Policy and Procedure

DEPARTMENT: Pharmacy, Medical Directors	**DOCUMENT NAME:** Onasemnogene Abeparvovec-xioi (Zolgensma®) Criteria
PAGE: 1 of 4	**REPLACES DOCUMENT:**
APPROVED DATE:	**RETIRED:**
EFFECTIVE DATE: 10/01/2019	**REVIEWED/REVISED:** 2/19/2020
PRODUCT TYPE STAR, STAR Plus, STAR Kids, STAR Health, CHIP	**REFERENCE NUMBER:** TX.PHAR.79

SCOPE:

Superior HealthPlan Pharmacy Department, Medical Directors

PURPOSE:

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.
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 requests submitted with the unlisted procedure code J3590 will be limited to once per lifetime, by any provider. If the code is updated in the future it will still be limited to once per lifetime.

BACKGROUND:

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.

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. Onset of clinical signs and symptoms consistent with SMA at birth up to 6 months of age.
4. Member age is 24 months of age or younger.
5. Zolgensma will not be a benefit for clients with a tracheostomy or invasive ventilator support.
6. Medical record supporting the mutation or deletion of genes in chromosome 5q.

   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])
7. Confirmed diagnosis of Type I SMA (diagnosis code G120) based on gene mutation analysis with biallelic SMN1 mutation (deletion or point mutation) and at least 2 copies of SMN2
8. Documentation of the Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorder (CHOP-INTEND) score to evaluate the client's motor skills
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) has been previously prescribed, the prescriber must also provide the following documentation before switching to onasemnogene abeparvovec-xioi-xioi (Zolgensma) therapy:
   a. Evidence of clinical deterioration (e.g., decreased physical function and CHOP-INTEND score) while on nusinersen (Spinraza) therapy
   b. Neurologist attestation to the discontinuation of nusinersen (Spinraza) therapy
12. 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.
13. 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.

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REFERENCES:

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

REVISION LOG

REVISION	DATE
Policy created	09/27/2019
Per the Vendor Drug Program's guidance, adjusted step 7 to read "at least 2 copies of SMN2"	2/19/2020

POLICY AND PROCEDURE APPROVAL

Karen Tadlock, V.P., Pharmacy Operations — Approval on file

Dr. David Harmon, Sr. V.P., Chief Medical Officer — Approval on file

Pharmacy & Therapeutics Committee: — Approval on file

NOTE: The electronic approval retained in Compliance 360, Centene's P&P management software, is considered equivalent to a physical signature.