

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 Kebilidi (eladocogene exuparvovec, Zynteglo (betibeglogene autotemcel), Skysona (elivaldogene autotemcel), Hemgenix (etranacogene dezaparvovec), Roctavian (valoctocogene roxaparvovec), Elevidys (delandistrogene moxeparvovec-rokl), Lenmeldy (atidarsagene autotemcel), Casgevy (exagamglogene autotemcel) and Lyfgenia (Lovotibeglogene autotemcel). 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
- The member is not currently enrolled in a clinical trial for the requested drug or has previously received the requested gene therapy or any other gene therapy.
- Does not have any contraindications to the requested medication

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 $\leq 1:678$ 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 and is currently using factor VIII 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 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 hepatitis B or C exposures, currently controlled by antiviral therapy
 - Prior hemophilia AAV-vector based gene therapy
- **Duration of Approval:** One lifetime dose

For Elevidys (delandistrogene moxeparvovec-rokl) requests:

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

- 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
- Member meets either of the following criteria:
 - Member is ambulatory (e.g., able to walk with or without assistance, not wheelchair dependent).
 - Member is non-ambulatory and has a Performance Upper Limb (PUL) entry item score of at least 3 and a total PUL score of 20 – 40.
- The member must not have any deletion in exon 8 and/or exon 9 in the DMD gene
- Member does not have signs of cardiomyopathy (e.g., ejection fraction < 40%)
- Member does not currently have an active infection
- 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
- Elevidys will not be used in combination with exon-skipping therapies (e.g., casimersen, eteplirsen, golodirsen, viltolarsen).
- There is documentation of a baseline evaluation, including a standardized assessment of motor function, by a neurologist with experience treating DMD and liver function, platelet count, and troponin-I levels have been assessed at baseline and will be monitored as clinically appropriate.
- **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 β^0/β^0 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

- Member must not have had previous treatment with HSC gene therapy
- Member must not be pregnant or breast-feeding
- All patients 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

For Skysona (elivaldogene autotemcel) requests:

Coverage may be provided with a diagnosis of **cerebral adrenoleukodystrophy (CALD)** and the following criteria is met:

- Must have early, active CALD defined by:
 - Elevated very long chain fatty acids (VLCFA) values
 - Active CNS disease established by central radiographic review of brain magnetic resonance imaging (MRI)
 - Loes score between 0.5 and 9
 - Gadolinium enhancement (GdE+) on MRI of demyelinating lesions
 - Neurologic function score (NFS) of ≤ 1 demonstrating asymptomatic or mild disease
- Member must have confirmed mutations in the ABCD1 gene
- Must be prescribed by a neurologist or ALD specialist.
- The requested dose and frequency is in accordance with FDA-approved labeling, nationally recognized compendia, and/or evidence-based practice guidelines
- Skysona should not be administered in members with active infections.
- Member must have a negative serology test for HIV.
- Member must not have been a recipient of an allogenic transplant or gene therapy
- **Duration of Approval:** One lifetime dose

For Casgevy (exagamglogene autotemcel) requests:

Coverage may be provided with a diagnosis of **severe sickle cell disease (SCD)** and the following criteria is met:

- Diagnosis is confirmed by electrophoresis demonstrating the presence of sickle cell disease (HbSS, HbSC, HbS β^0 -thalassemia, or HbS β^+ -thalassemia).
- Member must be eligible for a hematopoietic stem cell transplantation and a human leukocyte antigen matched related hematopoietic stem cell donor is not available
- Must have a history of at least 2 severe vaso-occlusive crisis (VOC) events during each of the prior 2 years. Severe VOC defined as :
 - Acute pain event requiring a visit to a medical facility and administration of pain medications (opioids or intravenous [IV] non-steroidal anti-inflammatory drugs [NSAIDs]) or RBC transfusions
 - Acute chest syndrome
 - Priapism lasting > 2 hours and requiring a visit to a medical facility
 - Splenic sequestration

- Must be prescribed by or in consultation with a hematologist/oncologist or sickle cell disease specialist.
- Member must not have:
 - Advanced liver disease
 - History of untreated Moyamoya disease or presence of Moyamoya disease
- For members who are 12-18 years of age, members must have normal transcranial Doppler (TCD)
- Members who are currently on disease modifying therapies for SCD (e.g., hydroxyurea, crizanlizumab, voxelotor) must discontinue them 8 weeks before the planned start of mobilization and conditioning.
- Member must not have clinically significant and active bacterial, viral, fungal or parasitic infection.
- Member must not have been a recipient of an allogenic transplant or gene therapy
- **Duration of Approval:** One lifetime dose

For Casgevy (exagamglogene autotemcel) requests:

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

- Transfusion-dependent β -thalassemia (TDT) as defined by:
 - Documented homozygous β -thalassemia or compound heterozygous β -thalassemia including β -thalassemia/hemoglobin E (HbE)
 - History of at least 100 mL/kg/year or ≥ 10 units/year of packed RBC transfusions in the prior 2 years
- Member must be eligible for autologous stem cell transplant
- 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
- Member must not have any of the following:
 - Severely elevated iron in the heart (i.e. cardiac T2* less than 10 msec by magnetic resonance imaging [MRI] or left ventricular ejection fraction [LVEF] < 45% by echocardiogram)
 - Advanced liver disease (aspartate transaminase [AST] or alanine transaminase [ALT] > 3 \times the upper limit of normal [ULN], or direct bilirubin value > 2.5 \times ULN, or if a liver biopsy demonstrated bridging fibrosis or cirrhosis [liver biopsy was performed if liver iron content was ≥ 15 mg/g by MRI])
 - An available 10/10 human leukocyte antigen matched related hematopoietic stem cell donor
 - Associated α -thalassemia and >1 alpha deletion or alpha multiplications
 - Sickle cell beta thalassemia variant
 - Clinically significant and active bacterial, viral, fungal, or parasitic infection
 - White blood cell (WBC) count < 3 $\times 10^9$ /L or platelet count < 50 $\times 10^9$ /L not related to hypersplenism
- Member must not have had previous treatment with a hematopoietic stem cell (HSC) gene therapy or prior allo-HSCT
- Member must not be pregnant or breast-feeding

- All members should be tested for HIV-1, HIV-2, HBV, HCV prior to mobilization and apheresis to ensure acceptance of the apheresis material for manufacturing
- **Duration of Approval:** One lifetime dose

For Lyfgenia (lovotibeglogene autotemcel) requests:

Coverage may be provided with a diagnosis of **severe sickle cell disease (SCD)** and the following criteria is met:

- Diagnosis is confirmed by electrophoresis demonstrating the presence of sickle cell disease with either $\beta S/\beta S$ or $\beta S/\beta 0$ or $\beta S/\beta +$ genotype
- Member must be eligible for a hematopoietic stem cell transplantation and a human leukocyte antigen matched related hematopoietic stem cell donor is not available
- Must have a history of at least 4 severe vaso-occlusive event (VOE) in the past 24 months. A severe VOE is defined as:
 - an event with no medically determined cause other than a vaso-occlusion, requiring a ≥ 24 -hour hospital or Emergency Room (ER) observation unit visit or at least 2 visits to a day unit or ER over 72 hours with both visits requiring intravenous treatment. Exception: priapism does not require hospital admission but does require a medical facility visit; 4 priapism episodes that require a visit to a medical facility (without inpatient admission) are sufficient to meet criterion.
- Must be prescribed by or in consultation with a hematologist/oncologist or sickle cell disease specialist.
- Member must have a Karnofsky performance status of ≥ 60 (≥ 16 years of age) or a Lansky performance status of ≥ 60 (< 16 years of age).
- The member has either experienced hydroxyurea (HU) failure at any point in the past or must have intolerance to HU (defined as patient being unable to continue to take HU)
- Member must not have:
 - Advanced liver disease
 - History of untreated Moyamoya disease or presence of Moyamoya disease
- For members who are 12-18 years of age, members must have normal transcranial Doppler (TCD)
- Member must not need curative anticoagulation therapy during the period of conditioning through platelet engraftment
- Member must be able to receive a red blood cell transfusion
- Member must have a negative serology test for HIV.
- Member must not have clinically significant and active bacterial, viral, fungal or parasitic infection
- Member must not have been a recipient of an allogenic transplant or gene therapy
- **Duration of Approval:** One treatment per lifetime

For Lennmeldy (atidarsagene autotemcel) requests:

Coverage may be provided with a diagnosis of **metachromatic leukodystrophy (MLD)** and the following criteria is met:

- Member must have one of the following:
 - Pre-symptomatic late infantile (PSLI) MLD
 - Pre-symptomatic early juvenile (PSEJ) MLD
 - Early symptomatic early juvenile (ESEJ) MLD
- A confirmed diagnosis by **all** of the following:
 - Biochemical testing documenting human arylsulfatase A (ARSA) gene activity is below the normal range for the laboratory performing the test
 - The presence of two disease-causing ARSA alleles, either known or novel mutations, identified on genetic testing
 - If novel mutations are identified, a 24-hour urine collection showing elevated sulfatide levels
- Must be prescribed by or in consultation with a physician who specializes in the treatment of MLD
- The member does not have evidence of residual cells of donor origin if the member has received a prior allogeneic hematopoietic stem cell transplant (allo-HSCT).
- **Duration of Approval:** One lifetime dose

For Kebilidi (eladocagene exuparvovec-tneq) requests:

Coverage may be provided with a diagnosis of **aromatic L amino acid decarboxylase (AADC) deficiency** and the following criteria is met:

- Diagnosis must be confirmed based on **all** of the following:
 - Genetic testing showing biallelic mutations in the DOPA decarboxylase (DDC) gene
 - Reduced levels of 5-hydroxyindoleacetic acid (5-HIAA), homovanillic acid (HVA) and 3-methoxy-4-hydroxyphenylglycol (MHPG) and high concentrations of 3-O-methyldopa (3-OMD), L-Dopa, and 5OH tryptophan (5-HTP) in the cerebral spinal fluid (CSF)
 - Reduced aromatic L-amino acid decarboxylase (AADC) activity in the plasma
- Must be prescribed by or in consultation with a pediatric neurologist
- Must present with classical clinical characteristics of AADC deficiency, such as oculogyric crises, hypotonia, and developmental delay
- Must not have any significant structural brain abnormality
- Must not have an anti-AAV2 neutralizing antibody titer over 1,200 folds
- Member must not have received prior treatment with any other AAV2-based gene therapy despite indication or are being considered for treatment with any other AAV2-based gene therapy
- **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-



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DMMA Approved: 02/2025

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.

**GENE THERAPY AGENTS
PRIOR AUTHORIZATION FORM- PAGE 1 of 4**

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 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? <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: <input type="checkbox"/> at a pharmacy OR <input type="checkbox"/> medically, JCODE: _____	
Place of Service: <input type="checkbox"/> Hospital <input type="checkbox"/> Provider's office <input type="checkbox"/> Member's home <input type="checkbox"/> Other	

Place of Service Information

Name:	NPI:
Address:	Phone:

MEDICAL HISTORY (Complete for ALL requests)

Diagnosis:	ICD Code:
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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

**GENE THERAPY AGENTS
PRIOR AUTHORIZATION FORM (CONTINUED) – PAGE 2 OF 4**

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If needed, you may call to speak to a Pharmacy Services Representative. **PHONE: (844) 325-6251 Mon – Fri 8 am to 7 pm**

MEMBER INFORMATION

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

MEDICAL HISTORY (Complete for ALL requests)

Diagnosis:	ICD Code:
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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

Is the member non-ambulatory? Yes No If yes, what is the member's total PUL score? _____

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

Does the member have cardiomyopathy with an ejection fraction less than 40%? Yes No

Does the member currently have an active infection? 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 the requested medication being used in combination with exon-skipping therapies? Yes No

Is there documentation of a baseline evaluation including a standardized assessment of motor function done by a neurologist with experience in treating DMD and liver function, platelet count and troponin-I levels assessed to be clinically appropriate?

Yes No

Beta-Thalassemia:

Is the member transfusion-dependent β -thalassaemia (TDT) who does not have a $\beta 0 / \beta 0$ 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

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

CALD:

Does the member have early, active CALD? Yes No

Does the member have elevated VLCFA? Yes No Value: _____

Has the member had an MRI establishing active CNS disease with GdE+ of demyelinating lesions? Yes No

What is the Loes score? _____

PRIOR AUTHORIZATION FORM (CONTINUED) – PAGE 3 OF 4

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MEMBER INFORMATION

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

MEDICAL HISTORY (Complete for ALL requests)

Diagnosis:	ICD Code:
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CALD (continued):
 What is the NFS score? _____
 Does the member have confirmed mutations in the ABCD1 gene? Yes No
 Does the member have an active infection? Yes No
 Does the member have HIV? Yes No
 Has the member received an allogenic transplant or gene therapy previously? Yes No

Sickle Cell Disease (SCD):
For Casgevy only:
 Does the member have severe sickle cell disease confirmed by electrophoresis? Yes, what is the genotype? _____ No
 Is the member eligible for a hematopoietic stem cell transplant but a stem cell donor is not available? Yes No
 Does the member have a history of at least 2 severe vaso-occlusive crisis (VOC) events during the past 24 months? Yes, please list the dates: _____ No
 Does the member have advanced liver disease or a history of untreated Moyamoya disease or the presence of Moyamoya disease? Yes No
 For members who are 12-18 years of age, does the member have a normal transcranial Doppler (TCD)? Yes No
 If the member is currently on disease modifying therapies for SCD (e.g., hydroxyurea, crizanlizumab, voxelotor), have they discontinued the product at least 8 weeks prior to start of mobilization and conditioning? Yes No
 Does the member have clinically significant and active bacterial, viral, fungal or parasitic infection? Yes No
 Has the member been a recipient of an allogenic transplant or gene therapy previously? Yes No

For Lyfgenia only:
 Does the member have severe sickle cell disease confirmed by electrophoresis? Yes, what is the genotype? _____ No
 Is the member eligible for a hematopoietic stem cell transplant but a stem cell donor is not available? Yes No
 Does the member have a history of at least 4 severe vaso-occlusive events (VOE) during the past 24 months? Yes, please list the dates: _____ No
 Does the member have a Karnofsky performance status of ≥ 60 (≥ 16 years of age) or a Lansky performance status of ≥ 60 (< 16 years of age)? Yes No
 Has the member tried and failed hydroxyurea (HU) at any point in the past or had an intolerance to HU? Yes No
 Does the member have advanced liver disease or a history of untreated Moyamoya disease or the presence of Moyamoya disease? Yes No
 For members who are 12-18 years of age, does the member have a normal transcranial Doppler (TCD)? Yes No
 Does the member need curative anticoagulation therapy during the period of conditioning through platelet engraftment?
 Is the member able to receive a red blood cell transfusion? Yes No
 Does the member have a negative serology test for HIV? Yes No
 Does the member have clinically significant and active bacterial, viral, fungal or parasitic infection? Yes No
 Has the member been a recipient of an allogenic transplant or gene therapy previously? Yes No

Metachromatic Leukodystrophy (MLD):
 Does the member have one of the following (please select the appropriate one): Yes No Other: _____
 Pre-symptomatic late infantile (PSLI) MLD
 Pre-symptomatic early juvenile (PSEJ) MLD
 Early symptomatic early juvenile (ESEJ) MLD

**GENE THERAPY AGENTS
PRIOR AUTHORIZATION FORM (CONTINUED) – PAGE 4 OF 4**

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MEMBER INFORMATION

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

MEDICAL HISTORY (Complete for ALL requests)

Diagnosis:	ICD Code:
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MLD continued:
 Has the diagnosis been confirmed by all of the following (submit confirmatory laboratory results): biochemical testing documenting human arylsulfatase A (ARSA) gene activity is below the normal range for the laboratory performing the test, the presence of two disease-causing ARSA alleles, either known or novel mutations, identified on genetic testing, and if novel mutations are identified, a 24-hour urine collection showing elevated sulfatide levels? Yes No
 Is the requested medication being prescribed by or in consultation with a physician who specializes in the treatment of MLD?
 Yes No
 Is there evidence of residual cells of donor origin if the member has received a prior allogeneic hematopoietic stem cell transplant (allo-HSCT)? Yes No

Aromatic L amino acid decarboxylase (AADC) deficiency:
 Has the diagnosis been confirmed based on **all** of the following (submit confirmatory laboratory results): Yes No

- Genetic testing showing biallelic mutations in the DOPA decarboxylase (DDC) gene
- Reduced levels of 5-hydroxyindoleacetic acid (5-HIAA), homovanillic acid (HVA) and 3-methoxy-4hydroxyphenylglycol (MHPG) and high concentrations of 3-O-methyl dopa (3-OMD), L-Dopa, and 5OH tryptophan (5-HTP) in the cerebral spinal fluid (CSF)
- Reduced aromatic L-amino acid decarboxylase (AADC) activity in the plasma

Is the requested medication being prescribed by or in consultation with a pediatric neurologist? Yes No
 Does the member present with classical clinical characteristics of AADC deficiency, such as oculogyric crises, hypotonia, and developmental delay? Yes No
 Does the member have any significant structural brain abnormality? Yes No
 Does the member have anti-AAV2 neutralizing antibody titer over 1,200 folds? Yes No
 Has the member received prior treatment with any other AAV2-based gene therapy despite indication or are being considered for treatment with any other AAV2-based gene therapy? 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



Updated: 01/2025
DMMA Approved: 02/2025