

POLICY AND PROCEDURE

POLICY NAME: Enzyme Replacement Therapy (Fabrazyme, Lumizyme, Nexviazyme, Brineura, Vimizim, Naglazyme, Elaprase, Cerezyme, Aldurazyme, Ceprotin, Kanuma, Elelyso, Vpriv, Xenpozyme)	POLICY ID: TX.PHAR.104
BUSINESS UNIT: Superior HealthPlan	FUNCTIONAL AREA: Pharmacy, Medical Directors
EFFECTIVE DATE: 01/24/2022	PRODUCT(S): STAR, STAR Health, STAR Kids, Star Plus, CHIP, CHIP Perinate
REVIEWED/REVISED DATE: 01/24/2022, 01/24/2023, 03/28/2023, 04/28/2023	
REGULATOR MOST RECENT APPROVAL DATE(S): N/A	

POLICY STATEMENT:

The purpose of this Clinical Policy is to provide a guide to medical necessity reviews for Enzyme Replacement Therapy (Fabrazyme, Lumizyme, Nexviazyme, Brineura, Vimizim, Naglazyme, Elaprase, Cerezyme, Aldurazyme, Ceprotin, Kanuma, Elelyso, Vpriv, Xenpozyme).

PURPOSE:

It is the policy of Superior HealthPlan to follow state guidance for medical necessity review of Enzyme Replacement Therapy (Fabrazyme, Lumizyme, Nexviazyme, Brineura, Vimizim, Naglazyme, Elaprase, Cerezyme, Aldurazyme, Ceprotin, Kanuma, Elelyso, Vpriv, Xenpozyme).

Consistent with the regulation at 42 CFR Section 438.210 and 42 CFR Section 457.1230(d), services covered under managed care contracts, including clinician-administered drugs, must be furnished in an amount, duration, and scope that is no less than the amount, duration, and scope for the same services specified in the state plan.

While MCOs may place appropriate limits on drugs, MCOs may not use a standard for determining medical necessity that is more restrictive than what is used in the state plan, i.e., developed by the Vendor Drug Program. For example, if a member is denied a clinician administered drug in managed care because of the MCO's prior authorization criteria, but would have received the drug under the criteria specified in the state plan, then the MCO's prior authorization criteria would violate the amount, duration, and scope requirements cited above. HHSC intends to amend the Managed Care Contracts at the next opportunity to include this requirement. This same standard applies to CHIP formulary and CAD coverage.

Refer to the Outpatient Drug Services Handbook of the Texas Medicaid Provider Procedure Manual for more details on the clinical policy and prior authorization requirements.

It is the policy of Superior HealthPlan to follow state guidance for medical necessity review of cerliponase alfa (Brineura®). This medication is a pass through drug and should follow state guidance for medical necessity review for Medicaid/CHIP due to the manner in which it is reimbursed. All determinations will be performed by a Superior Medical Director. A pharmacy clinician will review the prior authorization request and make a recommendation to the Medical Director but will not make the ultimate determination on any case.

SCOPE:

This policy applies to Superior HealthPlan Pharmacy Department, Medical Directors

DEFINITIONS: N/A

POLICY:

It is the policy of Superior HealthPlan to follow state guidance for medical necessity review of Enzyme Replacement Therapy (Fabrazyme, Lumizyme, Nexviazyme, Brineura, Vimizim, Naglazyme, Elaprase, Cerezyme, Aldurazyme, Ceprotin, Kanuma, Elelyso, Vpriv, Xenpozyme).

PROCEDURE:

Provider must submit documentation (which may include office chart notes and lab results) supporting that member has met all approval criteria.

I. Initial Approval Criteria

A. FDA Approved Indications

1. Prior authorization approval for any of the enzyme replacement therapy listed below will be considered when the following criteria are met (a and b):
 - a. A request for the specific enzyme replacement therapy
 - b. The laboratory evidence of the enzyme deficiency. See Appendix A with for examples of applicable diagnostic confirming labs.
2. Specific for olipudase alfa-rpcp (Xenpozyme) requests, in addition to 1.a. and b. (above), client must also meet the following criteria:
 - a. Client has a diagnosis of non-central nervous system manifestations of acid sphingomyelinase deficiency (ASMD) in adult and pediatric patients (diagnosis code: E75.241 and E75.244)
 - b. Verification of pregnancy status for female clients of reproductive potential prior to therapy initiation
 - c. Documentation of baseline transaminase assessment prior to treatment with Xenpozyme
 - d. Prescriber attestation to check alanine transaminase (ALT) and aspartate aminotransferase (AST) levels within one month prior to initiation of Olipudase alfa-rpcp, within 72 hours prior to any infusion during dose escalation, or prior to the next scheduled Olipudase alfa-rpcp infusion upon resuming treatment following a missed dose
3. Listed below are the FDA approved indications, age restrictions and diagnosis codes (as applicable):
 - a. Agalsidase beta (Fabrazyme) is indicated in clients age 2 years and older with Fabry disease. Diagnosis code: E7521.
 - b. Alglucosidase alfa (Lumizyme) is indicated for clients with Pompe disease (GAA deficiency). Diagnosis code: E7402.
 - c. Avalglucosidase alfa-ngpt (Nexviazyme) is indicated for clients age 1 year and older with late onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency). Diagnosis code: E7402.
 - d. Cerliponase alfa (Brineura) is indicated to slow the loss of ambulation in symptomatic pediatric clients age 3 years and older with late infantile neuronal ceroid lipofuscinosis type 2 (CLN2). Diagnosis code: E754.
A Medical Director is required to review and approve or deny all requests for Brineura
 - e. Elosulfase alfa (Vimizim) is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme indicated for clients age 5 years and older with mucopolysaccharidosis type IVA. Diagnosis code: E76210.
 - f. Galsulfase (Naglazyme) is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme indicated for clients with mucopolysaccharidosis VI (MPS VI; Maroteaux-Lamy syndrome). Diagnosis codes: E7629.
 - g. Idursulfase (Elaprase) is a hydrolytic lysosomal glycosaminoglycan (GAG)-specific enzyme indicated for clients with Hunter syndrome (mucopolysaccharidosis II, MPS II). Diagnosis code: E761.
 - h. Imiglucerase (Cerezyme) is indicated for long-term enzyme replacement therapy for clients age 2 years and older with a confirmed diagnosis of Type 1 Gaucher disease (diagnosis code: E7522) that results in one or more of the following conditions:
 - i. Anemia
 - ii. Thrombocytopenia
 - iii. Bone disease
 - iv. Hepatomegaly or splenomegaly
 - i. Laronidase (Aldurazyme) is indicated in clients with Hurler and Hurler-Scheie forms of mucopolysaccharidosis I (MPS I) and clients with the Scheie form who have moderate to severe symptoms. Diagnosis codes: E7601, E7602, and E7603.
 - j. Protein C Concentrate, human (Ceprotrin) is indicated in pediatric and adult clients with severe congenital protein C deficiency for the prevention and treatment of venous thrombosis and purpura fulminans. Diagnosis code: D6859.
 - k. Sebelipase alfa (Kanuma) is indicated for the treatment of pediatric and adult clients with a diagnosis of Lysosomal Acid Lipase (LAL) deficiency. Diagnosis code: E755.
 - l. Taliglucerase alfa (Elelyso) is indicated for long-term enzyme replacement therapy for adult clients with a diagnosis of Type 1 Gaucher disease. Diagnosis code: E7522.

- m. Velaglucerase alfa (Vpriv) is indicated for long-term replacement therapy for pediatric and adult clients with Type 1 Gaucher disease. Diagnosis code: E7522.
- n. Olipudase alfa-rpcp (Xenpozyme) is indicated for treating non-central nervous system manifestations of acid sphingomyelinase deficiency (ASMD) in adult and pediatric patients. Diagnosis code: E75.241 and E75.244.

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to the off-label use policy for the relevant line of business: CP.PMN.53 for Medicaid

Appendix A.

Enzyme replacement therapy	Indication	Diagnostic Lab Examples
Agalsidase beta (Fabrazyme)	Fabry disease	Enzyme assay demonstrating a deficiency of alpha-galactosidase activity OR DNA testing
Alglucosidase alfa (Lumizyme)	Pompe disease	Enzyme assay confirming low GAA activity OR DNA testing
Avalglucosidase alfa-ngpt (Nexviazyme)	Pompe disease	Enzyme assay confirming low GAA activity OR DNA testing
Cerliponase alfa (Brineura)	Late infantile neuronal ceroid lipofuscinosis type 2 (CLN2)	TPP1 enzyme activity test demonstrating deficient TPP1 enzyme activity in leukocytes AND Identification of 2 pathogenic mutations in trans in the TPP1/CLN2 gene
Elosulfase alfa (Vimizim)	Mucopolysaccharidosis type IVA	Enzyme assay demonstrating a deficiency of N-acetylgalactosamine-6-sulfatase activity OR DNA testing
Galsulfase (Naglazyme)	Mucopolysaccharidosis VI (MPS VI; Maroteaux-Lamy syndrome)	Enzyme assay demonstrating a deficiency in N-acetylgalactosamine 4-sulfatase (arylsulfatase B) activity OR DNA testing
Idursulfase (Elaprase)	Hunter syndrome (Mucopolysaccharidosis II, MPS II)	Enzyme assay demonstrating a deficiency of iduronate 2-sulfatase activity OR DNA testing
Imiglucerase (Cerezyme)	Type 1 Gaucher disease	Enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) activity OR DNA testing
Laronidase (Aldurazyme)	Mucopolysaccharidosis I: Hurler, Hurler-Scheie, and Scheie Forms	Enzyme assay demonstrating deficiency of alpha-L-iduronidase activity OR DNA testing
Protein C Concentrate, human (Ceprotin)	congenital Protein C deficiency	Lab result confirming low protein C activity (due to low protein C levels or function or both)
Sebelipase alfa (Kanuma)	Lysosomal Acid Lipase (LAL) deficiency	Enzyme assay demonstrating a deficiency of LAL activity OR Lipase A - Lysosomal acid type (LIPA) gene mutation
Taliglucerase alfa (Elelyso)	Type 1 Gaucher disease	Enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) activity OR DNA testing
Velaglucerase alfa (Vpriv)	Type 1 Gaucher disease	Enzyme assay demonstrating a deficiency of beta-glucocerebrosidase (glucosidase) activity OR DNA testing
Olipudase alfa-rpcp (Xenpozyme)	Acid Sphingomyelinase Deficiency (ASMD)	Enzyme assay confirming decreased acid sphingomyelinase (ASM) enzyme activity OR DNA testing

REFERENCES:

1. Outpatient Drug Services Handbook of the Texas Medicaid Provider Procedure Manual, Accessed January 2022

ATTACHMENTS: N/A

ROLES & RESPONSIBILITIES: N/A

REGULATORY REPORTING REQUIREMENTS: N/A

REVISION LOG

REVISION TYPE	REVISION SUMMARY	DATE APPROVED & PUBLISHED
New Policy		01/24/2022
Ad Hoc Review	Annual Review: Corrected chemical drug name: Avalglucosidase alfa-ngpt (Nexviazyme). Verbiage change: 'age "xxx" years and older' for consistency throughout policy.	01/24/2023
Ad Hoc Review	Formatted to new template. Per HHSC guidance, added prior auth requirements for olipudase alfa-rpcp (Xenpozyme): The client has a diagnosis of acid sphingomyelinase deficiency (diagnosis code E75.241 and E75.244); Verification of pregnancy status for female clients of reproductive potential prior to therapy initiation; Documentation of baseline transaminase assessment prior to treatment with Xenpozyme; Prescriber attestation to check alanine transaminase (ALT) and aspartate aminotransferase (AST) levels before initiation of therapy, during any dose escalations of therapy and before any missed doses.	03/28/2023
Ad Hoc Review	Updated verbiage in Section I. A. 2.a. and 2.d. to align with verbiage in CAD Manual	04/28/2023

POLICY AND PROCEDURE APPROVAL

Karen Tadlock, V.P., Pharmacy Operations

Approval on file

Dr. David Harmon, Sr. V.P., Chief Medical Officer

Approval on file

Pharmacy & Therapeutics Committee:

Approval on file

The electronic approval retained in RSA Archer, the Company's P&P management software, is considered equivalent to a signature.