Policy Title:	Zolgensma (onasemnogene abeparvovec-xioi) Intravenous		
Policy Number:	*To be determined*	**Department:**	PHA
Effective Date:	08/14/2019		
Review Date:	08/14/2019		
Revision Date:	08/14/2019		

Purpose: To support safe, effective and appropriate use of Zolgensma (onasemnogene abeparvovec-xioi) in the treatment of spinal muscular atrophy (SMA).

Scope: Medicaid, Exchange, Integrity

Policy Statement:

Zolgensma (onasemnogene abeparvovec-xioi) is covered under the Medical Benefit when used within the following guidelines. Use outside of these guidelines may result in non-payment unless approved under an exception process.

Procedure:

Coverage of Zolgensma (onasemnogene abeparvovec-xioi) will be reviewed prospectively via the prior authorization process based on criteria below.

Initial Criteria Coverage:

- Patient must have a confirmed diagnosis* of spinal muscular atrophy (SMA type 1) and documentation of the mutation or deletion of genes in chromosome 5q resulting in one of the following:
    - Homozygous gene deletion or mutation of SMN1 gene (e.g., homozygous deletion of exon 7 at locus 5q13); OR
    - Compound heterozygous mutation of SMN1 gene (e.g., deletion of SMN1 exon 7 [allele 1] and mutation of SMN1 [allele 2]); AND
- Patient must have diagnosis of Type 1 SMA by a board certified pediatric neurologist; AND
- Patient has 1-2 copies of SMN2 without SMN2 gene modifier mutation (c.859G.C); AND
- Patient displays clinical signs and symptoms prior to and including up to 6 months of age; AND
- Patient is less than two years of age at the time of infusion; AND
- For use in a neonatal patient born prematurely, the full-term gestational age has been reached; AND
- Patient must weigh between 2.6 kg and 13.5 kg; AND
- Patient is not dependent on either of the following:
    - Invasive ventilation or tracheostomy

- Use of non-invasive ventilation beyond use for naps and nighttime sleep
- Patient does not have limb paralysis; AND
- Patient is not to receive routine concomitant SMN modifying therapy (e.g. Spinraza). Patient's medical record will be reviewed and any current authorizations for SMN modifying therapy will be terminated upon Zolgensma approval; AND
- Pediatric neurologist documents baseline anti-AAV9 antibody titer of less than or equal to 1:50; AND
- Patient does not have pre-existing hepatic insufficiency and prescriber submits baseline documentation of baseline AST, ALT, total bilirubin, and prothrombin time lab values; AND
- Submission of medical records (chart notes, laboratory testing) confirming the patient's most recent **CHOP INTEND** score is greater than or equal to 40; AND
- Patient cannot have acute viral infection(s) and must be negative for HIV, Hepatitis B or C, and Zika virus.
- Documentation that patient is up to date on all childhood vaccinations or has medical documentation to support a contraindication to a vaccine; AND
- Patient will receive Zolgensma (onasemnogene abeparvovec-xioi) intravenously within accordance of the United States Food and Drug Administration approved labeling; AND
- Patients have not been treated in the past with Zolgensma (onasemnogene abeparvovec-xioi); AND
- Authorizations will only be granted if Zolgensma (onasemnogene abeparvovec-xioi) is provided at a Neighborhood Health Plan of Rhode Island authorized and approved facility for Zolgensma (onasemnogene abeparvovec-xioi) administration.

*Genetic testing must be obtained from an in-network provider (such as Labcorp or Myriad Genetics).

Renewal coverage:

- Cannot be renewed, approval is for one kit once per lifetime

Coverage durations:

- Initial coverage: one kit for one time infusion

Investigational Use: All therapies are considered investigational when used at a dose or for a condition other than those that are recognized as medically accepted indications as defined in any one of the following standard reference compendia: American Hospital Formulary Service Drug Information (AHFS-DI), Thomson Micromedex DrugDex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs, or Peer-reviewed published medical literature indicating that sufficient evidence exists to support use. Neighborhood does not provide coverage for drugs when used for investigational purposes.

Zolgensma (onasemnogene abeparvovec-xioi) is not proven or medically necessary for the treatment of pre-symptomatic patients diagnosed by newborn screening who are unlikely to develop Type I

SMA, for the treatment of Type II, Type III, Type IV SMA, or for SMA without chromosome 5q mutations or deletions.

Applicable Codes:
Below is a list of billing codes applicable for covered treatment options. The below tables are provided for reference purposes and may not be all-inclusive. Requests received with codes from tables below do not guarantee coverage. Requests must meet all criteria provided in the procedure section.

The following HCPCS/CPT codes are:

HCPCS/CPT Code	Description
C9399	Unclassified drugs or biologicals

References:

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8. Day JW, Feltner DE, Ogrinc F, et al. AVXS-101, gene-replacement therapy for spinal muscular atrophy type 1 (SMA1): Pivotal study (STR1VE) update. Poster presented at: The 23rd International Annual Congress of the World Muscle Society, Mendoza, Argentina, October 2–6, 2018.
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15. Pre-Symptomatic Study of Intravenous AVXS-101 in Spinal Muscular Atrophy (SMA) for Patients With Multiple Copies of SMN2 (SPR1NT). Clinicaltrials.gov website. https://clinicaltrials.gov/ct2/show/NCT03505099?term=AVXS-101&rank=1 Accessed October 19, 2018.
16. Study of Intrathecal Administration of AVXS-101 for Spinal Muscular Atrophy (STRONG). Clinicaltrials.gov website. https://clinicaltrials.gov/ct2/show/NCT03381729?term=AVXS-101&rank=3 Accessed October 19, 2018.
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