Mass General Brigham
Health Plan

Enzyme and Metabolic Disorder Therapies
Aldurazyme (laronidase)
Cerezyme (imiglucerase)
Elaprase (idursulfase)
Elelyso (taliglucerase alfa)
Elfabrio (pegunigalsidase alfa-iwxj)
Fabrazyme (agalsidase beta)
Kanuma (sebelipase alfa)
Lamzede (velmanase alfa-tycv)
Lumizyme (alglucosidase alfa)
Mepsevii (vestronidase alfa-vjbk)
Naglazyme (galsulfase)
Nexviazyme (avalglucosidase alfa-ngpt)
Nulibry (fosdenopterin)
Revcovi (elapegademase-lvlr)
Ryplazim (plasminogen, human-tvmh)
Vimizim (elosulfase alfa)
Vpriv (velaglucerase alfa)
Xenpozyme (olipudase alfa-rpcp)
Effective 01/01/2026

Plan	☒ MassHealth UPPL ☐Commercial/Exchange	**Program Type**	☒ Prior Authorization ☐ Quantity Limit ☐ Step Therapy
Benefit	☐ Pharmacy Benefit ☒ Medical Benefit		
Specialty Limitations	N/A		
Contact Information	**Medical Benefit** **Pharmacy Benefit**	Phone: 833-895-2611 Phone: 800-711-4555	Fax: 888-656-6671 Fax: 844-403-1029
Exceptions	Elfabrio, Fabrazyme, Revcovi, Ryplazim are also available on the pharmacy benefit. Please see the MassHealth Drug List for coverage and criteria. Additional agents from this class are available through the pharmacy benefit. Please see the MassHealth Drug List for coverage and criteria.		

Overview

Lysosomal storage disorders are caused by a deficiency or absence of required enzymes. The consequence is an accumulation of compounds that are normally degraded causing cell and organ dysfunction. Prior to the development of enzyme replacement therapy (ERT), management of these conditions mainly consisted of supportive care and treatment of the complications. Today a number of exogenously supplied enzymes are available for lysosomal storage disorders such as Gaucher Type I disease, Fabry disease, mucopolysaccharidosis Type I, II, and VI and Pompe disease.

Coverage Guidelines

Authorization may be reviewed on a case by case basis for members who are new to the plan currently receiving treatment with requested medication excluding when the product is obtained as samples or via manufacturer's patient assistance programs.

OR
Authorization will be granted when all the following criteria has been met:

Aldurazyme (laronidase)
1. Diagnosis of Mucopolysaccharidosis I (MPS I)
2. Results from genetic testing showing mutations in IDUA gene or an enzyme assay test showing reduced lysosomal alpha-L-iduronidase activity in peripheral blood leukocytes, plasma, or cultured fibroblasts
3. Prescriber is a specialist in genetic or metabolic diseases or consultation notes from a specialist are provided
4. Member's current weight *(use to verify correct dosing)*

Cerezyme (imiglucerase)
Vpriv (velaglucerase alfa*)*
1. Diagnosis of Gaucher disease (Type I)
2. Results from genetic test confirming mutation in GBA gene or an enzyme assay test showing reduced activity of the enzyme glucocerebrosidase
3. Prescriber is a specialist in genetic or metabolic diseases or consultation notes from a specialist are provided
4. Member's current weight *(use to verify correct dosing)*

Elaprase (idursulfase)
1. Diagnosis of Hunter Syndrome (Mucopolysaccharidosis II)
2. Results from genetic testing confirming mutation in IDS gene or iduronate-2-sulfatase assay test showing reduced or absent activity in the serum, white blood cells, or fibroblasts
3. Prescriber is a specialist in genetic or metabolic diseases or consultation notes from a specialist are provided
4. Member's current weight *(use to verify correct dosing)*

Elelyso (taliglucerase alfa)
1. Diagnosis of Gaucher disease (Type I)
2. Results from genetic test confirming mutation in GBA gene or an enzyme assay test showing reduced activity of the enzyme glucocerebrosidase
3. Prescriber is a specialist in genetic or metabolic diseases or consultation notes from a specialist are provided
4. Member is ≥ 4 years of age
5. Member's current weight *(use to verify correct dosing)*

Elfabrio (pegunigalsidase alfa-iwxj)
Fabrazyme (agalsidase beta)
1. Diagnosis of Fabry disease
2. **ONE** of the following confirming diagnosis:
    a. Results from an enzyme assay test showing reduced or absent α-GAL enzyme activity in plasma, leukocytes, tears, or biopsied tissue
    b. Genetic testing confirming mutation in GAL gene
    c. Biomarker demonstrating an increase in Gb3 concentration
3. Prescriber is a specialist in genetic or metabolic diseases or consultation notes from a specialist are provided
4. Member's current weight *(use to verify correct dosing)*

5. For Elfabrio (pegunigalsidase alfa-iwxj), inadequate response, adverse reaction, or contraindication to Fabrazyme (agalsidase beta)

Kanuma (sebelipase alfa)
1. Diagnosis of lysosomal acid lipase deficiency
2. **ONE** of the following:
    a. Lab assay documenting low lysosomal acid lipase activity
    b. Genetic testing confirming full or partial loss of LAL gene
3. Prescriber is a specialist in genetic or metabolic diseases or consultation notes from a specialist are provided
4. Member's current weight *(use to verify correct dosing)*

Lamzede (velmanase alfa-tycv)
1. Diagnosis of alpha-mannosidosis
2. Member is ≥ 3 years of age
3. Prescriber is a specialist in genetic or metabolic diseases or consult notes from a specialist are provided
4. Copy of a genetic test confirming diagnosis of alpha-mannosidosis (e.g., mutation of MAN2B1 gene)
5. Baseline measurements for **ALL** of the following tests:
    a. serum oligosaccharides
    b. forced vital capacity
    c. **ONE** of the following motor function tests:
        i. 3-minute stair climb test
        ii. 6-minute walk test
6. Member's current weight *(use to verify correct dosing)*

Lumizyme (alglucosidase alfa)*
1. Diagnosis of Pompe Disease
2. **ONE** of the following confirming diagnosis:
    a. Results from GAA assay test showing reduced or absent activity from cultured skin fibroblasts
    b. lymphocyte testing
    c. blood spot assay
    d. genetic testing confirming mutation in GAA gene
3. Prescriber is a specialist in genetic or metabolic diseases or consultation notes from a specialist are provided
4. Member's current weight *(use to verify correct dosing)*

* Lumizyme and Nexviazyme should not be used concurrently

Mepsevii (vestronidase alfa-vjbk)
1. Diagnosis of Mucopolysaccharidosis VII (MPS VII, Sly syndrome)
2. Results from genetic testing showing mutations in the beta glucuronidase gene
3. Prescriber is a specialist in genetic or metabolic diseases or provides documentation of a consultation notes from a specialist are provided
4. Member's current weight *(use to verify correct dosing)*

Naglazyme (galsulfase)
1. Diagnosis of Mucopolysaccharidosis VI (MPS VI)

2. Results from an enzyme assay test showing reduced arylsulfatase B (ASB) enzyme activity in leukocytes or fibroblasts along with elevated urine glycosaminoglycan (GAG) levels
3. Prescriber is a specialist in genetic or metabolic diseases or provides documentation of a consultation notes from a specialist are provided
4. Member's current weight *(use to verify correct dosing)*

Nexviazyme (avalglucosidase alfa-ngpt) *
1. Diagnosis of late-onset Pompe Disease
2. **ONE** of the following confirming diagnosis:
    a. results from GAA assay test showing reduced or absent activity from cultured skin fibroblasts
    b. lymphocyte testing
    c. blood spot assay
    d. genetic testing confirming mutation in GAA gene
3. Member is ≥ one year of age
4. Prescriber is a specialist in genetic or metabolic diseases or consult notes from a specialist are provided.
5. Member's current weight *(use to verify correct dosing)*

* Lumizyme and Nexviazyme should not be used concurrently

Nulibry (fosdenopterin)
1. Diagnosis of molybdenum cofactor deficiency (MoCD) Type A confirmed by genetic testing
2. Prescriber is a specialist in genetic or metabolic diseases or consultation notes from a specialist are provided
3. Appropriate dosing
4. Member's current weight *(use to verify correct dosing)*

Revcovi (elapegademase-lvlr)
1. Diagnosis of adenosine deaminase severe combined immunodeficiency (ADA-SCID)
2. Laboratory results documenting **ONE** of the following:
    a. Absent ADA enzymatic activity in lysed erythrocytes
    b. Elevated levels of adenosine and deoxyadenosine in the urine and plasma
    c. A marked increase in deoxyadenosine triphosphate (dATP) levels in erythrocyte lysates
    d. A significant decrease in ATP concentration in red blood cells
    e. Absent or extremely low levels of N adenosylhomocysteine hydrolase in red blood cells
    f. Severe T cell deficiency manifested by lymphopenia and poor T cell responses to mitogens and antigens
    g. Absent thymic shadow on chest radiograph
3. Prescriber is a specialist in genetic or metabolic diseases or consultation notes from a specialist are provided
4. Member's current weight *(use to verify correct dosing)*

Ryplazim (plasminogen, human-tvmh)
1. Diagnosis of PLGD type 1
2. History of lesions (external and/or internal) and symptoms consistent with a diagnosis of PLGD type 1 (e.g., ligneous conjunctivitis, ligneous gingivitis or gingival overgrowth, vision abnormalities, respiratory distress and/or obstruction, abnormal wound healing)
3. Baseline plasminogen activity level ≤45%
4. ONE of the following:
    a. Results from genetic testing showing mutations in PLG gene

    b. Member has plasminogen antigen levels ≤9 mg/dL
5. Requested dose is ≤ 6.6 mg/kg every two to four days

Vimizim (elosulfase alfa)
1. Diagnosis of Mucopolysaccharidosis IVA (Morquio A syndrome)
2. Member is ≥ 5 years of age
3. Results from an enzyme assay test showing reduced N-acetylgalactosamine-6-sulfatase activity in blood and/or skin cells
4. Prescriber is a specialist in genetic or metabolic diseases or consultation notes from a specialist are provided
5. Member's current weight *(use to verify correct dosing)*

Xenpozyme (olipudase alfa-rpcp)
1. Diagnosis of non-central nervous system manifestations of acid sphingomyelinase deficiency (ASMD) type B, or ASMD type A/B
2. Prescriber is a specialist (e.g., medical geneticist or a specialist familiar with lysosomal storage disorders) or consultation notes from a specialist are provided
3. **ONE** of the following:
    a. For members ≥ 18 years of age, **BOTH** of the following:
        i. DLco ≤ 70% of predicted normal value
        ii. Spleen volume ≥ 6 MN
    b. For members < 18 years of age, spleen volume ≥ 5 MN
4. Member does **NOT** have acute or rapidly progressive neurologic abnormalities
5. **BOTH** of the following:
    a. Member does **NOT** require invasive ventilatory support
    b. Member does **NOT** require noninvasive ventilatory support while awake for > 12 hours a day
6. Member's current weight *(use to verify correct dosing)*
7. Appropriate dosing

<u>Continuation of Therapy</u>
Lamzede:
Prescriber provides documentation of **ONE** of the following:
1. Current tests (within the past 3 months) documenting positive response to therapy for **ALL** of the following tests:
    a. serum oligosaccharides
    b. forced vital capacity
    c. **ONE** of the following motor function tests:
        i. 3-minute stair climb test
        ii. 6-minute walk test
2. Medical necessity for continuing therapy (e.g., disease stabilization or a reduction in normal motor decline)

Ryplazim and **Nulibry**: Reauthorization will require physician documentation of a positive response to therapy or clinical rationale for continued use if dosing is appropriate based on updated member's weight where applicable.

Xenpozyme: Prescriber provides documentation of **BOTH** of the following:
1. Improvement from baseline in DLco and spleen volume

2. Updated member weight

All other drugs: Resubmission by prescriber will infer a positive response to therapy.

Limitations

1. Initial approvals will be granted for the following:
    a. Lamzede, Nulibry, Xenpozyme: 6 months
    b. Ryplazim: 24 weeks
    c. All other agents: 1 year
2. Reauthorizations will be granted for the following:
    a. Xenpozyme – improvement in DLco and spleen volume: 6 months
    b. All other agents: 1 year
3. Members who are stable on Nulibry (fosdenopterin) must meet both the initial and reauthorization criteria for approval.

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Review History

02/08/2023 - Reviewed and created for Feb P&T; matched MH UPPL. Created criteria to be in compliance with Masshealth unified formulary requirements (Effective 4/1/23).
05/10/23 – Reviewed and updated for P&T. Added new drug, Ryplazim (plasminogen, human-tvmh), to policy. Added initial and reauthorization criteria for Xenpozyme for the treatment of non-central nervous system manifestations of acid sphingomyelinase deficiency (ASMD) in adult and pediatric patients. A noted was added for Fabrazyme to clarify that Gb3 may be referred to as GL-3. References updated. Effective 6/5/23
06/14/23 – Reviewed and updated for P&T. Removed preferred product requirement from Palynziq and Nexviazyme for requests through MB. Effective 6/30/23
07/12/23 – Reviewed and updated for P&T. Added new drug, Lamzede, to policy requiring PA under MB. Brand preferred and mandatory generic language was added under Limitations. Effective 7/31/23
11/15/23 – Reviewed and updated for P&T. Policy update to restrict Nexviazyme to medical billing. Genetic testing requirement was removed from criteria for Kuvan and Palynziq. Effective 12/4/23
12/13/23 – Reviewed and updated for P&T. Elfabrio, Joenja, and Olpruva added to criteria requiring PA. Effective 1/2/24

05/15/25 – Reviewed and updated for P&T. Updated formatting and references. Removed drugs that are managed through pharmacy benefit as it is available on MHDL. Medical benefit drugs remain. Effective 6/1/25
11/12/25 – Reviewed and updated for P&T. Removed Palynziq from policy and allowed to remain on MB in preparation for Prime implementation. Effective 1/1/26