

HEALTH PLAN POLICY	
Policy Title: Spinal Muscular Atrophy - Gene Therapy	Policy Number: MUM67 Revision: New
Department: Medical Management	Sub-Department: Utilization Management
Applies to Product Lines: <input type="checkbox"/> Medicaid <input checked="" type="checkbox"/> USFHP <input type="checkbox"/> Children's Health Insurance Plan <input checked="" type="checkbox"/> Commercial Insured <input checked="" type="checkbox"/> Health Insurance Exchange <input type="checkbox"/> Non Insured Business <input checked="" type="checkbox"/> Medicare	
Origination/Effective Date: 10/09/2019	
Reviewed Date(s):	Revision Date(s):

SCOPE:

This document addresses gene therapy for SMA. This disease is very rare and often fatal. It is most common in very young children less than 6 months of age. Currently there are 2 very new pharmacologic agents approved by the FDA including Zolgensma in 2019 for SMA Type 1 and Spinraza in 2016 for SMA Type 2.

DEFINITIONS AND ACRONYMS:

- **Spinal Muscular Atrophy (SMA)**
- **Werdnig Hoffman Disease** – Another Name for SMA Type 1

POLICY: Pharmacologic treatment for SMA is medically necessary under the following provisions:

- 1) Genetically confirmed diagnosis of SMA
- 2) Age less than 6 months
- 3) No use of ventilator support equipment exceeding 16 hours per day
- 4) No co morbidities including infection, organ failure of heart, liver or kidneys

Rationale:

SMA is a neuromuscular disease of the spine and brainstem. Muscle weakness and atrophy occur especially in the mouth, throat and respiratory capacity. SMA is the most common genetic cause of childhood death.

The incidence of SMA is 1 in 10,000 births. Average life span is about 7.5 months. The pattern of inheritance is autosomal recessive resulting in the disease's rarity given that each parent must provide a defective gene on their respective Chromosomes #5.

REFERENCES:

- International Consensus Statement -Wang 2007 – Standards of Care for SMA
- Parente 2018 – Statistical Analysis of SMA- Incidence

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- Verhaart 2017- Statistical Analysis of SMA- Incidence
- Farrar 2013 – Statistical Analysis of SMA – Age as a Predictor of Death

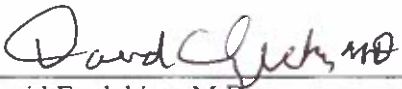
RELATED DOCUMENTS:

None



Nancy Horstmann
Chief Executive Officer Health Plans

10/21/19
Date



David Engleking, M.D.
Medical Director

10/15/19
Date

REVISION HISTORY:

Revision	Date	Description of Change	Committee
New	10/09/2019	Initial release.	Executive Leadership