support.
5. Medical record supporting any of the following mutation or deletion of genes in chromosome 5q (a, b, or c):
    a. Homozygous gene deletion of the SMN1 gene (e.g., absence of SMN1 gene)
    b. Homozygous mutation of the SMN1 gene (e.g., biallelic mutation of exon 7)
    c. Compound heterozygous mutation in the SMN1 gene (e.g., deletion of SMN1 exon 7 [allele 1] and mutation of SMN1 [allele 2])
6. Confirmed diagnosis of SMA (diagnosis code G120) based on gene mutation analysis with biallelic SMN1 mutation (deletion or point mutation).
7. Administration of onasemnogene abeparvovec-xioi (Zolgensma) may cause serious liver injury or failure. Providers must meet the following to administer the drug (a, b, and c):
    a. Client's liver function must be examined by clinical examination and laboratory testing (e.g., hepatic aminotransferases (aspartate aminotransferase (AST) and alanine aminotransferase (ALT), total bilirubin, and prothrombin time) before infusion of Zolgensma.
    b. Systemic corticosteroid must be administered before and after the administration of the drug.
    c. Provider must continue monitoring the client's liver function at least 3 months after the drug infusion.
8. Evaluation of motor skill and function must be documented using a standardized test. However, it is not a prerequisite of therapy and should not delay treatment. Standardized testing tools that may be used to evaluate motor skill/function include, but are not limited to:
    a. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorder (CHOP-INTEND) Score
    b. Bayley scale of infant and toddler development screening test
    c. WHO Multicenter Growth Reference Study (WHO MGRS)
9. Baseline documentation of AAV9 antibody titer of 1:50 or lower, as determined by enzyme-linked immunosorbent assay (ELISA)
10. Physician attestation that client has not received prior onasemnogene abeparvovec-xioi-xioi (Zolgensma) therapy
11. If nusinersen (Spinraza) (procedure code J2326) or Evrysdi (risdiplam) have been previously prescribed, the prescriber must also provide the following documentation of one of the following before switching to onasemnogene abeparvovec-xioi (Zolgensma) therapy:
    a. Evidence of clinical deterioration (e.g., decreased physical function and motor skill/function test scores) while on nusinersen (Spinraza) or risdiplam (Evrysdi) therapy
    b. Neurologist attestation to the discontinuation of nusinersen (Spinraza) or risdiplam (Evrysdi) therapy
12. Documentation of the requested dosage and administration schedule, including the number of injections to be administered during the prior authorization period, the requested units per injection, and the dosage calculation.

Approval duration: Only 1 dose per lifetime will be provided on this drug regardless of Provider.

REFERENCES:

Texas Medicaid Provider Procedures Manual: Outpatient Drug Services Handbook

Zolgensma (onasemnogene abeparvovec-xioi-xioi) [prescribing information]. Bannockburn, IL: AveXis Inc; May 2019

ATTACHMENTS:

ROLES & RESPONSIBILITIES: N/A

REGULATORY REPORTING REQUIREMENTS: N/A

REVISION LOG

REVISION TYPE	REVISION SUMMARY	DATE APPROVED & PUBLISHED
New Policy Document		09/27/19
Ad Hoc Review	Per the Vendor Drug Program's guidance, adjusted step 7 to read "at least 2 copies of SMN2"	02/19/20
Ad Hoc Review	Per the Vendor Drug Program's guidance: • Adjusted step 7 to read "3 or less copies of SMN2" • Added options for motor skill and function test and statement that they must be documented but are not a prerequisite for therapy and should not delay treatment. • Added Evrysdi (risdiplam) in step 10 to specify clients previously on this drug are to follow same criteria as clients on Spinraza (nusinersen) • Added procedure code J3399 to policy	03/29/21
Ad Hoc Review	Step #11 – added "documentation of one of the following" to match the TMPPM Manual Criteria. Criteria	06/25/21
Ad Hoc Review	Removed "Onset of clinical signs and symptoms consistent with SMA at birth up to 6 months of age." As it is no longer in the TMPPM criteria Updated reference to include TMPPM Manual	04/11/22
Ad Hoc Review	Per the Vendor Drug Program's guidance: Added following update to clinical policy- The administration of onasemnogene abeparvovec-xioi (Zolgensma) may cause serious liver injury or failure. Providers must meet the following to administer the drug: • The client's liver function must be examined by clinical examination and laboratory testing (e.g., hepatic aminotransferases (aspartate aminotransferase (AST) and alanine aminotransferase (ALT), total bilirubin, and prothrombin time) before infusion of Zolgensma • Systemic corticosteroid must be administered prior to and after the administration of the drug. • The provider must continue monitoring the client's liver function at least 3 months after the drug infusion.	09/16/22
Ad Hoc Review	Per HHSC guidance: Removing the Spinal Muscular Atrophy (SMA) type and the number of SMN 2 copies language from the Zolgensma clinical policy. Updated criteria point to read: Confirmed diagnosis of SMA (diagnosis code G120) based on gene mutation analysis with bi-allelic SMN1 mutation (deletion or point mutation).	11/28/22

Ad Hoc Review	Per HHSC guidance: 1) A neurologist prescription or consultation is not required. 2) The diagnosis requirement for prior authorization requests is a confirmed diagnosis of spinal muscular atrophy (diagnosis code G120), based on gene mutation analysis with biallelic survival motor neuron 1 mutation (deletion or point mutation). To address: 1) Removing criteria point: Prescribed by or in consultation with a board-certified neurologist or pediatric neurologist who is familiar with the diagnosis and management of spinal muscular atrophy and 2) Criteria point regarding confirmed diagnosis requirements updated in Ad Hoc Review dated 11/28/22.	01/23/23

POLICY AND PROCEDURE APPROVAL

The electronic approval retained in RSA Archer, the Company's P&P management software, is considered equivalent to a signature.