Policy and Procedure

DEPARTMENT: Pharmacy, Medical Directors	**DOCUMENT NAME:** Enzyme Replacement Therapy (Fabrazyme, Lumizyme, Nexviazyme, Brineura, Vimizim, Naglazyme, Elaprase, Cerezyme, Aldurazyme, Ceprotin, Kanuma, Elelyso, Vpriv)
PAGE: 1 of 7	**REPLACES DOCUMENT:**
APPROVED DATE:	**RETIRED:**
EFFECTIVE DATE: 1/24/2022	**REVIEWED/REVISED:** 1/24/2022
PRODUCT TYPE: Star, Star Health, Star Kids, Star Plus, Chip, Chip Perinate	**REFERENCE NUMBER:** TX.PHAR.104

SCOPE:
Superior HealthPlan Pharmacy Department, Medical Directors

PURPOSE:
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.

BACKGROUND:

Description:

Enzyme replacement therapy (ERT) is a medical treatment that replaces a specific enzyme deficient or absent in the body

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. 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. Alglucosidase alfa-ngpt (Nexviazyme) is indicated for clients who are one year of age 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 3 years of age 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**

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 and above 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 (Ceprotin) 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.

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

Alglucosidase 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

REFERENCES:

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

ATTACHMENTS:

REVISION LOG

REVISION	DATE
New Policy	1/24/2022

Policy and Procedure

DEPARTMENT: Pharmacy, Medical Directors	**DOCUMENT NAME:** Enzyme Replacement Therapy (Fabrazyme, Lumizyme, Nexviazyme, Brineura, Vimizim, Naglazyme, Elaprase, Cerezyme, Aldurazyme, Ceprotin, Kanuma, Elelyso, Vpriv)
PAGE: 7 of 7	**REPLACES DOCUMENT:**
APPROVED DATE:	**RETIRED:**
EFFECTIVE DATE: 1/24/2022	**REVIEWED/REVISED:** 1/24/2022
PRODUCT TYPE: Star, Star Health, Star Kids, Star Plus, Chip, Chip Perinate	**REFERENCE NUMBER:** TX.PHAR.104

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

NOTE: The electronic approval retained in RSA Archer, Centene's P&P management software, is considered equivalent to a physical signature.