New Hampshire Medicaid Fee-for-Service Program

Zolgensma® Criteria

Approval Date: July 12, 2022

Pharmacology

Onasemnogene abeparvovec-xioi (Zolgensma®) is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric patients < 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene.

Medications

Brand Names	Generic Names	Dosage Strengths	Dosage Form
Zolgensma®	onasemnogene abeparvovec-xioi	2.0×10^{13} vg/mL each vial contains an extractable volume of not less than either 5.5 mL or 8.3 mL; each kit contains 2 to 9 vials; available as multiple kit sizes based on weight	Intravenous (IV) infusion

Criteria for Approval

1. Patient must be < 2 years of age; **AND**
2. Patient has a diagnosis of spinal muscular atrophy (SMA) confirmed by either bi-allelic deletion or dysfunctional point mutation of the SMN1 gene; **AND**
3. Patient must have SMA confirmed by ≥ 1 of the following:
   a. Patient must have **one or two** copies of the SMN2 gene; **OR**
   b. Patient has **three** copies of the SMN2 gene; **AND**
4. Patient must have a baseline anti-AAV9 antibody titer of ≤ 1:50 measured by ELISA; **AND**
5. Patient does not have pre-existing hepatic impairment as assessed by pre-treatment liver function tests (i.e., total bilirubin, prothrombin time, AST, ALT); **AND**
6. Patient must not have advanced disease (e.g., complete limb paralysis, permanent ventilation support); **AND**
7. Onasemnogene abeparvovec-xioi must be used concomitantly with parenteral corticosteroids (see dosage/administration); **AND**
8. Onasemnogene abeparvovec-xioi must not be used in combination with nusinersen or risdiplam; **AND**

9. Coverage will be provided for one dose and may not be renewed.

Criteria for Denial

1. Prior approval will be denied if the approval criteria are not met.
2. Prior approval will be denied if concurrently on nusinersen or risdiplam.

Quantity Limit: 1 kit

Length of Approval: 1 administration per lifetime

References

Available upon request.

Revision History

Reviewed by	Reason for Review	Date Approved
DUR Board	New	10/28/2019
Commissioner Designee	Approval	12/03/2019
DUR Board	Review	12/15/2020
Commissioner Designee	Approval	02/24/2021
DUR Board	Revision	06/02/2022
Commissioner Designee	Approval	07/12/2022