TEXAS Health and Human Services

Medicaid Drug Use Criteria

Complement Inhibitor and Enzyme/Protein Replacement Therapy

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Notes: Information on indications for use or diagnosis is assumed to be unavailable. All criteria may be applied retrospectively; prospective application is indicated with an asterisk [*]. The information contained is for the convenience of the public. The Texas Health and Human Services Commission is not responsible for any errors in transmission or any errors or omissions in the document.

Medications listed in the tables and non-FDA approved indications included in these retrospective criteria are not indicative of Vendor Drug Program formulary coverage.

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1 Dosage

Complement inhibitor and enzyme/protein replacement therapy is FDA-approved for conditions characterized by enzyme deficiencies such as: [1-39]

- adenosine deaminase (ADA) deficiency in severe combined immunodeficiency (SCID) patients (elapegademase)
- atypical hemolytic uremic syndrome (eculizumab, ravulizumab)
- congenital sucrase isomaltase deficiency (CSID) (sacrosidase)
- Fabry disease (agalsidase beta)
- Gaucher disease (imiglucerase, taliglucerase alfa, velaglucerase alfa)
- hereditary angioedema (HAE) (C1 esterase inhibitor, ecallantide, icatibant; kallikrein inhibitor, lanadelumab)
- hypophosphatasia (asfotase)
- lysosomal acid lipase (LAL) deficiency (sebelipase alfa)
- mucopolysaccharidoses (MPS) [Maroteaux-Lamy syndrome (MPS VI) - galsulfase; Hunter syndrome (MPS II) – idursulfase; Hurler and Hurler-Scheie forms of MPS 1 – laronidase; Morquio A syndrome (MPS IVA) – elosulfase; Sly syndrome (MPS VII – vestronidase-alfa-vjbk)]
- myasthenia gravis (eculizumab)
- neuromyelitis optica spectrum disorder (eculizumab)
- paroxysmal nocturnal hemoglobinuria (eculizumab, ravulizumab)
- Pompe disease (alglucosidase alfa)
- severe congenital protein C deficiency (protein C concentrate)

1.1 Adults

Recommended doses for complement inhibitor as well as enzyme/protein replacement therapy FDA-approved for use in adults are summarized in **Tables 1-3.** Patient profiles containing doses exceeding maximum recommendations will be reviewed.

Table 1. Adult Complement Inhibitor Maximum Dosages[1-13]

Treatment Indication	Drug Name	Dosage Form / Strength	Maximum Recommended Dosage
Hereditary angioedema (HAE) treatment	C1 esterase inhibitor, human (Berinert®)	500 international unit (IU) single-use vial for reconstitution	20 IU/kg by IV injection as a single dose
HAE attacks, routine prevention	C1 esterase inhibitor, human (Cinryze®)	500 unit single-use vial for reconstitution	1000 U by IV infusion every 3-4 days+

Treatment Indication	Drug Name	Dosage Form / Strength	Maximum Recommended Dosage
HAE attacks, routine prevention	C1 esterase inhibitor, human (Haegarda®)	2000, 3000 IU single-use vials for reconstitution	60 IU/kg subcutaneously twice weekly (every 3 to 4 days)
HAE treatment	C1 esterase inhibitor, recombinant (Ruconest®)	2100 IU/14 mL single-use vial for reconstitution	Less than 84 kg: 50 units/kg as single IV dose up to a maximum of 4200 units per dose; may repeat x1 if attack symptoms persist in 24 hour period Greater than or equal to 84 kg: 4200 units as a single IV dose; may repeat x1 if attack symptoms persist in 24 hour period
HAE treatment	ecallantide (Kalbitor®)	10 mg/ml single-use vials x 3	30 mg subcutaneously as three separate 10 mg injections; may repeat x1 in 24 hour period if attack symptoms persist
Atypical hemolytic uremic syndrome	eculizumab (Soliris®)	300 mg/30 mL single-dose vial	1200 mg as IV infusion over 35 minutes every two weeks
Myesthenia gravis, generalized			1200 mg as IV infusion over 35 minutes every two weeks
Paroxysmal nocturnal hemoglobinuria			900 mg as IV infusion over 35 minutes every two weeks
Neuromyelitis optica spectrum disorder			1200 mg as IV infusion over 35 minutes every two weeks
HAE treatment	icatibant acetate (Firazyr®)	30 mg/3 mL syringe	30 mg subcutaneously as single dose; may repeat x2 with 6 hours between doses in 24 hour period if attack symptoms persist (maximum 3 doses per 24 hour period)
HAE, routine prevention	lanadelumab-flyo (Takhzyro®)	300 mg/2 mL vial	300 mg subcutaneously every 2 weeks; every 4 week dosing possible in patients well-controlled for greater than 6 months

Treatment Indication	Drug Name	Dosage Form / Strength	Maximum Recommended Dosage
Atypical hemolytic uremic syndrome^	ravulizumab (Ultomiris®)	300 mg/30 mL intravenous solution in single-dose vial	40-59 kg: 3000 mg as intravenous infusion every 8 weeks starting 2 weeks after loading dose (loading dose = 2400 mg) 60-99 kg: 3300 mg as intravenous infusion every 8 weeks starting 2 weeks after loading dose (loading dose = 2700 mg) greater than or equal to 100 kg: 3600 mg as intravenous infusion every 8 weeks starting 2 weeks after loading dose (loading dose = 3000 mg)
Paroxysmal nocturnal hemoglobinuria			40-59 kg: 3000 mg as intravenous infusion every 8 weeks starting 2 weeks after loading dose (loading dose = 2400 mg) 60-99 kg: 3300 mg as intravenous infusion every 8 weeks starting 2 weeks after loading dose (loading dose = 2700 mg) greater than 100 kg: 3600 mg as intravenous infusion every 8 weeks starting 2 weeks after loading dose (loading dose = 3000 mg)

- + in patients not responding adequately, doses up to 2500 units (not exceeding 100 u/kg) every 3 or 4 days may be utilized based on individual patient response
- ^ treat atypical hemolytic-uremic syndrome with ravulizumab for at least six months

Table 2. Adult Enzyme Replacement Therapy Maximum Dosages[10-38]

Treatment Indication	Drug Name	Dosage Form / Strength	Maximum Recommended Dosage
Fabry disease	agalsidase beta (Fabrazyme®)	5 mg, 35 mg single-use vials	1 mg/kg by intravenous (IV) infusion every 2 weeks
Pompe disease	alglucosidase alfa (Lumizyme®)	50 mg single-use vial	20 mg/kg as an IV infusion every 2 weeks

Treatment Indication	Drug Name	Dosage Form / Strength	Maximum Recommended Dosage
Adenosine deaminase severe combined immunodeficiency	elapegademase (Revcovi®)	2.4 mg/1.5 mL intramuscular solution	treatment naïve: 0.2 mg/kg as intramuscular injection twice weekly for a minimum of 12 to 24 weeks; titrate up by 0.033 mg/kg/week to maintain ADA trough greater than 30 mmol/hr/L, trough deoxyadenosine nucleotides less than 0.02 mmol/L, and maintain immune reconstitution patients transitioning from pegademase: Patients receiving pegademase doses less than or equal to 30 U/kg should receive elapegademase 0.2 mg/kg intramuscularly once weekly; patients with pegademase doses greater than 30 U/kg should receive equivalent elapegademase dose*
Mucopoly-saccharidosis (MPS) IVA (Morquio A syndrome)	elosulfase (Vimizim®)	5 mg/5 mL single-use vial	2 mg/kg by IV infusion over a minimum of 3.5 to 4.5 hours once weekly
MPS VI (Maroteaux-Lamy syndrome)	galsulfase (Naglazyme®)	5 mg/5 mL preservative-free vials	1 mg/kg by IV infusion once weekly
Hunter syndrome	idursulfase (Elaprase®)	6 mg/3 mL single-use vial	0.5 mg/kg as IV infusion once weekly
Gaucher disease, type 1 (nonneuropathic)	imiglucerase (Cerezyme®)	400 mg vials for reconstitution	60 U/kg by IV infusion over 1-2 hours every 2 weeks
MPS 1 (Hurler, Hurler-Scheie forms; Scheie form with moderate to severe symptoms)	laronidase (Aldurazyme®)	2.9 mg/5 mL single-use vials	0.58 mg/kg by IV infusion over 3-4 hours once weekly
Congenital sucrase-isomaltase deficiency (CSID)	sacrosidase (Sucraid®)	8500 international units/mL as 118 mL oral solution bottles	greater than 15 kg: 17,000 units orally mixed in 2-4 ounces of water or milk with each meal or snack
Lysosomal acid lipase (LAL) deficiency	sebelipase alfa (Kanuma®)	20 mg/10 mL single-use vial	1 mg/kg by IV infusion once every two weeks

Treatment Indication	Drug Name	Dosage Form / Strength	Maximum Recommended Dosage
Gaucher disease, type 1	taliglucerase alfa (Elelyso®)	200 unit single-use vials for reconstitution	treatment-naïve: 60 U/kg by IV infusion once every 2 weeks previously treated with imiglucerase: use same unit/kg dosage for taliglucerase that was prescribed for imiglucerase and administer every two weeks
Gaucher disease, type 1	velaglucerase alfa (Vpriv®)	400 unit single-use vials for reconstitution	treatment-naïve: 60 U/kg as an IV infusion every 2 weeks previously treated with imiglucerase: use same unit/kg dosage for velaglucerase that was prescribed for imiglucerase and administer every two weeks
MPS VII (Sly syndrome), excluding central nervous system symptoms	vestronidase alfa-vjbk (Mepsevii™)	10 mg/5 mL single-use vial	4 mg/kg as IV infusion every two weeks

- * elapegademase equivalent dose to pegademase: pegademase dose (U/kg) divided by 150

Table 3. Adult Protein Replacement Therapy Maximum Dosages[10-13, 39]

Treatment Indication	Drug Name	Dosage Form / Strength	Maximum Recommended Dosage
Severe congenital protein C deficiency (acute episode*)	protein C concentrate (Ceprotin®)	500 IU, 1000 IU single-use vial for reconstitution	100-120 IU/kg initial dose by IV infusion, followed by 60-80 IU/kg every 6 hours for 3 doses by IV infusion
Severe congenital protein C deficiency (short-term prophylaxis/ maintenance dose*)	protein C concentrate (Ceprotin®)		45-60 IU/kg every 6 to 12 hours by IV infusion
Severe congenital protein C deficiency (long-term prophylaxis*)	protein C concentrate (Ceprotin®)		45-60 IU/kg every 12 hours by IV infusion

- * maximum protein C concentrate infusion rate: 2 ml/min

1.2 Pediatrics

Elapegademase is a newer enzyme replacement therapy indicated for use in infants, children, adolescents and adults with ADA deficiency due to SCID; pegademase, an older agent used to manage ADA deficiency in pediatric SCID patients is no longer commercially available.[17, 25-27] C1 esterase inhibitor safety and efficacy have not been determined in pediatric patients younger than 5 years of age.[1-4, 25-27]

Although not FDA-approved, some investigators have studied agalsidase use in children younger than 8 years of age to reduce or prevent complications associated with Fabry disease (e.g., kidney complications, cardiovascular disease, cerebrovascular dysfunction). Studies have included patients ranging in age from 2.5 to 8 years of age for boys and 4.4 to 8 years of age for girls. Results, based on small patient numbers, have shown improvements in disease manifestations, pain and quality of life without significant adverse effects in younger children. Further, long-term trials are necessary to confirm these results.[35, 36]

Alglucosidase alfa is FDA-approved and has been evaluated for early use to treat Pompe disease in all age groups from neonates to adolescents. In a small study, investigators found that alglucosidase therapy initiated early after diagnosis in neonates less than 1 month of age can improve clinical outcomes even before onset of clinical symptoms in infants with Pompe disease.[37]

Maximum recommended dosages for complement inhibitor and protein/enzyme replacement therapies FDA-approved for use in pediatric patients are summarized in Tables 4-6. Dosages exceeding these recommendations will be reviewed.

Table 4. Pediatric Complement Inhibitor Maximum Dosages[1-13]

Treatment Indication	Drug Name	Dosage Form / Strength	Maximum Recommended Dosage
Hereditary angioedema (HAE) treatment	C1 esterase inhibitor, human (Berinert®)	500 unit single-use vial for reconstitution	5 to less than 18 years: 20 IU/kg by IV injection as a single dose
HAE attacks, routine prevention	C1 esterase inhibitor, human (Cinryze®)	500 unit single-use vial for reconstitution	12 to less than 18 years: 1000 U by IV infusion every 3-4 days+
			6 to 11 years: 1000 U by IV infusion every 3-4 days

Treatment Indication	Drug Name	Dosage Form/ Strength	Maximum Recommended Dosage
HAE attacks, routine prevention (Haegarda®)	C1 esterase inhibitor, human (Haegarda®)	2000, 3000 IU single-use vials for reconstitution	12 to less than 18 years: 60 IU/kg subcutaneously twice weekly (every 3 to 4 days)
HAE treatment	C1 esterase inhibitor, recombinant (Ruconest®)	2100 IU/14 mL single-use vial for reconstitution	13 to less than 18 years (less than 84 kg): 50 units/kg as single IV dose up to a maximum of 4200 units per dose; may repeat x1 if attack symptoms persist in 24 hour period 13 to less than 18 years (greater than 84 kg): 4200 units as a single IV dose; may repeat x1 if attack symptoms persist in 24 hour period
HAE treatment	ecallantide (Kalbitor®)	10 mg/ml single-use vials x 3	12 to less than 18 years: 30 mg subcutaneously as three separate 10 mg injections; may repeat x1 in 24 hour period if attack symptoms persist
Atypical hemolytic uremic syndrome	eculizumab (Soliris®)	300 mg single-dose vial	greater than 2 months (5-9 kg): 300 mg by IV infusion over 1-4 hours every 3 weeks children, adolescents 10-19 kg: 300 mg by IV infusion over 1-4 hours every two weeks children, adolescents 20-29 kg: 600 mg by IV infusion over 1-4 hours every two weeks children, adolescents 30-39 kg: 900 mg by IV infusion over 1-4 hours every two weeks children, adolescents greater than or equal to 40 kg: 1200 mg by IV infusion over 1-4 hours every two weeks
HAE, routine prevention	lanadelumab-flyo (Takhzyro®)	300 mg/2 mL vial	300 mg subcutaneously every 2 weeks; every 4 week dosing possible in patients well-controlled for greater than 6 months

Treatment Indication	Drug Name	Dosage Form / Strength	Maximum Recommended Dosage
Atypical hemolytic uremic syndrome^	ravulizumab (Ultomiris®)	300 mg/30 mL intravenous solution	adolescents greater than or equal to 100 kg: 3600 mg as intravenous infusion every 8 weeks starting 2 weeks after loading dose (loading dose = 3000 mg) adolescents, children 60-99 kg: 3300 mg as intravenous infusion every 8 weeks starting 2 weeks after loading dose (loading dose = 2700 mg) adolescents, children 40-59 kg: 3000 mg as intravenous infusion every 8 weeks starting 2 weeks after loading dose (loading dose = 2400 mg) adolescents, children 30-39 kg: 2700 mg as intravenous infusion every 8 weeks starting 2 weeks after loading dose (loading dose = 1200 mg) children 20-29 kg: 2100 mg as intravenous infusion every 8 weeks starting 2 weeks after loading dose (loading dose = 900 mg) infants, children 10-19 kg: 600 mg as intravenous infusion every 4 weeks starting 2 weeks after loading dose (loading dose = 600 mg) infants, children 5-9 kg: 300 mg as intravenous infusion every 4 weeks starting 2 weeks after loading dose (loading dose = 600 mg)

- + in patients not responding adequately, doses up to 2500 units (not exceeding 100 u/kg) every 3 or 4 days may be utilized based on individual patient response
- ^ treat atypical hemolytic-uremic syndrome with ravulizumab for at least six months

Table 5. Pediatric Enzyme Replacement Therapy Maximum Dosages[10-38]

Treatment Indication	Drug Name	Dosage Form / Strength	Maximum Recommended Dosage
Fabry disease	agalsidase beta (Fabrazyme®)	5 mg, 35 mg single-use vials	8 to less than 18 years: 1 mg/kg by intravenous (IV) infusion every 2 weeks
Pompe disease	alglucosidase alfa (Lumizyme®)	50 mg single-use vial	Neonates to adolescents , 18 years of age: 20 mg/kg as an IV infusion every 2 weeks

Treatment Indication	Drug Name	Dosage Form / Strength	Maximum Recommended Dosage
Hypophosphatasia (perinatal/infantile- or juvenile-onset)	asfotase alfa (Strensiq®)	18 mg/0.45 mL, 28 mg/0.7 mL, 40 mg/mL, or 80 mg/0.8 mL single-use vials	Hypophosphatasia (perinatal/infantile-onset): birth to any age: 9 mg/kg weekly as subcutaneous injection as 3 mg/kg three times weekly Hypophosphatasia (juvenile-onset): 6 years and older: 6 mg/kg weekly as subcutaneous injection, given either as 2 mg/kg three times weekly or 1 mg/kg six times weekly
Late infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease	cerliponase alfa (Brineura®)	150 mg/5 ml as 2 single-use vials copackaged with intraventricular electrolytes	3 years and older: 300 mg every other week by intraventricular infusion, followed by intraventricular electrolytes
Adenosine deaminase severe combined immunodeficiency	elapegademase (Revcovi®)	2.4 mg/1.5 mL intramuscular solution	treatment naïve: 0.2 mg/kg as intramuscular injection twice weekly for a minimum of 12 to 24 weeks; titrate up by 0.033 mg/kg/week to maintain ADA trough greater than 30 mmol/hr/L, trough deoxyadenosine nucleotides less than 0.02 mmol/L, and maintain immune reconstitution patients transitioning from pegademase: Patients receiving pegademase doses less than or equal to 30 U/kg should receive elapegademase 0.2 mg/kg intramuscularly once weekly; patients with pegademase doses greater than 30 U/kg should receive equivalent elapegademase dose*
Mucopoly-saccharidosis (MPS) IVA (Morquio A syndrome)	elosulfase (Vimizim®)	5 mg/5 mL single-use vial	5 years and older: 2 mg/kg by IV infusion over a minimum of 3.5-4.5 hours once weekly
MPS VI (Maroteaux-Lamy syndrome)	galsulfase (Naglazyme®)	5 mg/5 mL preservative-free vials	3 months and older: 1 mg/kg by IV infusion once weekly
Hunter syndrome	idursulfase (Elaprase®)	6 mg/3 mL single-use vial	5 to less than 18 years: 0.5 mg/kg as IV infusion once weekly

Treatment Indication	Drug Name	Dosage Form / Strength	Maximum Recommended Dosage
Gaucher disease, type 1 (nonneuropathic)	imiglucerase (Cerezyme®)	400 mg vials for reconstitution	2 to 16 years: 60 U/kg by IV infusion over 1-2 hours every 2 weeks
MPS 1 (Hurler, Hurler-Scheie forms; Scheie form with moderate to severe symptoms)	laronidase (Aldurazyme®)	2.9 mg/5 mL single-use vials	6 months of age and older: 0.58 mg/kg by IV infusion over 3-4 hours once weekly
Congenital sucrase-isomaltase deficiency (CSID)	sacrosidase (Sucraid®)	8500 international units/mL as 118 mL oral solution bottles	5 months to less than 18 years: less than or equal to 15 kg: 8500 units orally mixed in 2-4 ounces of water, milk, or infant formula with each meal or snack > 15 kg: 17,000 units orally mixed in 2-4 ounces of water or milk with each meal or snack
Lysosomal acid lipase (LAL) deficiency	sebelipase alfa (Kanuma®)	20 mg/10 mL single-use vial	> 1 month of age to less than 18 years: 1 mg/kg by IV infusion once every two weeks
Rapidly progressive LAL deficiency (developing in first 6 months of life)	sebelipase alfa (Kanuma®)		1-12 months of age: 3 mg/kg by IV infusion once weekly
Gaucher disease, type 1	taliglucerase alfa (Elelyso®)	200 unit single-use vials for reconstitution	treatment-naïve (4 years and older): 60 U/kg by IV infusion once every 2 weeks previously treated with imiglucerase (4 years and older): use same unit/kg dosage for taliglucerase that was prescribed for imiglucerase and administer every two weeks
Gaucher disease, type 1	velaglucerase alfa (Vpriv®)	400 unit single-use vials for reconstitution	treatment-naïve (4 years and older): 60 U/kg by IV infusion once every 2 weeks previously treated with imiglucerase (4 years and older): use same unit/kg dosage for velaglucerase that was prescribed for imiglucerase and administer every two weeks

Treatment Indication	Drug Name	Dosage Form / Strength	Maximum Recommended Dosage
MPS VII (Sly syndrome), excluding central nervous system symptoms	vestronidase alfa-vjbk (Mepsevii™)	10 mg/5 mL single-use vial	birth to less than 18 years: 4 mg/kg as IV infusion every two weeks

- * elapegademase equivalent dose to pegademase: pegademase dose (U/kg) divided by 150

Table 6. Pediatric Protein Replacement Therapy Maximum Dosages[10-13, 39]

Treatment Indication	Drug Name	Dosage Form / Strength	Maximum Recommended Dosage
Severe congenital protein C deficiency (acute episode*)	protein C concentrate (Ceprotin®)	500 IU, 1000 IU single-use vial for reconstitution	birth to less than 18 years: 100-120 IU/kg initial dose by IV infusion, followed by 60-80 IU/kg every 6 hours for 3 doses by IV infusion*
Severe congenital protein C deficiency (short-term prophylaxis/ maintenance dose*)			birth to less than 18 years: 45-60 IU/kg every 6 to 12 hours by IV infusion
Severe congenital protein C deficiency (long-term prophylaxis*)			birth to less than 18 years: 45-60 IU/kg every 12 hours by IV infusion

- * maximum protein C concentrate infusion rate: 2 ml/min, except in children less than 10 kg, where infusion rate should not exceed 0.2 ml/kg/min

2 Duration of Therapy

There is no basis for limiting the duration of complement inhibitor and enzyme/protein replacement therapy as enzyme deficiencies represent chronic disorders and require sustained treatment. [1-39]

3 Duplicative Therapy

FDA-approved enzyme replacement therapies are indicated for specific enzyme deficiencies. Patients with multiple enzyme deficiencies may be prescribed multiple enzyme replacement therapies concurrently. Adjunctive administration of enzyme replacement therapies without multiple enzyme deficiency diagnoses is not clinically reasonable and will be evaluated.

4 References

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