JOHNS HOPKINS MEDICINE / JOHNS HOPKINS HEALTHCARE	Johns Hopkins HealthCare LLC **Pharmacy Public** **Medical Management Drug Policies**	*Policy Number*	MMDP008
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	Subject **Spinraza and Zolgensma**	*Revision Date*	11/10/2021
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This document applies to the following Participating Organizations:

US Family Health Plan

Keywords: spinraza, zolgensma

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I. POLICY

Spinraza (nusinersen) and Zolgensma (abeparvovec-xioi) will require prior authorization for medical benefit coverage to ensure appropriate use. The process for initiating a prior authorization request can be found in policy PHARM 20.

II. POLICY CRITERIA

A. **Spinraza** (nusinersen) may be approved for treatment of Spinal Muscular Atrophy (SMA) in patients meeting All the following criteria:

1. Patient has a diagnosis of SMA Type 1, 2, or 3
   a. * Patients with SMA Type 2 and 3 must have some functional upper extremity use
2. Documentation confirming diagnosis by FDA approved genetic diagnostic testing:
   a. Patient has at least 2 copies of survival motor neuron 2 (SMN2)
      AND
   b. Testing showing 5q SMA for any of the following:
      i. homozygous gene deletion, **OR**
      ii. homozygous conversion mutation, **OR**
      iii. compound heterzygote
3. Patient is not dependent on invasive ventilation or tracheostomy
4. Patient is not dependent on non-invasive ventilation beyond use for naps and nighttime sleep
5. Prescriber is a neurologist with expertise in treating SMA
6. Documentation that quantitative spot urine testing, platelet count and coagulation testing has been performed at baseline
7. Documentation showing baseline motor examination utilizing at least one of the following exams (based on patient age and motor ability) to establish baseline motor ability:
   a. Hammersmith Infant Neurological Exam (HINE)
   b. Hammersmith Functional Motor Scale Expanded (HFMSE)

c. Upper Limb Module Test (non-ambulatory)
d. Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND)

8. Patient has not previously received gene replacement therapy for SMA.
9. Spinraza is dosed according to FDA approved dosing guidelines, and administered intrathecally by a physician or other healthcare professional with experience in performing lumbar punctures

B. **Zolgensma** (onasemnogene abeparvovec-xioi) may be approved for treatment of SMA in patients meeting All the following criteria:

1. Patient is less than 2 years of age, and has a diagnosis of SMA Type 1
2. Documentation has been submitted confirming diagnosis by FDA approved genetic diagnostic testing:
    1. Patient has bi-allelic mutations in the survival motor neuron 1 (SMN1) gene (deletions or point mutations) AND
    2. Patient has at least 2 copies of SMN2 AND
    3. Patient has an absence of the c.859G>C modification in exon 7 of the SMN2 gene
3. Documentation has been submitted showing the following:
    1. Patient had onset of clinical SMA symptoms before 6 months of age
    2. Patient does not have advanced SMA, evidenced by, but limited to any of the following:
        1. Complete paralysis of limbs
        2. Dependence on invasive ventilation or tracheostomy
        3. Dependence on non-invasive ventilation beyond use for naps and nighttime sleep
    3. Patient has an anti-adeno-associated virus 9 (AAV9) antibody titer less than or equal to 1:50 per Enzyme-linked Immunosorbent Assay (ELISA) binding immunoassay
4. Prescriber is a neurologist with expertise in treating SMA
5. Patient has not previously received gene replacement therapy for SMA
6. Zolgensma is dosed according to FDA approved dosing guidelines

III. AUTHORIZATION PERIOD/LIMITATIONS

A. **Spinraza**

1. Initial authorization period will be restricted to 3 months, requiring provider follow-up for continued treatment approval
2. Continuation of therapy authorization will be granted in 6 months increments with clinical documentation showing clinical benefit from treatment.
    I. Documented repeat motor testing must be submitted after the most recent Spinraza dose, but not more thant 1 month prior to the next scheduled dose. The same motor test used to establish the patient's baseline motor ability must be used for the repeat test, unless it is determined that the original test is no longer appropriate:
        i. Repeat motor testing must shows a response to treatment as defined by the following:
            A. HINE:
                a. Improvement or maintenance of previous improvement of at least 2 points (or max score of 4) in ability to kick (improvement in at least 2 milestones) **OR**
                b. Improvement or maintenance of previous improvement of at least 1 point increase in motor milestones of head control, rolling, sitting, crawling, standing or walking (consistent with improvement by at least 1 milestone) **AND**
                c. Improvement or maintenance of previous improvement in more HINE motor milestones than worsening
            B. HFMSE:

a. Improvement or maintenance of improvement of at least a 3 point increase in score

C. ULM:

a. Improvement or maintenance of previous improvement of at least 2 point increase in score

D. CHOP-INTEND:

a. Improvement or maintenance of previous improvement of at least 4 point increase in score.

II. Documentation that quantitative spot urine testing, platelet count and coagulation testing has been performed prior to each dose of Spinraza

B. **Zolgensma**

1. Zolgensma is a one-time SMN1 gene replacement therapy. No reauthorization will be considered for Plan coverage.

IV. EXCLUSIONS

A. Spinraza will not be approved for the following:

- Indications that do not have clinical trials to support therapeutic use
- Maintenance dosing greater than 12mg every 4 months
- SMA without Chromosome 5q mutation or deletion

B. Zolgensma will not be approved for the following:

- Indications that do not have clinical trials to support therapeutic use

C. The use of physician samples, or manufacturer product discounts, does not guarantee coverage under the provisions of the medical and/or pharmacy benefit. All pertinent criteria must be met in order to be eligible for benefit coverage.

V. CODES

Note: The following CPT/HCPCS codes are included below for informational purposes. Inclusion or exclusion of a CPT/HCPCS code(s) below does not signify or imply member coverage or provider reimbursement. The member's specific benefit plan determines coverage.

Medication	HCPCS/CPT Code
Spinraza 12 MG/5ML SOLN Injection, nusinersen, 0.1 mg	J2326
Zolgensma Kit Injection, onasemnogene abeparvovec-xioi, per treatment, up to 5x10^15 vector genomes	J3399

Diagnosis Description	ICD-10 Diagnosis Code
Infantile spinal muscular atrophy, type I [Werdnig-Hoffman]	G12.0
Other inherited spinal muscular atrophy	G12.1
Spinal muscular atrophy, unspecified	G12.9

VI. REFERENCES

1. Spinraza [prescribing information]. Cambridge, MA: Biogen Inc; October 2018.
2. Zolgensma [prescribing information]. Bannockburn, IL: AveXis, Inc; May 2019

VII. APPROVALS

Signature on file at JHHC

DATE OF REVISION	SUMMARY OF CHANGE
3/16/2017	Policy Creation
7/19/2017	Modifed clinical criteria regarding covered SMA Type
7/27/2017	Updated Exclusions section regarding physician samples
7/18/2018	Modified clinical criteria regarding covered SMA Type, and reauthorization requirements
07/17/2019	Added clinical criteria for Zolgensma
11/04/2019	Clarified that Spinraza (effective 1/1/2019) and Zolgensma (effective 1/1/2020) will be carved out to the Maryland Medicaid Fee-for Service program
01/01/2021	Clarified that Spinraza and Zolgensma will be carved in to Priority Partners coverage effective 1/1/2021 per MDH mandate
11/10/2021	Removed Priority Partners & EHP as applicable LOBs

Date of Review: 3/16/2017, 7/19/2017, 7/27/2017,7/18/2018, 07/17/2019

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