HIGHMARK HEALTH OPTIONS

Updated: 07/2024
Approved: 08/2024

Request for Prior Authorization for Gene Therapy Agents
Website Form – www.highmarkhealthoptions.com
Submit request via: Fax - 1-833-547-2030

All requests for Gene Therapy Agents without their own policy require a prior authorization and will be screened for medical necessity and appropriateness using the criteria listed below.

Gene Therapy Agents Prior Authorization Criteria:

Gene therapies include betibeglogene autotemcel (Zynteglo), eladocagene exuparvovec (effective upon FDA approval), elivaldogene tavalentivec (Lenti-DTM) (effective upon FDA approval), etranacogene dezaparvovec (Hemgenix), valoctocogene roxaparvovec (Roctavian) and delandistrogene moxeparvovec-rokl (Elevidys). New products with this classification will require the same documentation.

For all requests the following criteria must be met in addition to the diagnosis specific criteria below:

- Is prescribed for an indication that is included in the U.S. Food and Drug Administration (FDA)-approved package labeling OR a medically accepted indication
- The requested dose and frequency is in accordance with FDA-approved labeling, nationally recognized compendia, and/or evidence-based practice guidelines
- Must be age-appropriate according to FDA-approved labeling, nationally recognized compendia, or evidence-based practice guidelines

For Hemgenix (etranacogene dezaparvovec) requests:

Coverage may be provided with a diagnosis of Hemophilia B (congenital Factor IX deficiency) and the following criteria is met:

- Member must have severe or moderately severe hemophilia B (congenital factor IX deficiency) defined as equal to or less than 2% of normal circulating factor IX confirmed by blood coagulation testing
- Must have baseline liver function tests assessed prior to and after therapy for at least three months and be within normal range
- Members with preexisting risk factors for hepatocellular carcinoma (e.g., members with cirrhosis, advanced hepatic fibrosis, hepatitis C or B, non-alcoholic fatty liver disease (NAFLD), chronic alcohol consumption, non-alcoholic steatohepatitis (NASH), and advanced age) must have abdominal ultrasound screenings and be monitored regularly (e.g., annually) for alpha-fetoprotein (AFP) elevations following administration
- Is prescribed by a hematologist or hemophilia treatment center practitioner
- Member has received IX prophylactic or on-demand replacement therapy for ≥ 150 accumulated days and is currently using factor IX prophylaxis therapy
- Member has ≥ 12 bleeding episodes if receiving on-demand therapy over the preceding 12 months. Does **not** apply to patients on prophylaxis.
- Member must have a baseline anti-AAV5 antibody titer of ≤ 1:678 measured by ELISA
- Member must not have any of the following:
    - Inhibitor antibodies to factor IX
    - A positive HIV test during time of screening that is not controlled with anti-viral therapy

  - Active infection with hepatitis B or C virus at screening
  - History of hepatitis B or C exposures, currently controlled by antiviral therapy
  - Prior hemophilia AAV-vector based gene therapy
- **Duration of Approval:** One lifetime dose

For Roctavian (valoctocogene roxaparvovec) requests:

Coverage may be provided with a diagnosis of Hemophilia A (congenital Factor VIII deficiency) and the following criteria is met:

- Member must have severe hemophilia A (congenital factor VIII deficiency) defined as less than 1% of normal circulating factor VIII confirmed by blood coagulation testing
- Member must not have any pre-existing antibodies to adeno-associated virus serotype 5 detected by an FDA approved test.
- Member must not have any contraindications to receiving therapy such as active infections (either acute or uncontrolled chronic), significant hepatic fibrosis (stage 3 or 4) or cirrhosis or a known hypersensitivity to mannitol.
- Member meets both of the following:
  - No previous documented history of a detectable FVIII inhibitor
  - Member has inhibitor level assay < 1 Bethesda units (BU) on 2 consecutive occasions at least one week apart within the last 12 months
- Must have baseline liver function tests assessed prior to and after therapy for at least three months and be within normal range
- Members with preexisting risk factors for hepatocellular carcinoma (e.g., members with cirrhosis, advanced hepatic fibrosis, hepatitis C or B, non-alcoholic fatty liver disease (NAFLD), chronic alcohol consumption, non-alcoholic steatohepatitis (NASH), and advanced age) must have abdominal ultrasound screenings and be monitored regularly (e.g., annually) for alpha-fetoprotein (AFP) elevations following administration
- Is prescribed by a hematologist or hemophilia treatment center practitioner
- Member has received VIII prophylactic or on-demand replacement therapy for ≥ 150 accumulated days
- Member has ≥ 12 bleeding episodes if receiving on-demand therapy over the preceding 12 months. Does not apply to patients on prophylaxis.
- Member must not have any of the following:
  - A positive HIV test during time of screening that is not controlled with anti-viral therapy
  - Active infection with hepatitis B or C virus at screening
  - History of chronic or active hepatitis B or active hepatitis C or currently controlled by antiviral therapy
  - Prior hemophilia AAV-vector based gene therapy

For Elevidys (**delandistrogene moxeparvovec-rokl) requests**:

Coverage may be provided with a diagnosis of Duchenne muscular dystrophy (DMD) and the following criteria is met:

- The member must be ambulatory and age 4 through 5 years of age
- A confirmed diagnosis of DMD by submission of lab testing demonstrating mutation of the dystrophin (DMD) gene by either:

  - A confirmed frameshift mutation OR
  - A premature stop codon mutation between exons 18 to 58 in the DMD gene
- The member must not have any deletion in exon 8 and/or exon 9 in the DMD gene
- The member must be on a stable dose of corticosteroids for DMD for at least 12 weeks prior to therapy unless contraindicated
- The member must have a baseline anti-AAVrh74 antibody titers <1:400 measured by ELISA
- Must be prescribed by or in consultation with a neurologist who has experience in the treatment and management of DMD
- There is documentation of a baseline evaluation, including a standardized assessment of motor function, by a neurologist with experience treating DMD
- **Duration of Approval:** One lifetime dose

For Zynteglo (betibeglogene autotemcel) requests:

Coverage may be provided with a diagnosis of beta-thalassemia and the following criteria is met:

- The member must be transfusion-dependent β-thalassaemia (TDT) who do not have a β0 /β0 genotype, for whom haematopoietic stem cell (HSC) transplantation is appropriate but a human leukocyte antigen (HLA)-matched related HSC donor is not available
- Members are considered to be transfusion-dependent if they had a history of transfusions of at least 100 mL/kg/year of RBCs or with ≥8 transfusions of RBCs per year in the 2 years preceding enrolment.
- Is prescribed by a hematologist, stem cell transplantation specialist or in the treatment of members with TDT
- Must be administered in a qualified treatment center
- Physician must confirm that HSC transplantation is appropriate for the member before myeloablative conditioning is initiated
- Does not have a history of a contraindication to the requested medication
- Member must not have had previous treatment with HSC gene therapy
- Member must not be pregnant or breast-feeding
- All members should be tested for HIV prior to mobilization and apheresis to ensure acceptance of the apheresis material for manufacturing
- **Duration of Approval:** One lifetime dose

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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GENE THERAPY AGENTS
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. **1-833-547-2030.**
If needed, you may call to speak to a Pharmacy Services Representative. **PHONE**: (844) 325-6251 Mon – Fri 8:00 am to 7:00 pm

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)

Diagnosis:	ICD Code:

Hemophilia A:

Does the member have severe hemophilia A? ☐ Yes, normal factor activity level:_____ ☐ No
Does the member have any pre-existing antibodies to adeno-associated virus serotype 5 detected by an FDA approved test? ☐ Yes ☐ No
Does the member have any contraindications to receiving therapy? ☐ Yes ☐ No
Did the member have baseline liver function tests assessed prior to therapy and was it within normal range? ☐ Yes ☐ No
Will the member have liver function testing done for at least 3 months after therapy? ☐ Yes ☐ No
Did the member have abdominal ultrasound screenings if they have preexisting risk factors for hepatocellular carcinoma? ☐ Yes ☐ No
Has the member had any documented history of a detectable FVIII inhibitor or an inhibitor level assay <1 BU on 2 consecutive occasions at least one week apart with the last 12 months? ☐ Yes, please explain below. ☐ No
Has the member had ≥ 12 bleeding episodes if receiving on-demand therapy over the preceding 12 months? Does **not** apply to patients on prophylaxis. ☐ Yes ☐ No
Has the member received FVIII prophylactic or on-demand replacement therapy for ≥ 150 accumulated days and still on current therapy? ☐ Yes ☐ No
Does the member have a positive HIV test or active infection with Hepatitis B or C? ☐ Yes ☐ No
Has the member had prior hemophilia AAV-vector based gene therapy? ☐ Yes ☐ No

Hemophilia B:

Does the member have severe or moderately severe B? ☐ Yes, normal factor activity level:_____ ☐ No
Did the member have baseline liver function tests assessed prior to therapy and was it within normal range? ☐ Yes ☐ No
Will the member have liver function testing done for at least 3 months after therapy? ☐ Yes ☐ No
Did the member have abdominal ultrasound screenings if they have preexisting risk factors for hepatocellular carcinoma? ☐ Yes ☐ No

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GENE THERAPY AGENTS
PRIOR AUTHORIZATION FORM (CONTINUED) – PAGE 2 OF 3

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MEMBER INFORMATION

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

MEDICAL HISTORY (Complete for ALL requests)

Diagnosis:	ICD Code:

Hemophilia B (continued):

Has the member had ≥ 12 bleeding episodes if receiving on-demand therapy over the preceding 12 months? Does **not** apply to patients on prophylaxis. ☐ Yes ☐ No

Has the member received IX prophylactic or on-demand replacement therapy for ≥ 150 accumulated days and still on current therapy? ☐ Yes ☐ No

What is the members baseline anti-AAV5 antibody titer measured by ELISA? _____

Does the member have inhibitor antibodies to factor IX? ☐ Yes ☐ No

Does the member have a positive HIV test or active infection with Hepatitis B or C? ☐ Yes ☐ No

Has the member had prior hemophilia AAV-vector based gene therapy? ☐ Yes ☐ No

DMD:

Does the member have a diagnosis of DMD confirmed by submission of lab testing demonstrating mutation of the dystrophin (DMD) gene by either a confirmed frameshift mutation OR a premature stop codon mutation between exons 18 to 58 in the DMD gene? ☐ Yes ☐ No

Is the member ambulatory? ☐ Yes ☐ No

Does the member have any deletion in exon 8 and/or exon 9 in the DMD gene? ☐ Yes ☐ No

Is the member on a stable dose of corticosteroids for DMD for at least 12 weeks prior to therapy? ☐ Yes ☐ No

What is the member's baseline anti-AAVrh74 antibody titers level measured by ELISA? _____

Is there documentation of a baseline evaluation including a standardized assessment of motor function done by a neurologist with experience in treating DMD? ☐ Yes ☐ No

Beta-Thalassemia:

Is the member transfusion-dependent β-thalassaemia (TDT) who does not have a β0 /β0 genotype, for whom haematopoietic stem cell (HSC) transplantation is appropriate but a human leukocyte antigen (HLA)-matched related HSC donor is not available? ☐ Yes ☐ No

Is the member considered transfusion-dependent? ☐ Yes ☐ No

Does the member have a history of at least 100 mL/kg/year or ≥10 units/year of packed RBC transfusions in the prior 2 years? ☐ Yes ☐ No

Is the medication being administered in a qualified treatment center? ☐ Yes ☐ No

Has the physician confirmed that HSC transplantation is appropriate for the member before myeloablative conditioning is initiated? ☐ Yes ☐ No

Does the member have any contraindications to requested therapy? ☐ Yes ☐ No

Has the member had previous treatment with HSC gene therapy? ☐ Yes ☐ No

Is the member pregnant or breast-feeding? ☐ Yes ☐ No

Has the member been tested for HIV prior to mobilization and apheresis to ensure acceptance of the apheresis material for manufacturing? ☐ Yes ☐ No

Has the member been a recipient of an allogenic transplant or gene therapy previously? ☐ Yes ☐ No

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GENE THERAPY AGENTS
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MEMBER INFORMATION

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

CURRENT or PREVIOUS THERAPY

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

SUPPORTING INFORMATION or CLINICAL RATIONALE

Prescribing Provider Signature	Date