Drug/Group	Exclusion Criteria	Required Medical Information	Age Restriction	Prescriber Restriction	Coverage Duration	Other Criteria	NCD	LCD	Rationale for criteria	References
ALPHA-1 PROTEINASE INHIBITORS ARALAST NP GLASSIA PROLASTIN-C ZEMAIRA	Immunoglobulin A (IgA) deficient members with antibodies against IgA	Diagnosis. Must have a trial of Prolastin-C. Diagnosis of emphysema due to a congenital deficiency of alpha-1 proteinase inhibitor. Diagnosis confirmed by one of the following: a high risk alpha-1 antitrypsin deficiency (AATD) genetic variant Pi*ZZ, Pi*Z(null), Pi*(null)(null), or Pi*SZ protein phenotypes (homozygous) or other rare AAT deficiency disease-causing alleles associated with serum AAT level < 11 µmol/L. Member has a baseline circulating serum concentration of AATD < 11 µmol/L using rocket immunoelectrophoresis (which corresponds to < 80 mg/dl if measured by radial immunodiffusion or < 57 mg/dl if measured by nephelometry). To confirm a diagnosis of emphysema, must have a predicted FEV1 value between 30 and 65% or FEV1 from greater than 65% to less than 80% of predicted, post-bronchodilator, and a rapid decline in lung function showing a change in FEV1 greater than 100 mL/year. Member is currently a nonsmoker or ex-smoker.	Coverage is provided for members 18 years of age and older.	By or in consultation with a pulmonologist	Initial: 6 months Reauth: 12 months	For reauth: documentation of improvement or stabilization of the signs and symptoms of emphysema associated with alpha-1 antitrypsin deficiency including slowed progression of emphysema as evidenced by annual spirometry testing or a decrease in frequency, duration or severity of pulmonary exacerbations	None	None	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Aralast NP [package insert]. Westlake Village, CA: Baxalta US Inc.; March 2023. 2. Glassia [package insert]. Westlake Village, CA: Baxalta US Inc.; September 2022. 3. Prolastin-C [package insert]. Research Triangle Park, NC: Grifols Therapeutics, Inc.; May 2020. 4. Zemaira [package insert]. Kankakee, IL: CSL Behring LLC; September 2022. 7. American Thoracic Society / European Respiratory Society Statement: Standards for the diagnosis and management of individuals with alpha-1 antitrypsin deficiency. Am J Respir Crit Care Med. 2003; 168:818-900.
SPEVIGO (SPESOLIMAB-SBZO)		Diagnosis. Must have a moderate-to-severe flare of generalized pustular psoriasis (GPP) defined by ALL of the following: 1) GPPGA total score greater than or equal to 3 (moderate or severe), 2) presence of fresh pustules, 3) GPPGA postulation subscore of at least 2 (mild, moderate, or severe), and 4) at least 5% BSA covered with erythema and presence of pustules.	Coverage is provided for members 18 years of age or older.	By or in consultation with a dermatologist	One treatment course (up to 2 infusions over 2 weeks)		None	None	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	Spevigo [package insert]. Ridgefield, CT: Boehringer Ingelheim Pharmaceuticals Inc; September 2022.
CHIMERIC ANTIGEN RECEPTOR THERAPY (CAR-T) ABECMA (IDECABTAGENE VICLEUCEL), BREYANZI (LISOCABTAGENE MARALEUCEL), CARVYKTI CILTACABTAGENE AUTOLEUCEL), KYMRIAH (TISAGENLECLEUCEL), TECARTUS (BREXUCABTAGENE AUTOLEUCEL), YESCARTA (AXICABTAGENE CILOLEUCEL)	Must not be used in combination with other chemotherapy agents. Must not be given as repeat treatment in members who have received CAR-T treatment previously. Must not be given if the member has primary central nervous system (CNS) lymphoma.	Diagnosis. Must have tried and failed FDA labeled treatments required in prescribing information labeling. Must have documentation of appropriate CD tumor expression for specific CAR-T therapy. The member has received or will receive lymphodepleting chemotherapy within two weeks preceding infusion unless the member's WBC count is less than or equal to $1x10^9$/L within 1 week prior to infusion.	For ALL, coverage is provided up to 25 years old. For all other indications, must be 18 years or older.	By or in consultation with an Oncologist/hematologist	One treatment course		Chimeric Antigen Receptor (CAR) T-cell Therapy	None	This is a rare disease state requiring a mutlidisciplinary team for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Kymriah [Product Information]. Novartis Pharmaceuticals Corporation, East Hanover, NJ; May 2022. 2. Yescarta [Product Information]. Kite Pharma, Inc. Santa Monica, CA; April 2022. 3.Tecartus [Product Information]. Kite Pharma, Inc. Santa Monica, CA; October 2021. 4.Breyanzi [package insert]. Juno Therapeutics Inc., a Bristol-Myers Squibb Company, Bothell, WA; June 2022. 5. Abecma [package insert]. Celgene Corporation, a Bristol-Myers Squibb Company, Summit, NJ; March 2021. 6.Carvykti [package insert]. Janssen Biotech, Inc, Horsham, PA; February 2023.
VYJUVEK (BEREMAGENE GEPERPAVEC)		Diagnosis of Dystrophic Epidemolysis Bullosa (DEB) with a mutation in the collagen type VII alpha 1 chain (COL7A1) gene confirmed by genetic testing. Must have a wound with no evidence or history of squamous-cell carcinoma or active infection.	Coverage is provided for members 6 months of age or older.	By or in consultation with a dermatologist	6 months	Reauthorization: must have documentation from prescriber indicating improvement in condition.	None	None	This is a rare disease state requiring a mutlidisciplinary team for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Vyjuvek [package insert]. Pittsburgh, PA: Krystal Biotech; May 2023. 2.Guide SV, et al. Trial of Beremagene Geperpavec (B-VEC) for Dystrophic Epidermolysis Bullosa. N Engl J Med. 2022 Dec 15; 387(24):2211-2219 doi: 10.1056/NEJMoa2206663. Available at: Trial of Beremagene Geperpavec (B-VEC) for Dystrophic Epidermolysis Bullosa | NEJM
DUCHENNE MUSCULAR DYSTROPHY AGENTS AMONDYS 45 (CASIMERSEN), EXONDYS 51 (ETEPLIRSEN), VILTEPSO (VILTOLARSEN), VYONDYS (GOLODIRSEN)	Cannot be used in combination with another DMD agent	Diagnosis. A confirmed diagnosis of DMD by submission of lab testing demonstrating mutation of the dystrophin gene amenable to the appropriate exon skipping. The member will receive concurrent corticosteroids unless contraindicated or intolerant. Documentation of a baseline evaluation, including a standardized assessment of motor function.		By or in consultation with a neurologist who has experience in the treatment and management of DMD	12 months	Reauth: The member has documentation of an annual evaluation, including an assessment of motor function ability; the member continues to benefit from treatment; the member will receive concurrent corticosteroids unless contraindicated or intolerant (severe adverse reactions)	None	None	This is a rare disease state requiring a mutlidisciplinary team for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Exondys 51 [package insert]. Sarepta Therapeutics, Inc., Cambridge, MA; Jan 2022. 2. Vyondys 53 [package insert]. Sarepta Therapeutics, Inc., Cambridge, MA; Feb 2021. 3. Viltepso [package insert]. NS Pharma, Inc., Paramus, NJ; March 2021. 4. Amonyds 45 [package insert]. Sarepta Therapeutics, Inc., Cambridge, MA; February 2021. 7. Brushby, K. et.al Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and pharmacological and psychosocial management. The Lancet Neurology 2010, 9(1) 77-93.

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ZYNTEGLO (BETIBEGLOGENE AUTOTEMCEL)	Recipient of an allogenic transplant or gene therapy; pregnant or breast-feeding; severely elevated iron in the heart or advanced liver disease or members with an MRI of the liver with results demonstrating liver iron content greater than or equal to 15 mg/g unless biopsy confirms absence of advanced disease.	Diagnosis. The member must have a diagnosis of beta-thalassemia confirmed by *HBB* sequence gene analysis showing biallelic pathogenic variants or member has severe microcytic hypochromic anemia, anisopoikilocytosis with nucleated red blood cells on peripheral blood smear, and hemoglobin analysis that reveals decreased amounts or complete absence of hemoglobin A and increased amounts of hemoglobin F.The member must be 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. 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 greater than or equal to 8 transfusions of RBCs per year in the 2 years preceding therapy. Iron chelation therapy has been discontinued for at least 7 days prior to initiating myeloablative conditioning therapy.	Coverage is provided for members 4 years of age and older	Prescribed by or in consultation with a hematologist, stem cell transplantation specialist or in the treatment of members with transfusion-dependent β-thalassaemia (TDT)	Once per lifetime	Must be used as a single agent therapy. Must be administered in a qualified treatment center. Member must be confirmed that HSC transplantation is appropriate for the member before myeloablative conditioning is initiated.	None	None	This is a rare disease state requiring a mutlidisciplinary team for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Zynteglo [package insert]. Somerville, MA. bluebird bio, Inc, August 2022.
CASGEVY (EXAGAMGLOGENE AUTOTEMCEL)	Advanced liver disease or a history of untreated Moyamoya disease or presence of Moyamoya disease; clinically significant and active bacterial, viral, fungal or parasitic infection; recipient of an allogenic transplant or gene therapy.	Diagnosis. Diagnosis is confirmed by electrophoresis demonstrating the presence of sickle cell disease with either βS/βS or βS/β0 or βS/β+ 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 2 severe vaso-occlusive crisis (VOC) events during each of the prior 2 years. 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 be 12 years of age or older	By or in consultation with hematologist/oncologist or sickle cell disease specialist	Once per lifetime		None	None	This is a rare disease state requiring a mutlidisciplinary team for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	Casgevy [package insert]. Boston, MA Vertex Pharmaceuticals. December 2023.
LYFGENIA (LOVOTIBEGLOGENE AUTOTEMCEL)	Advanced liver disease, history of untreated Moyamoya disease, presence of Moyamoya disease; clinically significant and active infection; recipient of an allogenic transplant or gene therapy; need for curative anticoagulation therapy during the period of conditioning through platelet engraftment; positive serology test for HIV.	Diagnosis. Diagnosis is confirmed by electrophoresis demonstrating the presence of sickle cell disease with either βS/βS or βS/β0 or βS/β+ 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 crisis (VOC) events during each of the prior 2 years. 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). 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 be able to receive a red blood cell transfusion.	Member must be 12 years of age or older	By or in consultation with hematologist/oncologist or sickle cell disease specialist	Once per lifetime		None	None	This is a rare disease state requiring a mutlidisciplinary team for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	Lyfgenia [package insert]. Somerville, MA. Bluebird bio, Inc. December 2023.
SKYSONA (ELIVALDOGENE AUTOTEMCEL)	Active infection including clinically important localized infections; recipient of an allogenic transplant or gene therapy; vaccinations administered within the 6 weeks prior to the start of therapy.	Diagnosis. Member must be a male with a diagnosis of early, active cerebral adrenoleukodystrophy (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 leass than or equal to 1 demonstrating asymptomatic or mild disease. Member must have confirmed mutations in the ABCD1 gene and does not have a full ABCD1-gene deletion. Member must have a negative serology test for HIV.	Coverage is provided for members 4 to 17 years of age	Prescribed by or in consultation with a neurologist or adrenoleukodystrophy (ALD) specialist.	Once per lifetime	Must be used as a single agent therapy. Must be administered in a qualified treatment center.	None	None	This is a rare disease state requiring a mutlidisciplinary team for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Skysona (elivaldogene autotemce) [prescribing information]. Somerville, MA. Bluebird bio, Inc; September 2022.

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BERINERT (C1 ESTERASE INHIBITOR)		Diagnosis of HAE is confirmed by laboratory values obtained on two separate instances (laboratory reports must contain reference ranges). For Type I: Low C4 level and low C1-INH antigenic level. For Type II: Low C4 level and normal or elevated C1-INH antigenic level and low C1-INH functional level. For all types, must have chart documentation of each previous HAE attack in the absence of hives or a medication known to cause angioedema. Member must not be taking any medications that may exacerbate HAE, including angiotensin-converting enzyme (ACE) inhibitors, Tamoxifen, and estrogen-containing medications. Must be using to treat acute HAE attacks. Must not be receiving more than one medication for the acute treatment of an HAE attack at a time.		Prescribed by or in consultation with an allergist/immunologist, hematologist, dermatologist	Initial: 3 months Reauth: 12 months	Reauthorization: Documentation of improvement or stabilization.	None	None	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Berinert [package insert]. Kankakee, IL: CSL Behring; September 2021.
CINRYZE (C1 ESTERASE INHIBITOR)		Diagnosis. Must have a trial of Haegarda. Diagnosis of HAE is confirmed by laboratory values obtained on two separate instances (laboratory reports must contain reference ranges). For Type I: Low C4 level and low C1-INH antigenic level. For Type II: Low C4 level and normal or elevated C1-INH antigenic level and low C1-INH functional level. For all types, must have chart documentation of each previous HAE attack in the absence of hives or a medication known to cause angioedema. To demonstrate member is candidate for prophylactic therapy, must include one of the following: history of frequent HAE attacks (defined as 2 or more HAE attacks per month) or history of severe HAE attacks (defined as 1 or more abdominal attack in past 12 months or any attack of respiratory tract which compromised airway). Member must not be taking any medications that may exacerbate HAE, including angiotensin-converting enzyme (ACE) inhibitors, Tamoxifen, and estrogen-containing medications. Must be using Cinryze as prophylactic therapy for the prevention of HAE attacks.	Coverage is provided for members 6 years of age and older.	Prescribed by or in consultation with an allergist/immunologist, hematologist, dermatologist	Initial: 6 months Reauth: 12 months	Reauthorization: Documentation of improvement or stabilization.	None	None	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	Cinryze [package insert]. Lexington, MA: Shire ViroPharma Inc.; February 2023
HAEGARDA (C1 ESTERASE INHIBITOR)		Diagnosis of HAE is confirmed by laboratory values obtained on two separate instances (laboratory reports must contain reference ranges). For Type I: Low C4 level and low C1-INH antigenic level. For Type II: Low C4 level and normal or elevated C1-INH antigenic level and low C1-INH functional level. For all types, must have chart documentation of each previous HAE attack in the absence of hives or a medication known to cause angioedema. To demonstrate member is candidate for prophylactic therapy, must include one of the following: history of frequent HAE attacks (defined as 2 or more HAE attacks per month) or history of severe HAE attacks (defined as 1 or more abdominal attack in past 12 months or any attack of respiratory tract which compromised airway). Member must not be taking any medications that may exacerbate HAE, including angiotensin-converting enzyme (ACE) inhibitors, Tamoxifen, and estrogen-containing medications. Must be using as prophylactic therapy for the prevention of HAE attacks.	Coverage is provided for members 6 years of age and older.	Prescribed by or in consultation with an allergist/immunologist, hematologist, dermatologist	Initial: 6 months Reauth: 12 months	Reauthorization: Documentation of improvement or stabilization.	None	None	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	Haegarda [package insert]. Kankakee, IL: CSL Behring; January 2022
KALBITOR (ECALLANTIDE)		Diagnosis of HAE is confirmed by laboratory values obtained on two separate instances (laboratory reports must contain reference ranges). For Type I: Low C4 level and low C1-INH antigenic level. For Type II: Low C4 level and normal or elevated C1-INH antigenic level and low C1-INH functional level. For all types, must have chart documentation of each previous HAE attack in the absence of hives or a medication known to cause angioedema. Member must not be taking any medications that may exacerbate HAE, including angiotensin-converting enzyme (ACE) inhibitors, Tamoxifen, and estrogen-containing medications. Must be using to treat acute HAE attacks. Must not be receiving more than one medication for the acute treatment of an HAE attack at a time.	Coverage is provided for members 12 years of age and older.	Prescribed by or in consultation with an allergist/immunologist, hematologist, dermatologist	Initial: 3 months Reauth: 12 months	Reauthorization: Documentation of improvement or stabilization.	None	None	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Kalbitor [package insert]. Burlington, MA: Dyax Corp; November 2021.

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RUCONEST (C1 ESTERASE INHIBITOR)		Diagnosis of HAE is confirmed by laboratory values obtained on two separate instances (laboratory reports must contain reference ranges). For Type I: Low C4 level and low C1-INH antigenic level. For Type II: Low C4 level and normal or elevated C1-INH antigenic level and low C1-INH functional level. For all types, must have chart documentation of each previous HAE attack in the absence of hives or a medication known to cause angioedema. Member must not be taking any medications that may exacerbate HAE, including angiotensin-converting enzyme (ACE) inhibitors, Tamoxifen, and estrogen-containing medications. Must be using to treat acute HAE attacks. Must not be receiving more than one medication for the acute treatment of an HAE attack at a time.	Coverage is provided for members 13 years of age and older.	Prescribed by or in consultation with an allergist/immunologist, hematologist, dermatologist	Initial: 3 months Reauth: 12 months	Reauthorization: Documentation of improvement or stabilization.	None	None	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	Ruconest [package insert]. Raleigh, NC: Salix Pharmaceuticals, Inc.; April 2020.
TAKHZYRO (LANADELUMAB-FLYO)		Diagnosis. Must have a trial of Haegarda. Diagnosis of HAE is confirmed by laboratory values obtained on two separate instances (laboratory reports must contain reference ranges). For Type I: Low C4 level and low C1-INH antigenic level. For Type II: Low C4 level and normal or elevated C1-INH antigenic level and low C1-INH functional level. For all types, must have chart documentation of each previous HAE attack in the absence of hives or a medication known to cause angioedema. To demonstrate member is candidate for prophylactic therapy, must include one of the following: history of frequent HAE attacks (defined as 2 or more HAE attacks per month) or history of severe HAE attacks (defined as 1 or more abdominal attack in past 12 months or any attack of respiratory tract which compromised airway). Member must not be taking any medications that may exacerbate HAE, including angiotensin-converting enzyme (ACE) inhibitors, Tamoxifen, and estrogen-containing medications. Must be using as prophylactic therapy for the prevention of HAE attacks.	Coverage is provided for members 12 years of age and older.	Prescribed by or in consultation with an allergist/ immunologist, hematologist, dermatologist	Initial: 6 months Reauth: 12 months	Reauthorization: Documentation of improvement or stabilization.	None	None	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Takhzyro [package insert]. Lexington, MA: Dyax Corp; February 2023.
HATTR AMYLOIDOSIS AMVUTTRA (VUTRISIRAN), ONPATTRO (PATISIRAN), TEGSEDI (INOTERSEN),	Member will not be receiving the requested medication in combination with another TTR stabilizer.	Diagnosis. Documentation the member has hATTR amyloidosis with polyneuropathy confirmed by the presence of a transthyretin (TTR) gene mutation (e.g., V30M, A97S, T60A, E89Q, S50R). Documentation of a baseline modified Neuropathy Impairment Scale +7 (mNIS+7) composite score and Norfolk Quality of Life-Diabetic Neuropathy (QoL-DN) total score have been performed. Documentation of baseline polyneuropathy disability (PND) score of ≤ IIIb or baseline familial amyloid polyneuropathy (FAP) Stage 1 or 2. Member has clinical signs and symptoms of polyneuropathy (i.e. weakness, sensory loss, decreased motor strength, decreased gait speed). Other causes of peripheral neuropathy have been assessed and ruled out.	Coverage is provided for members 18 years of age and older	By or in consultation with a neurologist or a specialist in the treatment of amyloidosis	12 months	Reauthorization criteria: Documentation of a therapeutic response as evidenced by stabilization or improvement from baseline in PND, FAP, mNIS or QoL-DN scores.	None	None	These disease states requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Onpattro [prescribing information]. Cambridge, MA: Alnylam Pharmaceuticals. January 2023. 2. Tegsedi [prescribing information]. Carlsbad, CA: Ionis Pharmaceuticals. June 2022. 3. Amvuttra [prescribing information]. Cambridge, MA. Alnylam Pharmaceuticals, Inc. February 2023.
REMODULIN (TREPROSTINIL)		Diagnosis. Pulmonary arterial hypertension (PAH) WHO Group I confirmed by chart documentation of right-heart catheterization (RHC) indicating a mean pulmonary arterial pressure greater than 20 mmHg, pulmonary vascular resistance greater than 2 wood units, and mean pulmonary capillary wedge pressure less than or equal to 15 mmHg. If provider indicates RHC is not recommended, must have documentation of an echocardiography. Must provide documentation of trial and failure, contraindication, or intolerance to generic epoprostenol.	Coverage is provided for members 18 years of age or older.	Prescribed by or in consultation with cardiologist or pulmonologist.	Initial: 3 months Reauth: 12 months	For reauth: documentation from prescriber that demonstrates member is tolerating and receiving clinical benefit from treatment	None	External Infusion Pumps (L33794)	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Humbert M, Kovacs G, Hoeper M,, et al. 2022 ESC/ERS Guidelines for the diagnosis and treatment of pulmonary hypertension. The Joint Task Force for the Diagnosis and Treatment of Pulmonary Hypertension of the European Society of Cardiology (ESC) and the European Respiratory Society (ERS). Eur Heart J. 2022:43:3617-3731. 2. Klinger JR, Elliott CG, Levine DJ, et al. Therapy for Pulmonary Arterial Hypertension in Adults: Update of the CHEST Guideline and Expert Panel Report. Chest. 2019; 155(3):565-586. 3. Taichman DB, Ornelas J, Chung L, et al. Pharmacologic therapy for pulmonary arterial hypertension in adults: CHEST guideline and expert panel report. Chest. 2014; 146(2):449-75.

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TYVASO (TREPROSTINIL)		Diagnosis. **Pulmonary arterial hypertension (PAH, WHO Group I):** must be confirmed by chart documentation of right-heart catheterization (RHC) indicating a mean pulmonary arterial pressure (MPAP) greater than20 mmHg, pulmonary vascular resistance (PVR) greater than 2 wood units, and mean pulmonary capillary wedge pressure (PCWP) less than or equal to 15 mmHg. If provider indicates RHC is not recommended, must have documentation of an echocardiography. Must have WHO Functional Class III-IV symptoms. Must provide documentation of trial and failure, contraindication, or intolerance to generic epoprostenol. **Pulmonary hypertension associated with interstitial lung disease (PH-ILD; WHO Group 3):** must be confirmed by RHC documentation meeting one of the following hemodynamic value combinations: 1) MPAP greater than 20 mmHg 2) PVR greater than or equal to 2 Wood units. If provider indicates RHC is not recommended, must have documentation of an echocardiography. Must have a concurrent chronic lung disease diagnosis (COPD, emphysema, pulmonary fibrosis, sarcoidosis, etc.)	Coverage is provided for members 18 years of age or older.	Prescribed by or in consultation with a pulmonologist or cardiologist	12 months	For reauthorization: documentation from prescriber that demonstrates member is tolerating and receiving clinical benefit from treatment	None	None	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Humbert M, Kovacs G, Hoeper M,, et al. 2022 ESC/ERS Guidelines for the diagnosis and treatment of pulmonary hypertension. The Joint Task Force for the Diagnosis and Treatment of Pulmonary Hypertension of the European Society of Cardiology (ESC) and the European Respiratory Society (ERS). Eur Heart J. 2022:43:3617-3731. 2. Klinger JR, Elliott CG, Levine DJ, et al. Therapy for Pulmonary Arterial Hypertension in Adults: Update of the CHEST Guideline and Expert Panel Report. Chest. 2019; 155(3):565-586. 3. Taichman DB, Ornelas J, Chung L, et al. Pharmacologic therapy for pulmonary arterial hypertension in adults: CHEST guideline and expert panel report. Chest. 2014; 146(2):449-75.
VENTAVIS (ILOPROST)		Diagnosis. Pulmonary arterial hypertension (PAH) WHO Group I confirmed by chart documentation of right-heart catheterization (RHC) indicating a mean pulmonary arterial pressure greater than 20 mmHg, pulmonary vascular resistance greater than 2 wood units, and mean pulmonary capillary wedge pressure less than or equal to 15 mmHg. If provider indicates RHC is not recommended, must have documentation of an echocardiography. Must have WHO Functional Class III-IV symptoms.	Coverage is provided for members 18 years of age or older.	Prescribed by or in consultation with a pulmonologist or cardiologist	12 months	For reauthorization: documentation from prescriber that demonstrates member is tolerating and receiving clinical benefit from treatment	None	None	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Humbert M, Kovacs G, Hoeper M,, et al. 2022 ESC/ERS Guidelines for the diagnosis and treatment of pulmonary hypertension. The Joint Task Force for the Diagnosis and Treatment of Pulmonary Hypertension of the European Society of Cardiology (ESC) and the European Respiratory Society (ERS). Eur Heart J. 2022:43:3617-3731. 2. Klinger JR, Elliott CG, Levine DJ, et al. Therapy for Pulmonary Arterial Hypertension in Adults: Update of the CHEST Guideline and Expert Panel Report. Chest. 2019; 155(3):565-586. 3. Taichman DB, Ornelas J, Chung L, et al. Pharmacologic therapy for pulmonary arterial hypertension in adults: CHEST guideline and expert panel report. Chest. 2014; 146(2):449-75.
POMPE DISEASE, ENZYME REPLACEMENT THERAPY LUMIZYME (ALGLUCOSIDASE ALFA), NEXVIAZYME (AVALGLUCOSIDASE ALFA)		Diagnosis. For Pompe Disease, documentation of one of the following: deficiency of acid alpha-glucosidase enzyme activity or detection of pathogenic variants in the GAA gene by molecular gene testing. Documentation of baseline vlaues for one or more of the following: infantile-onset disease- muscle weakness, motor function, respiratory function, cardiac involvement, percent predicted forced vital capacity (FVC) OR for late-onset (non-infantile) disease -percent predicted forced vital capacity (FVC), walking distance or 6-minute walk test (6MWT) or gastrointestinal symptoms. 6MWT excluded for members at an age not able to walk.		By or in consultation with a biochemical geneticist or metabolic physician	12 months	Reauth: Documentation of a clinical benefit to therapy compared to pre-treatment baseline in one or more of the following: infantile-onset disease- stabilization or improvement in muscle weakness, motor function, respiratory function, cardiac involvement, or FVC OR late-onset (non-infantile) disease- stabilization or improvement in FVC and/or 6MWT and signs or symptoms of the condition.	None	None	This is a rare disease state requiring a mutlidisciplinary team for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Lumizyme [package insert]. Cambridge, MA: Genzyme Corporation; March 2023. 2. American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM). Diagnostic criteria for late-onset (childhood and adult) Pompe disease. Muscle Nerve. 2009; 40(1):149-160. 3. Nexviazyme [package insert]. Cambridge, MA: Genzyme Corporation; April 2023.
ENSPRYNG (SATRALIZUMAB-MWGE)	Active hepatitis B infection, active or untreated latent tuberculosis	Diagnosis. **For Neuromyelitis Optica Spectrum Disorder (NMOSD):** positive test for AQP4-IgG antibodies; At least 1 relapse in the last 12 months or 2 relapses in the last 24 months that required rescue therapy; Expanded Disability Status Scale (EDSS) score ≤ 6.5. Must have documentation of inadequate response, contraindication or intolerance to one (1) immunosuppressant (e.g., mycophenolate mofetil, azathioprine, methotrexate) or to rituximab or any of its biosimilars.	Coverage is provided for members 18 years of age and older	By or in consultation with a neurologist	12 months	For reauth: documentation of stabilization or improvement in condition	None	None	These disease states requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Enspryng (satralizumab-mwge) [prescribing information]. South San Francisco, CA; Genentech, Inc; March 2022. 2. Pittock, S. J., Berthele, A., Fujihara, K., Kim, H. J., Levy, M., Palace, J.,Wingerchuk, D. M. (2019). Eculizumab in Aquaporin-4–Positive Neuromyelitis Optica Spectrum Disorder. New England Journal of Medicine. doi: 10.1056/nejmoa1900866

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SOLIRIS (ECULIZUMAB)		Diagnosis. **For Paroxysmal Nocturnal Hemoglobinuria (PNH):** confirmatory flow cytometry testing, LDH level at least 1.5 times the upper limit of the normal range, and hemoglobin less than or equal to 7 g/dL or hemoglobin less than or equal to 9 g/dL and documentation of anemia symptoms. Must provide documentation of trial and failure, contraindication, or intolerance to Ultomiris. **For Atypical Hemolytic Uremic Syndrome (aHUS):** documentation of hemolysis such as an elevation in serum LDH and serum creatinine above upper limits of normal or required dialysis. Must provide documentation of the absence of Shiga toxin-producing E. coli infection and disintegrin and metalloproteinase with thrombospondin type 1 motif member 13 (ADAMTS13) deficiency. Must provide documentation of trial and failure, contraindication, or intolerance to Ultomiris. **For Generalized Myasthenia Gravis (gMG):** positive serologic test for anti-acetylcholine antibodies, Myasthenia Gravis-Specific Activities of Daily Living total score greater than or equal to 6, meets Myasthenia Gravis Foundation of America Clinical Classification II to IV criteria, and treatment failure over 1 year or more with 1 or more immunosuppressive therapies. **For Neuromyelitis Optica Spectrum Disorder (NMOSD):** positive test for AQP4-IgG antibodies; At least 2 relapses in the last 12 months or 3 or more relapses in the last 24 months with at least 1 relapse in the last 12 months; Expanded Disability Status Scale (EDSS) score of less than or equal to 7; if using concurrent corticosteroids, dose is less than or equal to the equivalent of 20mg prednisone per day. Must have documentation of inadequate response, contraindication or intolerance to rituximab or any of its biosimilars.	aHUS: Coverage is provided for members 2 months of age or older. All other DX: Coverage is provided for members 18 years of age or older.	Prescribed by or in consultation with a hematologist, oncologist, immunologist, genetic specialist, neurologist, or nephrologist.	Initial: 6 months Reauth: 12 months	**PNH Reauth:** LDH level (within 3 mo) that shows a reduction from baseline and one of the following: -If baseline Hgb was 9 g/dL or higher, it has not dropped by more than 2 g/dL from baseline. -If baseline Hbg was less than 9 g/dL, it is above 7g/dL **aHUS Reauth:** any one of the following-increase in Plt count from baseline, maintenance of normal Plt counts and LDH levels for at least 4 weeks, 25% reduction in serum creatinine for a minimum of 4 wks, or member has not experienced a decrease in Plt count >25% from baseline, plasma exchange, plasma infusion, or a new dialysis requirement in at least 12 weeks **gMG Initial Reauth:** 3 point improvement in member's MG-ADL score or 5 point improvement in QMG total score **gMG Subsequent Reauth:** stabilization or improvement in condition **NMOSD Reauth:** stabilization or improvement in condition	None	None	These disease states requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Soliris (eculizumab) [package insert]. Cheshire, CT: Alexion Pharm; November 2020.
ULTOMIRIS (RAVULIZUMAB-CWVZ)		Diagnosis. **For Paroxysmal Nocturnal Hemoglobinuria (PNH):** confirmatory flow cytometry testing, LDH level at least 1.5 times the upper limit of the normal range, and hemoglobin less than or equal to 7 g/dL or hemoglobin less than or equal to 10 g/dL and documentation of anemia symptoms. **For Atypical Hemolytic Uremic Syndrome (aHUS):** documentation of hemolysis such as an elevation in serum LDH and serum creatinine above upper limits of normal or required dialysis. Must provide documentation of the absence of Shiga toxin-producing E. coli infection and disintegrin and metalloproteinase with thrombospondin type 1 motif member 13 (ADAMTS13) deficiency.	aHUS: Coverage is provided for members 1 month of age or older. All other DX: Coverage is provided for members 18 years of age or older.	Prescribed by or in consultation with a hematologist, oncologist, immunologist, genetic specialist, or nephrologist.	Initial: 6 months Reauth: 12 months	**PNH Reauth:** Documentation of LDH level (within 3 months) that shows a reduction from baseline and one of the following: -If baseline Hgb was 9 g/dL or higher, it has not dropped by more than 2 g/dL from baseline. -If baseline Hgb was less than 9 g/dL, it is above 7g/dL **aHUS Reauth:** any one of the following-increase in Plt count from baseline, maintenance of normal Plt counts and LDH levels for at least 4 weeks, 25% reduction in serum creatinine for a minimum of 4 weeks, or member has not experienced a decrease in Plt count >25% from baseline, plasma exchange, plasma infusion, or a new dialysis requirement in at least 12 weeks	None	None	These disease states requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Ultomiris [package insert]. Boston, MA: Alexion Pharmaceuticals, Inc.; January 2022.
UPLIZNA (INEBILIZUMAB-CDON)	Active hepatitis B infection, active or untreated latent tuberculosis	Diagnosis. **For Neuromyelitis Optica Spectrum Disorder (NMOSD):** positive test for AQP4-IgG antibodies; At least 1 relapse in the last 12 months or 2 relapses in the last 24 months that required rescue therapy; Expanded Disability Status Scale (EDSS) score of less than or equal to 8; Must have documentation of inadequate response, contraindication or intolerance to an immunosuppressant (e.g., mycophenolate mofetil, azathioprine, methotrexate) or to rituximab or any of its biosimilars.	Coverage is provided for members 18 years of age and older	By or in consultation with a neurologist	12 months	For reauth: documentation of stabilization or improvement in condition	None	None	These disease states requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Uplizna (inebilizumab-cdon) [prescribing information]. Gaithersburg, MD; Viela Bio, Inc; July 2021.

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XIAFLEX (COLLAGENASE)	Sexual or erectile dysfunction associated with Peyronie's disease.	Diagnosis. **For Dupuytren's Contracture:** documentation the member has one of the following: a finger flexion contracture with a palpable cord of at least one finger (other than the thumb) or a positive "table top test" defined as the inability to simultaneously place the affected finger(s) and palm flat against a table top. Documentation that the flexion deformity results in functional limitations. Documentation of which cords on which hand are being treated and dates of treatment. A maximum of two cords in the same hand may be treated during a single treatment visit (all treatment visits must be at least 4 weeks apart) A cord may not be injected more than 3 times and at an interval less than 4 weeks. Must not have received a surgical treatment (e.g. fasciectomy, fasciotomy) on the selected primary joint within 90 days before the first injection. **For Peyronie's disease:** documentation of a palpable plaque and curvature deformity of at least 30 degrees and less than 90 degrees at the start of therapy. Documentation the member has stable disease (i.e. symptoms have remained unchanged for at least 3 months). Documentation erectile function is intact. Injections for Peyronie's disease are limited to 4 treatment cycles. (Each cycle consists of 2 Xiaflex injections and one remodeling procedure.)	Coverage is provided for members 18 years of age and older	Peyronie's disease: Prescribed by or in consultation with a urologist	Dupuytren's Contracture: 4 months Peyronie's disease: 6 months		None	None	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Trojian, T and Chu S. Dupuytren's Disease: Diagnosis and Treatment. *American Family Physician.* July 2007; 76(1): 86-89. 2. DA McGrouther, A Bayat. Management of Dupuytren's Disease- Clear Advice for an Elusive Condition. Annals Royal College of Surgeons England. 2006; 88: 3-8. 3. Xiaflex [package insert]. Malvern, PA: Endo Pharmaceuticals Inc.; November 2019. 4. Nehra A, Alterowitz R, Culkin DJ, et al. Peyronie's Disease: AUA Guideline. J Urol 2015; 194:745.
LUXTURNA (VORETIGENE NEPARVOVEC-RZYL)	Member has previously received treatment with voretigene neparvovec-rzyl in the requested treatment eye.	Diagnosis. Must have confirmed RPE65 mutation in both alleles confirmed by both of the following: Clinical documentation confirming diagnosis of Leber congenital amaurosis (LCA) or Retinitis pigmentosa (RP) including clinical features, funduscopic appearance, and results of testing such as dark-adapted thresholds, Ganzfeld-flash electroretinography (ERG), and when appropriate, perimetry AND Documentation of positive genetic test result confirming a biallelic pathogenic or likely pathogenic RPE65 mutation (homozygote or compound heterozygote) by a MoIDX-approved mutational test. Must have viable retinal cells as determined by at least one of the following: Area of retina within the posterior pole of greater than 100 µm thickness per optical coherence tomography (OCT), At least 3 disc areas of retina without atrophy or pigmentary degeneration within the posterior pole, OR Remaining visual field within 30 degrees of fixation as measured by a III4e isopter or equivalent.	Coverage is provided for members 1 year of age and older and less than 65 years of age.	By or in consultation with an ophthalmologist	1 injection per eye per lifetime		None	None	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Luxturna (voretigene neparvovec-rzyl) [package insert]. Philadelphia, PA: Spark Therapeutics; 2017. 2. 2.Russell S, Bennett J, Wellman JA, et al. Efficacy and safety of voretigene neparvovec (AAV2-hRPE65v2) in patients with RPE-mediated inherited retinal dystrophy: a randomized, controlled, open-label, phase 3 trial. Lancet. 2017; 390: 849-860. 3. 3.BLA 125610 Voretigene Neparvovec. FDA Briefing Document Advisory Committee Meeting. Accessed February 4, 2019 at https://www.fda.gov/downloads/AdvisoryCommittees/CommitteesMeetingMaterials/BloodVaccinesandOtherBiologics/CellularTissueandGeneTherapiesAdvisoryCommittee/UCM579290.pdf
ADAKVEO (CRIZALIZUMAB-TMCA)		Diagnosis must be confirmed by electrophoresis demonstrating the presence of sickle cell disease (HbSS, HbSC, HbSβ^o-thalassemia, or HbSβ^+-thalassemia). Must provide documentation showing the member has tried and failed or had an intolerance or contraindication to at least a 6 month trial of hydroxyurea. Member must have had at least 2 vaso-occlusive crises in the past 12 months.	Coverage is provided for members 16 years of age and older.	By or in consultation with hematologist/oncologist or sickle cell disease specialist	12 months	Reauthorization: Decrease or stabilization in vaso-occlusive events.	None	None	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Adakveo (crizanlizumab) prescribing information. East Hanover, New Jersey, USA. Novartis Pharmaceuticals Corporation; September 2022.
SPINRAZA (NUSINERSEN)	Must not be used concomitantly with Evrysdi	Diagnosis. 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. 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), or Six-minute walk test.		Prescribed by or in consultation with a neurologist with experience treating spinal muscular atrophy.	Initial: 4 months Reauth: 12 months	Reauthorization criteria: Documentation the member is responding to the medication as demonstrated by clinically significant improvement or maintenance of function from pretreatment baseline status using the same exam as performed at baseline assessment (i.e. ONE of the following assessments: 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), or Six-minute walk test).	None	None	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	1. Spinraza [package insert]. Cambridge, MA: Biogen, Inc, February 2023.

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ZOLGENSMA (ONASEMNOGENE ABEPARVOVEC-XIOI)	Member is dependent on either of the following: Invasive ventilation or tracheostomy OR use of non-invasive ventilation beyond use for naps and nighttime sleep. Member must not have received this therapy previously.	Diagnosis. Documentation of genetic testing confirming ALL of the following: Bi-allelic SMN1 deletions or pathogenic variants, Two copies of SMN2 gene, and lack of the c.859G>C modification in exon 7 of the SMN2 gene. Member must have an anti-AAV9 antibody titer below or equal to 1:50. 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), or Six-minute walk test.	Coverage is provided for members less than 2 years of age.	Prescribed by or in consultation with a neurologist with experience treating spinal muscular atrophy.	Once per lifetime	The prescriber attests that the member's weight for dosing must be 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.	None	None	This disease state requires specialists for appropriate care. A PA is required to ensure accurate diagnosis and appropriate treatment selection.	Zolgensma [package insert]. Bannockburn, Il, AveXis,inc. February 2023