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Enzyme Replacement Therapies Clinical Guideline for Home Intravenous Therapy

Section: Clinical Guideline
Policy ID: CG042
Effective: 10/20/2022
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Revised: 10/6/25, 4/13/26, 8/7/26
Approved by, Title and Date Approved: David Benedict, President, 10/20/22, 10/6/25, 4/13/26, 8/7/26

I. BACKGROUND

Enzyme replacement therapy (ERT) is treatment for patients with specific enzyme deficiencies or enzyme malfunctions. The most common method of enzyme replacement therapy is through intravenous infusions. Some disease states requiring ERT include lysosomal storage diseases (e.g., Lysosomal Acid Lipase deficiency), Fabry disease, Gaucher disease, and Pompe (glycogen storage) disease. ERT is not a cure for these disease states, and patients require lifelong therapy. The following outlines the procedures for servicing patients in need of enzyme replacement therapy in the home setting.

II. PATIENT ACCEPTANCE CRITERIA

A. All candidates for home care service must be evaluated by the clinician(s) and deemed appropriate to receive home care based upon admission criteria.

B. The decision to administer a first dose in the home by a field nurse will be determined on a case-by-case basis and reviewed by nursing and pharmacy management. The following criteria will be evaluated:

1. Prescriber preference
2. Allergy profile
3. Age
4. Other relevant social and/or medical history

C. Physician orders for ERT must include:

1. Patient weight
2. Drug and dose (including weight-dosing)
3. Route of administration
4. Frequency of administration
5. Emergency medications per protocol
6. Orders for pre-medications
7. Line care protocol
8. Routine lab monitoring, if applicable

D. Baseline labs or tests prior to starting therapy

1. Verify pregnancy status in female patients of childbearing age prior to starting:

a. Cipaglucosidase alfa-atga (Pombiliti) in combination with miglustat (Opfolda) or
b. Olipudase (Xenpozyme).

2. Baseline ALT and AST within one month prior to initiation of olipudase

E. Dispensing pharmacy treatment protocol for anaphylaxis will be instituted unless a more comprehensive patient-specific orders are provided by physician. See Appendix A (Nursing CarepathRx policy on *Management of Allergic/Anaphylactic Reactions*).

F. Summary of ERT Therapies:

Refer to each individual ERT appendix for specifics in the pharmacology overview and nursing procedures.

Brand Name	Generic Name	Indications	Drug Specific Appendix
Aldurazyme	Laranidase	Mucopolysaccharidosis I (MPS I)	B
Elaprase	Idursulfase	Mucopolysaccharidoses II (MPS II)	C
Vimizim	Elosulfase alpha	Mucopolysaccharidoses IVA (MPS IVA)	D
Naglazyme	Galsulfase	Mucopolysaccharidoses VI (MPS VI)	E
MEPSEVII	Vestronidase alpha	Mucopolysaccharidoses VII (MPS VII)	F
Kanuma	Sebelipase alfa	Lysosomal Acid Lipase deficiency (LAL)	G
Cerezyme	Imiglucerase	Gaucher Disease	H
VPRIV	Velaglucerase alpha	Gaucher Disease	I
Elelyso	Taliglucerase alpha	Gaucher Disease	J
Lumizyme	Alglucosidase alpha	Pompe Disease	K
Nexviazyme	Avalglucosidase alfa-ngpt	Pompe Disease	L
Fabrazyme	Agalsidase beta	Fabry Disease	M

Xenpozyme	Olipudase alfa	Acid Sphingomyelinase Deficiency	N
Pombiliti	Cipaglucosidase alfa	Pompe Disease	O
Elfabrio	Pegunigalsidase alfa-iwxj	Fabry Disease	P
Avlayah	Tividenofusp alfa	Mucopolysaccharidosis II (MPS II)	Q

III. ADMINISTRATIVE GUIDELINES

A. Administration: IV infusions should be given immediately after reconstitution and dilution. Begin infusion within 4 hours of preparation per current USP Immediate-Use Guidelines.

B. All enzyme replacement therapies require **0.2micron filter for administration.**

C. Do not co-administer ERT with other products in the same IV line.

IV. CLINICAL MONITORING

A. Prior to therapy:

1. Verify pregnancy status in female patients of childbearing age and counsel on the risks of getting pregnant prior to starting the following enzyme replacement therapies:
    a. Cipaglucosidase alfa-atga in combination with miglustat
    b. Olipudase
2. Olipudase:
    a. Baseline ALT and AST within one month prior to initiation.
    b. Confirm BMI and weight-based dosing
    c. Verify the patient has tolerated dose escalation phase in a controlled setting prior to transitioning to home infusion
3. Tividenofusp alfa:
    a. Verify the patient has tolerated dose escalation phase in a controlled setting prior to transitioning to home infusion
4. Consider the risks and benefits of treatment with sebelipase in patients with known systemic hypersensitivity reactions to eggs or egg products as it is manufactured in egg whites.
5. Baseline anti-drug antibody (ADA) level

B. During therapy

1. Monitor for anaphylaxis, hypersensitivity, and infusion related reactions with all enzyme replacement therapies.
2. Assess for respiratory illness as this can increase the risk for infusion reactions and acute respiratory complications during the ERT infusion.

3. Patients susceptible to fluid overload may be at increased risk for serious exacerbation of their cardiac or respiratory status during infusions. Consider a decreased total infusion volume and infusion rate when administering ERT.
4. Monitor patient weight at least every 6 months or more frequently as needed to determine the ERT appropriate dose.
5. Monitoring for patients with Mucopolysaccharidoses:
    a. Pulmonary function (including sleep apnea)
    b. Walking capacity
    c. Spinal or cervical cord compression (SCC) is a known and serious complication of MPS IVA and may occur as part of the natural history of the disease, Monitor patients with back pain, paralysis of limbs below the level of compression, urinary and fecal incontinence.
6. Monitoring in patients with LAL deficiency:
    a. Liver function
    b. Dyslipidemia
    c. Vomiting
    d. Poor weight gain
7. Monitoring in patients with Gaucher disease:
    a. Hemoglobin and platelet counts
    b. Improvement in bone disease
    c. Monitor liver and spleen size in patients with Type I Gaucher disease
8. Monitoring in patients with Pompe Disease:
    a. Walking capacity
    b. Liver enzymes
    c. Systemic immune complex-mediated reactions involving skin and other organs
    d. Anti-drug antibody levels as clinically indicated
9. Monitoring in patients with Fabry disease
    a. Numbness, tingling, burning in hands in feet
    b. Heat or cold intolerance
    c. Pain during physical activity
    d. Dizziness
    e. Flu-like symptoms
    f. Corneal opacities
    g. Kidney function
10. Monitoring in patients with Acid sphingomyelinase deficiency being treated with olipudase:
    a. Monitor ALT and AST within 72 hours prior to any infusion during dose escalation, or prior to the next scheduled infusion upon resuming treatment following a missed dose.
    b. Thrombocytopenia
    c. Pulmonary function
    d. Growth delays in pediatric patients

Refer to manufacturer's full Prescribing Information for most up to date information

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APPENDIX A: ANAPHYLAXIS KIT INTRUCTIONS

Emergency Medication After Your Infusion

Please call your pharmacy if you have any questions or concerns. In the event of an emergency, always call 911.

Your nurse will tell you when you need to use Emergency Medications.
The epinephrine dose will be noted on the Anaphylaxis Protocol included with your kit.

Start with a clean work surface and clean hands.

Open the supply bag labeled Anaphylaxis Kit Contents.

You will need:

1. **Bag containing Pills** (2 Acetaminophen and 2 Diphenhydramine)
2. **Bag containing Alcohol Prep Pads**
3. **Bag labeled IM Epinephrine**

All other contents will not be needed.

Open the IM Epinephrine Bag

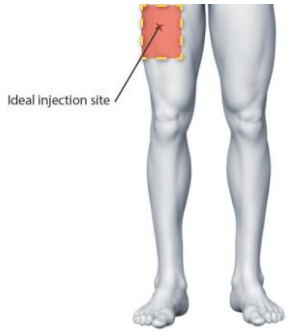
1. **Remove 1 of each item**
    a. 1 -syringe
    b. 1 – brown labeled filter needle (BD Filter Needle)- **for ampule use only**
    c. 1 – black labeled safety needle (Magellan Hypodermic Safety Needle 22G x 1”)
    d. 1 ampule of epinephrine

Prepare IM (intramuscular) injection of Epinephrine:

1. **Attach the brown filtered needle to syringe**
    a. Be careful to not touch the tip of the syringe or the needle.
2. Using an **alcohol swab, wipe the neck of the epinephrine ampule.**
3. Holding the ampule upright, **swirl and flick the ampule until all fluid flows to the bottom chamber** (the top chamber should be empty).
4. Using a new alcohol wipe, grasp the neck of the ampule and with your other hand grasp the bottom chamber of the ampule. **Quickly snap the top of the ampule off, directing the snap way from you.**
5. **Place the tip of the brown filter needle inside the ampule**. Tilting the ampule, **withdraw dose of medication into the syringe** by gently pulling back on the plunger. Be careful to not pull the plunger out of the syringe.
6. Remove the needle from the ampule and h**old the syringe upright** with the needle pointing upward. **Gently tap the side of the syringe to bring any air to the top of the syringe.**
7. **Push the air out of the syringe by gently pushing on the plunger**.
8. Replace the cap on the brown filter needle. Discard remainder in ampule.

9. **Remove the brown filter needle and place the black safety needle onto the syringe.**

Give your IM Epinephrine injection



1. **Grasp your leg muscle at the outer mid-thigh** and **cleanse the area** with a new alcohol wipe.
2. **Push the needle into your leg muscle straight** in at a 90-degree angle.
3. **Inject the medication** by depressing the plunger in a slow and steady motion.
4. **Remove the needle** and wipe the site with the alcohol wipe.
5. May repeat dose every 5 minutes (**maximum 3 doses**) if ordered per protocol.

Take the pills by mouth.

a. 2 – Acetaminophen
b. 2 – Diphenhydramine

Place all trash in the bag the pills came in and take with you when you seek medical care. **Give the bag to the nurse or EMT**, so your doctor will know what medication you already took. They will properly dispose of the syringe and needles.

Call 911 or have someone drive you to the emergency department.