HIGHMARK WHOLECARE

Updated: 12/2025
PARP Approved: 03/2026

Prior Authorization Criteria

Spinal Muscular Atrophy (SMA) Medications

Disclaimer: 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 include Spinraza (nusinersen), Zolgensma (onasemnogene Abeparvovec-xioi), Evrysdi (risdiplam) and Itvisma (onasemnogene abeparvovec-brve). New products with this classification will require the same documentation.

For all requests for Spinal Muscular Atrophy (SMA) medications, the following criteria must be met in addition to the diagnosis specific criteria below:

- 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.
- 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 that the member is responding to the medication based on the prescriber's assessment.
  - Documentation of an annual evaluation by a neurologist with experience treating and ongoing management of members with SMA
  - 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 that the member is responding and benefitting from the medication based on the prescriber's assessment
- **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 the following:
  - Bi-allelic SMN1 deletions or pathogenic variants
- Member is not dependent on either of the following:
  - invasive ventilation or tracheostomy
- 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)
- 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 is confirmed within 14 days of dose administration.

- The prescriber attests that member will receive prophylactic prednisolone (or glucocorticoid equivalent) prior to and approximately 30 days following therapy
- If individual is currently on nusinersen (Spinraza) or risdiplam (Evrysdi), the provider attests that further therapy will be discontinued
- Member must not have received Zolgensma 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 (nusinersen) or Evrysdi (risdiplam)]
- **Duration of Approval**: Once per lifetime

For Itvisma (onasemnogene abeparvovec-brve) all of the following criteria must be met:

- Confirmed diagnosis of SMA by genetic testing including the following:
  - confirmed mutation in survival motor neuron 1 (SMN1) gene
- Member is not dependent on the following:
  - invasive ventilation
- 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)
- Member does not have any of the following clinically significant abnormal lab values or complications:
  - Liver function levels indicating hepatic dysfunction (i.e. alanine aminotransferase (ALT), total bilirubin, gamma-glutamyl transferase (GGT) or glutamate dehydrogenase (GLDH), > upper limit of normal (ULN))
  - Baseline anti-AAV9 antibodies greater than 1:50
  - Platelet count less than 150,000uL
  - No active infection
  - Creatinine greater than or equal to 1.8mg/dL

The prescriber attests that the member's weight for dosing is confirmed within 14 days of dose administration.

- The prescriber attests that member will receive prophylactic prednisolone (or glucocorticoid equivalent) prior to and approximately 30 days following therapy
- Vaccination status must be up-to-date prior to administration
- If individual is currently on nusinersen (Spinraza) or risdiplam (Evrysdi), the provider attests that further therapy will be discontinued
- Members previously treated with Zolgensma (onasemnogene abeparvovec-xioi) are not to be treated with Itvisma
- Itvisma is to be administered intrathecally using a lumbar puncture by a healthcare professional (e.g., interventional radiologist or neurologist) experienced in performing lumbar punctures.
- 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 or Itvisma.

- Note: There is a lack of robust clinical evidence to support concomitant use of Itvisma with other therapies for the treatment of SMA [e.g. Spinraza (nusinersen), Evrysdi (risdiplam) or Zolgensma]
- **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.

When criteria are not met, the request will be forwarded to a Medical Director for review. The physician reviewer must override criteria when, in their professional judgment, the requested medication is medically necessary.

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SPINAL MUSCULAR ATROPHY (SMA) MEDICATIONS
PRIOR AUTHORIZATION FORM

Please complete and fax all requested information below including any progress notes, laboratory test results, or chart documentation as applicable to Highmark Wholecare Pharmacy Services. **FAX:** (888) 245-2049
If needed, you may call to speak to a Pharmacy Services Representative. **PHONE**: (800) 392-1147 Mon – Fri 8:30am to 5:00pm

PROVIDER INFORMATION

Requesting Provider:	Provider NPI:
Provider Specialty:	Office Contact:
State license #:	Office NPI:
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:	

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 member receiving comprehensive treatment based on standards of care for SMA? ☐ Yes ☐ No
Has the member had a baseline assessment of motor function? ☐ 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

If non-ambulatory:
☐ Upper Limb Module (ULM)
☐ Revised Upper Limb Module (RULM)

For Spinraza:
Has the diagnosis been confirmed by genetic testing demonstrating mutations or deletions in chromosome 5q13? ☐ Yes ☐ No
Does the member have one of the following: Homozygous deletions of SMN1 gene (e.g., absence of the SMN1 gene), OR Homozygous mutation in the SMN1 gene (e.g., biallelic mutations of exon 7), OR Compound heterozygous mutation in the SMN1 gene (e.g., deletion of SMN1 exon 7 (allele 1) and mutation of SMN1 (allele 2)? ☐ Yes ☐ No
Will the member be using the medication concomitantly with Evrysdi? ☐ Yes ☐ No
Has the member previously received gene replacement therapy (e.g., Zolgensma) for the treatment of SMA? ☐ Yes ☐ No
Has the member 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 Evrysdi:
Is there a confirmed diagnosis of 5q-autosomal recessive SMA? ☐ Yes ☐ No
Will the member be using the medication concomitantly with Spinraza? ☐ Yes ☐ No
Has the member previously received gene replacement therapy (e.g., Zolgensma) for the treatment of SMA? ☐ Yes ☐ No

Has the member 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:

If the member was born prematurely, have they reached full-term gestational age? ☐ Yes ☐ No
Is there documentation of a gene mutation analysis including bi-allelic SMN1 mutations? ☐ Yes ☐ No
Is member dependent on invasive ventilation or tracheostomy? ☐ Yes ☐ No
Has the member been treated with medications for ongoing immunosuppressive therapy with the last 3 months? ☐ Yes ☐ No
Does the member have any clinically significant abnormal lab values (e.g. platelets less than 150,000 uL, anti-AAV9 antibodies greater than 1:50, etc)? ☐ 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
Is the member currently on nusinersen (Spinraza) or risdiplam (Evrysdi) with a plan to discontinue these therapies? ☐ Yes ☐ No
Has the member previously received Zolgensma? ☐ 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

SPINAL MUSCULAR ATROPHY (SMA) MEDICATIONS 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 Wholecare Pharmacy Services. **FAX:** (888) 245-2049
If needed, you may call to speak to a Pharmacy Services Representative. **PHONE**: (800) 392-1147 Mon – Fri 8:30am to 5:00pm

MEMBER INFORMATION

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

MEDICAL HISTORY (CONTINUED)

For Itvisma:

Does the member have a confirmed diagnosis of SMA by genetic testing including confirmed mutation in survival motor neuron 1 (SMN1) gene? ☐ Yes ☐ No
Is the member dependent on invasive ventilation? ☐
Has the member been treated with medications for ongoing immunosuppressive therapy within the last 3 months (e.g.corticosteroids, cyclosporine, tacrolimus, methotrexate, cyclophosphamide, intravenous immunoglobulin, rituximab)? ☐ Yes ☐ No
Does the member have any of the following clinically significant abnormal lab values or complications? ☐ Yes, select all that apply
☐ No
☐ Liver function levels indicating hepatic dysfunction (i.e. alanine aminotransferase (ALT), total bilirubin, gamma-glutamyl transferase (GGT) or glutamate dehydrogenase (GLDH), > upper limit of normal (ULN))
☐ Baseline anti-AAV9 antibodies greater than 1:50
☐ Platelet count less than 150,000uL
☐ No active infection
☐ Creatinine greater than or equal to 1.8mg/dL
Will the prescriber ensure the member's weight for dosing is confirmed within 14 days of dose administration? ☐ Yes ☐ No
Will the prescriber ensure the member will receive prophylactic prednisolone (or glucocorticoid equivalent) prior to and approximately 30 days following therapy? ☐ Yes ☐ No
Is the member's vaccination status up to date? ☐ Yes ☐ No
If the member is currently on nusinersen (Spinraza) or risdiplam (Evrysdi), is there a plan by the provider to discontinue those therapies before receiving the requested agent? ☐ Yes ☐ No
Has the member previously been treated with Zolgensma (onasemnogene abeparvovec-xioi)? ☐ 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 or Itvisma? ☐ Yes ☐ No

CURRENT or PREVIOUS THERAPY

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

REAUTHORIZATION

Spinraza (nusinersen) and Evrysdi (risdiplam) Only: Is the member responding to the medication (i.e. clinically significant improvement or maintenance of function from pretreatment baseline status using the same exam as performed at baseline assessment)? ☐ Yes ☐ No

Has documentation of an annual evaluation, including a standardized assessment of motor function, by a neurologist with experience treating SMA been completed? ☐ Yes ☐ No

Is the member receiving Evrysdi concomitantly with Spinraza? ☐ Yes ☐ No

SUPPORTING INFORMATION or CLINICAL RATIONALE

Prescribing Provider Signature	Date