Geisinger

POLICIES AND PROCEDURE MANUAL

Policy: MBP 199.0

Section: Medical Benefit Pharmaceutical Policy

Subject: Zolgensma (onasemnogene abeparvovec-xioi)

I. Policy:
Zolgensma (onasemnogene abeparvovec-xioi)

II. Purpose/Objective:
To provide a policy of coverage regarding Zolgensma (onasemnogene abeparvovec-xioi)

III. Responsibility:
A. Medical Directors
B. Medical Management
C. Pharmacy Department

IV. Required Definitions
1. Attachment – a supporting document that is developed and maintained by the policy writer or department requiring/authoring the policy.
2. Exhibit – a supporting document developed and maintained in a department other than
3. the department requiring/authoring the policy.
4. Devised – the date the policy was implemented.
5. Revised – the date of every revision to the policy, including typographical and grammatical changes.
6. Reviewed – the date documenting the annual review if the policy has no revisions necessary.

V. Additional Definitions
Medical Necessity or Medically Necessary means Covered Services rendered by a Health Care Provider that the Plan determines are:

a. appropriate for the symptoms and diagnosis or treatment of the Member's condition, illness, disease or injury;
b. provided for the diagnosis and the direct care and treatment of the Member's condition, illness disease or injury;
c. in accordance with current standards good medical treatment practiced by the general medical community;
d. not primarily for the convenience of the Member, or the Member's Health Care Provider; and
e. the most appropriate source or level of service that can safely be provided to the Member. When applied to hospitalization, this further means that the Member requires acute care as an inpatient due to the nature of the services rendered or the Member's condition, and the Member cannot receive safe or adequate care as an outpatient

Medicaid Business Segment
Medically Necessary — A service, item, procedure, or level of care compensable under the Medical Assistance program that is necessary for the proper treatment or management of an illness, injury, or disability is one that:

i. Will, or is reasonably expected to, prevent the onset of an illness, condition, injury or disability.
ii. Will, or is reasonably expected to, reduce or ameliorate the physical, mental or developmental effects of an illness, condition, injury or disability.
iii. Will assist the Member to achieve or maintain maximum functional capacity in performing daily activities, taking into account both the functional capacity of the Member and those functional capacities that are appropriate for Members of the same age.

DESCRIPTION:
Zolgensma (onasemnogene abeparvovec-xioi) is a recombinant adeno-associated virus vector-based gene therapy that delivers a normal copy of the gene encoding human survival motor neuron (SMN) protein. Zolgensma is used to treat pediatric patients less than 2 years of age with Spinal Muscular Atrophy (SMA). Also indicated for SMA is Spinraza (nusinersen), a survival motor neuron-2 (SMN2)-directed antisense Oligonucleotide that was shown to increase exon 7 inclusion in SMN2 messenger ribonucleic acid (mRNA) transcripts and production of full-length SMN protein.

CRITERIA FOR USE: Requires Prior Authorization by Medical Director or Designee

Zolgensma (onasemnogene abeparvovec-xioi) will be considered medically necessary for all lines of business when ALL of the following criteria are met:

- Medical record documentation of a confirmed diagnosis of 5q Spinal Muscular Atrophy (SMA) by genetic testing with results showing one of the following:
    - Homozygous exon 7 gene deletion OR
    - Homozygous exon 7 conversion mutation OR
    - Compound heterozygous exon 7 mutation

  OR
    - Medical record documentation of diagnostic testing confirming zero (0) SMN1 copies

  AND
- Prescription is being prescribed by a neurologist or pediatric neurologist **AND**
- Medical record documentation that the patient will be less than 2 years of age at the time of dosing **AND**
- Medical record documentation that patient does not have anti-AAV9 antibody titers >1:50 as determined by ELISA (within two weeks of the anticipated infusion date) **AND**
- Medical record documentation that patient is not permanent ventilator-dependent **AND**
- Medical record documentation that patient has not received a prior dose of Zolgensma **AND**
- Medical record documentation that patient will not receive routine concomitant SMN modifying therapy (e.g. Spinraza) with Zolgensma (Note: Any current authorizations for SMN modifying therapy will be terminated upon Zolgensma approval)

AUTHORIZATION DURATION: 30 days (to receive the one-time infusion) or the date equivalent to the patient age of 2 years (whichever is less).
Note: Zolgensma must be administered prior to 2 years of age. If the medication is not administered during this initial month, re-review should occur to ensure patient is still eligible for the medication, particularly the anti-AAV9 antibody titers and ventilator dependence.

QUANTITY LIMIT: One (1) Zolgensma infusion per lifetime

Note: For Medicaid (GHP Family), any requests for services that do not meet criteria set in the PARP will be evaluated on a case-by-case basis.

LINE OF BUSINESS:

Eligibility and contract specific benefit limitations and/or exclusions will apply. Coverage statements found in the line of business specific benefit document will supersede this policy.

This policy will be revised as necessary and reviewed no less than annually**.**

Devised: 7/16/19

Revised: 5/9/23 (LOB carve out, Medicaid business segment)**,** 7/26/23 (Description update)

Reviewed: 7/1/20, 5/27/21, 5/11/22 (Medicaid PARP statement)