



Updated: 05/2024  
DMMA Approved: 05/2024

**Request for Prior Authorization for Spinal Muscular Atrophy Medications**  
**Website Form – [www.highmarkhealthoptions.com](http://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 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 and ongoing management of members with 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)
  - Children’s Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)
  - Six-minute walk test (6MWT)
  - If non-ambulatory: Upper Limb Module (ULM), Revised Upper Limb Module (RULM)

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 (risdiplam)
- The member has not previously received gene replacement therapy (e.g., Zolgensma) for the treatment of SMA; OR

- The member has received gene replacement therapy (e.g., Zolgensma) and the member has experienced a decline in clinical/functional status since receipt of gene replacement therapy as demonstrated by a decline in documentation on individual's functional ability score(s).
- **Initial Duration of Approval:** 4 months
- **Reauthorization criteria**
  - Documentation of an annual evaluation by a neurologist with experience treating and ongoing management of members with SMA
  - Documentation demonstrating the member is stable or shows clinically significant improvement in SMA-associated symptoms, as demonstrated by stable or improved functional abilities test results compared to baseline or previous functional abilities test whichever is most recent. The current test and the comparator test being utilized for reauthorization purposes must be at least four (4) months apart (HINE, CHOP-INTEND, HFMSE, 6MWT ULM, RULM, etc.)
  - The member will not be receiving Evrysdi (risdiplam) concomitantly
- **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 (nusinersen)
- The member has not previously received gene replacement therapy (e.g., Zolgensma) for the treatment of SMA; OR
- The member has received gene replacement therapy (e.g., Zolgensma) and the member has experienced a decline in clinical/functional status since receipt of gene replacement therapy as demonstrated by a decline in documentation on individual's functional ability score(s).
- **Initial Duration of Approval:** 12 months
- **Reauthorization criteria**
  - Documentation of an annual evaluation by a neurologist with experience treating and ongoing management of members with SMA
  - Documentation demonstrating the member is stable or shows clinically significant improvement in SMA-associated symptoms, as demonstrated by stable or improved functional abilities test results compared to baseline or previous functional abilities test whichever is most recent. The current test and the comparator test being utilized for reauthorization purposes must be at least four (4) months apart (HINE, CHOP-INTEND, HFMSE, 6MWT ULM, RULM, etc.)
  - Must not be used concomitantly with Spinraza (nusinersen)
- **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 function 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 Zolgensma previously
- If individual is currently on nusinersen (Spinraza) or risdiplam (Evrysdi), the provider attests that further therapy will be discontinued
- 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 (nusinersen) or Evrysdi (risdiplam)]
- **Duration of Approval:** Once per lifetime

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? <input type="checkbox"/> Yes <input type="checkbox"/> 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? <input type="checkbox"/> Yes <input type="checkbox"/> 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: \_\_\_\_\_  
Is the requested SMA medication being prescribed by or in consultation with a neurologist with experience treating and ongoing management of members with SMA?  Yes  No  
Has the member had a baseline assessment of motor function 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)  
 If non-ambulatory: Upper Limb Module (ULM), Revised Upper Limb Module (RULM)  
 Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND)  
 Six-minute walk test (6MWT)

**For Spinraza:**

Has the diagnosis been confirmed by genetic testing demonstrating mutations or deletions in chromosome 5q13?  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 (risdiplam)?  Yes  No  
 Has the member previously received gene replacement therapy (e.g., Zolgensma) for the treatment of SMA or received gene replacement therapy (e.g., Zolgensma) and the member has experienced a decline in clinical/functional status since receipt of gene replacement therapy as demonstrated by a decline in documentation on individual's functional ability score(s)?  Yes  No

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**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 Evrysdi:**  
Does the member have confirmed diagnosis of 5q-autosomal recessive SMA?  Yes  No  
Will member be using Spinraza (nusinersen) concomitantly?  Yes  No  
Has the member previously received gene replacement therapy (e.g., Zolgensma) for the treatment of SMA or received gene replacement therapy (e.g., Zolgensma) and the member has experienced a decline in clinical/functional status since receipt of gene replacement therapy as demonstrated by a decline in documentation on individual’s functional ability score(s)?  Yes  No

**For Zolgensma:**  
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?  
 Invasive ventilation or tracheostomy  Yes  No  
 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?  Yes  No  
 Will the member’s weight for dosing be confirmed within 14 days of dose administration?  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  
 If the member is currently on nusinersen (Spinraza) or risdiplam (Evrysdi), the provider attests that further therapy will be discontinued?  Yes  No  
 Is the member participating or is a recent participant in a SMA 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)

**REAUTHORIZATION**

Is there documentation of an annual evaluation by a neurologist with experience treating and ongoing management of members with SMA?  Yes  No  
 Is there documentation demonstrating the member is stable or shows clinically significant improvement in SMA-associated symptoms, as demonstrated by stable or improved functional abilities test results compared to baseline or previous functional abilities test whichever is most recent. The current test and the comparator test being utilized for reauthorization purposes must be at least four (4) months apart (HINE, CHOP-INTEND, HFMSE, 6MWT ULM, RULM)  Yes, documentation is provided  No

**SUPPORTING INFORMATION or CLINICAL RATIONALE**


<b>Prescribing Provider Signature</b>	<b>Date</b>



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