HIGHMARK HEALTH OPTIONS

Updated: 12/2025
DMMA Approved: 01/2026

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 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 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 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
    - Copies of SMN2 gene present
    - 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 advanced SMA including, but not limited to, complete paralysis of the limbs
- 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 member does not have an active viral or bacterial infection (including Hepatitis B, Hepatitis C, HIV, Zika virus, gastroenteritis, otitis media, bronchiolitis, etc.).
- The member does not have any concomitant illness(es) that may create unnecessary risks for gene replacement therapy such as:
  - Major renal or hepatic impairment
  - Known seizure disorder
  - Diabetes mellitus
  - Idiopathic hypocalcuria
  - Symptomatic cardiomyopathy
- 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

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

- Confirmed diagnosis of Type 2 SMA by genetic testing including the following:
  - confirmed mutation in survival motor neuron 1 (SMN1) gene
  - Member is able to sit but never able to walk independently (never ambulatory)
- Member is not dependent on either of the following:
  - invasive ventilation, awake noninvasive ventilation for greater than 6 hours during a 24-hour period, noninvasive ventilation for greater than 12 hours during a 24-hour period or tracheostomy

- Onset of clinical signs and symptoms at ≥ 6 months of age
- 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
    - Complications that would interfere with motor assessments including but not limited to, severe contractures or Cobb angle > 40 in a sitting position
    - Surgery for scoliosis or hip fixation in the 12 months prior to procedure or planned within the next 64 weeks
    - Clinically significant sensory abnormalities from a neurological examination
- 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.

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.

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SPINAL MUSCULAR ATROPHY (SMA) MEDICATIONS
PRIOR AUTHORIZATION FORM – PAGE 1 OF 3

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:_____

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:

- ☐ Copies of SMN2 gene present
- ☐ 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 3

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
- ☐ Copies of SMN2 gene present
- ☐ 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 the member have complete paralysis of the limbs? ☐ Yes ☐ No

Has the member been treated with medications for ongoing immunosuppressive therapy within the last three (3) months (e.g. corticosteroids, cyclosporine, tacrolimus, methotrexate, cyclophosphamide, intravenous immunoglobulin, rituximab)? ☐ Yes ☐ No

Does the member have an active viral or bacterial infection (including Hepatitis B, Hepatitis C, HIV, Zika virus, gastroenteritis, otitis media, bronchiolitis, etc.)? ☐ Yes ☐ No

Does the member have a concomitant illness(es) that may create unnecessary risks for gene replacement therapy such as major renal or hepatic impairment, known seizure disorder, diabetes mellitus, idiopathic hypocalcuria or symptomatic cardiomyopathy? ☐ 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

For Itvisma:

Has the diagnosis of Type 2 Spinal Muscular Atrophy (SMA) been confirmed by genetic testing? ☐ Yes ☐ No
Please select all that apply to the member and submit documentation:
- ☐ confirmed mutation in survival motor neuron 1 (SMN1) gene
- ☐ member is able to sit but never ambulatory

Is the 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

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SPINAL MUSCULAR ATROPHY
PRIOR AUTHORIZATION FORM (CONTINUED) – PAGE 3 OF 3

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)

At what age was the member exhibiting onset of clinical signs and symptoms? _____
Does the member have an active viral or bacterial infection (including Hepatitis B, Hepatitis C, HIV, Zika virus, gastroenteritis, otitis media, bronchiolitis, etc.)? ☐ Yes ☐ No
Is the members vaccination status up to date? ☐ Yes ☐ No
Is the member being treated with medications for ongoing immunosuppressive therapy within the last three (3) months? ☐ Yes ☐ No
Does the member have a concomitant illness(es) that may create unnecessary risks for gene replacement therapy? ☐ Yes ☐ No
Does member have an anti-AAV9 antibody titer below or equal to 1:50? ☐ Yes ☐ No
Does the member have a platelet count less than 150,000uL? ☐ Yes ☐ No
Does the member have any of the following (please mark which are applicable):
☐ Complications that would interfere with motor assessments including but not limited to, severe contractures or Cobb angle > 40 in a sitting position
☐ Surgery for scoliosis or hip fixation in the 12 months prior to procedure or planned within the next 64 weeks
☐ Clinically significant sensory abnormalities from a neurological examination
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 or Itvisma? ☐ Yes ☐ No
Will Itvisma be administered by a healthcare professional experienced in performing lumbar punctures? ☐ 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

Prescribing Provider Signature	Date