HIGHMARK HEALTH OPTIONS

Updated: 12/2025
Approved: 01/2026

Request for Prior Authorization for Zolgensma (onasemnogene Abeparvovec-xioi) and Itvisma (onasemnogene abeparvovec-brve)
Website Form – www.wv.highmarkhealthoptions.com
Submit request via: Fax - 1-833-547-2030.

All requests for Zolgensma (onasemnogene Abeparvovec-xioi) and Itvisma (onasemnogene abeparvovec-brve) require a prior authorization and will be screened for medical necessity and appropriateness using the criteria listed below.

Zolgensma (onasemnogene Abeparvovec-xioi) and Itvisma (onasemnogene abeparvovec-brve) Prior Authorization Criteria:

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
- Prescribed by or in consultation with a neurologist with experience treating SMA or a neuromuscular specialist in the treatment of SMA
- 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 fuction levels (hepatic aminotransferases [AST and ALT] greater than or equal to 2 times the upper limit of normal) or has pre-existing hepatic insufficiency
  - 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 member does not have an active viral infection
- The member does not have advanced SMA (such as complete paralysis of limbs or permanent ventilator dependence*)
- The requested dose and frequency is in accordance with FDA-approved labeling, nationally recognized compendia, and/or evidence-based practice guidelines
- 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

- 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.
- The requested medication will not be used in combination with nusinersen (Spinraza)
- **Duration of Approval**: Once per lifetime

*Permanent ventilator dependence is defined as requiring invasive ventilation (tracheostomy), or respiratory assistance for 16 or more hours per day (including noninvasive ventilatory support) continuously for 14 or more days in the absence of an acute reversible illness, excluding perioperative ventilation.

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
- Member must be 2 years of age or older
- 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.

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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ZOLGENSMA (ONASEMNOGENE ABEPARVOVEC-XIOI) AND ITVISMA (ONASEMNOGENE ABEPARVOVEC-BRVE)
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: (833)-547-2030.**
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)

For Zolgensma: Does the member have a confirmed diagnosis of spinal muscular atrophy (SMA) confirmed by genetic testing? ☐ Yes ☐ No ICD10 code: _____

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

If the member was born prematurely, have they reached full-term gestational age? ☐ Yes ☐
Is the member receiving comprehensive treatment based on standards of care for SMA? ☐ Yes ☐ No

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

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 (e.g. corticosteroids, cyclosporine, tacrolimus, methotrexate, cyclophosphamide, intravenous immunoglobulin, rituximab)? ☐ Yes ☐ No
Does the member have an active viral infection? ☐ Yes ☐ No
Does the member have advanced SMA (such as complete paralysis of limbs or permanent ventilator dependence*)? ☐ Yes ☐ No
Will the requested medication be used in combination with nusinersen (Spinraza)? ☐ Yes ☐ No
Has the member received Zolgensma previously? ☐ 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
Is the requested SMA medication being prescribed by or in consultation with a neurologist with experience treating SMA or a neuromuscular specialist in the treatment of SMA? ☐ Yes ☐ No

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ZOLGENSMA (ONASEMNOGENE ABEPARVOVEC-XIOI) AND ITVISMA (ONASEMNOGENE ABEPARVOVEC-BRVE)
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: (833)-547-2030.**
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)

Does the member have any of the following clinically significant abnormal lab values? Please select all that apply:
☐ Liver fuction levels (hepatic aminotransferases [AST and ALT] greater than or equal to 2 times the upper limit of normal) or has pre-existing hepatic insufficiency
☐ Baseline anti-AAV9 antibodies greater than 1:50
☐ Platelet count less than 150,000uL
☐ Creatinine greater than or equal to 1.8mg/dL
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 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

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

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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)**

SUPPORTING INFORMATION or CLINICAL RATIONALE

Prescribing Provider Signature	Date