HIGHMARK HEALTH OPTIONS

Updated: 07/2023
DMMA Approved: 08/2023

Request for Prior Authorization for Spinal Muscular Atrophy Medications
Website Form – www.highmarkhealthoptions.com
Submit request via: Fax - 1-855-476-4158

All requests for Spinal Muscular Atrophy (SMA) Medications require a Prior Authorization and will be screened for medical necessity and appropriateness using the criteria listed below.

Spinal Muscular Atrophy (SMA) Medications Prior Authorization Criteria:

Spinal Muscular Atrophy (SMA) Medications include Spinraza (nusinersen), Zolgensma (onasemnogene abeparvovec-xioi), and Evrysdi (risdiplam). New products with this classification will require the same documentation.

For all requests for Spinal Muscular Atrophy medications, all of the following criteria must be met:

- Diagnosis of Spinal Muscular Atrophy (SMA)
- Prescribed by or in consultation with a neurologist with experience treating SMA
- The requested dose and frequency is in accordance with FDA-approved labeling, nationally recognized compendia, and/or evidence-based practice guidelines.
- Is age-appropriate according to FDA-approved package labeling, nationally recognized compendia, or peer-reviewed medical literature.
- Member is receiving comprehensive treatment based on standards of care for SMA
- Member has documentation of a baseline evaluation, including a standardized assessment of motor function such as one of the following:
    - Hammersmith Functional Motor Scale Expanded (HFMSE)
    - Hammersmith Infant Neurologic Exam (HINE)
    - Upper limb module (ULM) score
    - Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)
    - Six-minute walk test

For Spinraza (nusinersen) all of the following criteria must be met:

- Confirmation of diagnosis by submission of laboratory testing demonstrating corresponding mutations or deletions in chromosome 5q13 that lead to survival motor neuron (SMN) protein deficiency.
- Documentation of genetic testing confirming either two or three copies of SMN2 gene
- Must have ONE of the following:
    - Homozygous deletions of SMN1 gene (e.g., absence of the SMN1 gene)
    - Homozygous mutation in the SMN1 gene (e.g., biallelic mutations of exon 7)
    - Compound heterozygous mutation in the SMN1 gene (e.g., deletion of SMN1 exon 7 (allele 1) and mutation of SMN1 (allele 2)
- Must not be used concomitantly with Evrysdi
- **Initial Duration of Approval:** 4 months
- **Reauthorization criteria**
    - Documentation that the member is responding to the medication as demonstrated by clinically significant improvement or maintenance of function from pretreatment

baseline status using the same exam as performed at baseline assessment (progression, stabilization, or decreased decline in motor function).
- **Reauthorization Duration of Approval:** 12 months

For Evrysdi (risdiplam) all of the following criteria must be met:
- Must have a confirmed diagnosis of 5q-autosomal recessive SMA
- Must not be used concomitantly with Spinraza
- **Initial Duration of Approval:** 12 months
- **Reauthorization criteria**
  - Documentation that the member is responding to the medication as demonstrated by clinically significant improvement or maintenance of function from pretreatment baseline status using the same exam as performed at baseline assessment (progression, stabilization, or decreased decline in motor function).
- **Reauthorization Duration of Approval:** 12 months

For Zolgensma (onasemnogene abeparvovec-xioi) all of the following criteria must be met:
- Must be less than 2 years of age
- If the member was born prematurely, they have reached full-term gestational age
- Confirmed by genetic testing including ALL of the following:
  - Bi-allelic *SMN1* deletions or pathogenic variants
  - Two copies of SMN2 gene
  - Lack of the c.859G>C modification in exon 7 of the SMN2 gene
- Member is not dependent on either of the following:
  - Invasive ventilation or tracheostomy
  - Use of non-invasive ventilation beyond use for naps and nighttime sleep
- Member does not have any of the following clinically significant abnormal lab values:
  - Liver fuction levels (hepatic aminotransferases [AST and ALT] greater than or equal to 2 times the upper limit of normal)
  - Baseline anti-AAV9 antibodies greater than 1:50
  - Platelet count less than 150,000uL
  - Creatinine greater than or equal to 1.8mg/dL
- The prescriber attests that the member's weight for dosing must be confirmed within 14 days of dose administration.
- The member has not been treated with medications for ongoing immunosuppressive therapy within the last three (3) months (e.g. corticosteroids, cyclosporine, tacrolimus, methotrexate, cyclophosphamide, intravenous immunoglobulin, rituximab)
- The prescriber attests that member will receive prophylactic prednisolone (or glucocorticoid equivalent) prior to and approximately 30 days following therapy.
- Member must not have received this therapy previously
- Member is not a participant or recent participant in a SMA treatment clinical trial that may cause risk for gene transfer or treatment with Zolgensma.
- Note: There is a lack of robust clinical evidence to support concomitant use of Zolgensma with other therapies for the treatment of SMA (e.g. Spinraza)
- **Duration of Approval:** Once per lifetime

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Coverage may be provided for any non-FDA labeled indication if it is determined that the use is a medically accepted indication supported by nationally recognized pharmacy compendia or peer-reviewed medical literature for treatment of the diagnosis(es) for which it is prescribed. These requests will be reviewed on a case by case basis to determine medical necessity.

Drugs are authorized in generic form unless the branded product is on the preferred drug list or the prescriber has indicated in writing that the branded product is medically necessary. If only the branded product is on the preferred drug list, the generic form will be considered non-preferred and shall not require the prescriber to indicate in writing that the branded product is medically necessary.

SPINAL MUSCULAR ATROPHY (SMA) MEDICATIONS
PRIOR AUTHORIZATION FORM – PAGE 1 OF 2

Please complete and fax all requested information below including any progress notes, laboratory test results, or chart documentation as applicable to Highmark Health Options Pharmacy Services. **FAX:** (855) 476-4158
If needed, you may call to speak to a Pharmacy Services Representative.
PHONE: (844) 325-6251 Monday through Friday 8:00am to 7:00pm

PROVIDER INFORMATION

Requesting Provider:	NPI:
Provider Specialty:	Office Contact:
Office Address:	Office Phone:
	Office Fax:

MEMBER INFORMATION

Member Name:	DOB:	
Member ID:	Member weight:	Height:

REQUESTED DRUG INFORMATION

Medication:	Strength:	
Directions:	Quantity:	Refills:
Is the member currently receiving requested medication? ☐ Yes ☐ No	Date Medication Initiated:	
Is this medication being used for a chronic or long-term condition for which the medication may be necessary for the life of the patient? ☐ Yes ☐ No		

Billing Information

This medication will be billed: ☐ at a pharmacy **OR** ☐ medically, JCODE:_____
Place of Service: ☐ Hospital ☐ Provider's office ☐ Member's home ☐ Other

Place of Service Information

Name:	NPI:
Address:	Phone:

MEDICAL HISTORY (Complete for ALL requests)

Does the member have a confirmed diagnosis of spinal muscular atrophy (SMA)? ☐ Yes ☐ No ICD10 code:_____
Has the member had a baseline assessment of motor milestones? ☐ Yes ☐ No
Please select all that apply and submit documentation of baseline assessment:

- ☐ Hammersmith Functional Motor Scale Expanded (HFMSE)
- ☐ Hammersmith Infant Neurologic Exam (HINE)
- ☐ Upper limb module (ULM) score
- ☐ Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)
- ☐ Six-minute walk test

For Spinraza:

Has the diagnosis been confirmed by genetic testing? ☐ Yes ☐ No
Please select all that apply to the member:
- ☐ Two or three copies of SMN2 gene
- ☐ Homozygous deletions of SMN1 gene (e.g., absence of the SMN1 gene)
- ☐ Homozygous mutation in the SMN1 gene (e.g., biallelic mutations of exon 7)
- ☐ Compound heterozygous mutation in the SMN1 gene [e.g., deletion of SMN1 exon 7 (allele 1) and mutation of SMN (allele 2)

Will the member be using the medication concomitantly with Evrysdi? ☐ Yes ☐ No

For Evrysdi:
Does member have confirmed diagnosis of 5q-autosomal recessive SMA? ☐ Yes ☐ No
Will member be using Spinraza concomitantly? ☐ Yes ☐ No

*****Continued on next page*****

SPINAL MUSCULAR ATROPHY
PRIOR AUTHORIZATION FORM (CONTINUED) – PAGE 2 OF 2

Please complete and fax all requested information below including any progress notes, laboratory test results, or chart documentation as applicable to Highmark Health Options Pharmacy Services. **FAX:** (855) 476-4158
If needed, you may call to speak to a Pharmacy Services Representative.
PHONE: (844) 325-6251 Monday through Friday 8:00am to 7:00pm

MEMBER INFORMATION

Member Name:	DOB:	
Member ID:	Member weight:	Height:

MEDICAL HISTORY (continued)

For Zolgensma:
If the member was born prematurely, have they reached full-term gestational age? ☐ Yes ☐ No
Has the diagnosis of Spinal Muscular Atrophy (SMA) been confirmed by genetic testing? ☐ Yes ☐ No
Please select all that apply to the member and submit documentation:
- ☐ Bi-allelic *SMN1* deletions or pathogenic variants
- ☐ Two copies of *SMN2* gene
- ☐ Lack of the c.859G>C modification in exon 7 of the SMN2 gene

Is member dependent on either of the following?
- o Invasive ventilation or tracheostomy ☐ Yes ☐ No
- o Use of non-invasive ventilation beyond use for naps and nighttime sleep ☐ Yes ☐ No

Does member have an anti-AAV9 antibody titer below or equal to 1:50 or any clinically significant lab values?
☐ Yes ☐ No
Will the member's weight for dosing be confirmed within 14 days of dose administration? ☐ Yes ☐ No
Has the member been treated with medications for ongoing immunosuppressive therapy within the last three (3) months?
☐ Yes ☐ No

Will the member receive prophylactic prednisolone (or glucocorticoid equivalent) prior to and approximately 30 days following therapy? ☐ Yes ☐ No

Has the member received Zolgensma previously? ☐ Yes ☐ No
Is the member a participant or recent participant in a SMA treatment clinical trial that may cause risk for gene transfer or treatment with Zolgensma? ☐ Yes ☐ No

CURRENT or PREVIOUS THERAPY

Medication Name	Strength/ Frequency	Dates of Therapy	Status (Discontinued & Why/Current)

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REAUTHORIZATION
Is the patient responding to the medication as demonstrated by clinically significant improvement or maintenance of function from pretreatment baseline status using the same exam as performed at baseline assessment (progression, stabilization, or decreased decline in motor function)? ☐ Yes, documentation is provided ☐ No
SUPPORTING INFORMATION or CLINICAL RATIONALE

Prescribing Provider Signature	Date