Pharmacy Focus:
Enzyme Replacement Therapy

About Enzyme Replacement Therapy

Enzyme replacement therapy (ERT) is typically used for a category of rare diseases called lysosomal storage disorders. These diseases are all inherited, and therefore, genetic testing is performed to confirm them (sometimes even before birth). Although the available therapies are not curative, they do attempt to slow the progression of the disease and target the underlying cause.

Prior to ERT, most of these diseases did not have any other viable treatment options, only symptom management. For diseases where ERT is the only treatment option, patients require infusions for the rest of their lives. With these therapies being safe and effective, patients typically utilize them indefinitely, so they are associated with long-term expenses. Costs can vary radically between different ERTs and even between different recipients of the same ERT.[1,2]

Current Diseases with Enzyme Replacement Therapies[3-8]

Disease	ICD 10 Code(s)	ERT	HCPCS Code(s)	Estimated Current Cost Per Plan Year	Additional Treatments	Pipeline Possibilities
Adenosine Deaminase Severe Combined Immune Deficiency[9,10]	D81.31	Revcovi® (Elapegademase-lvlr)	J3590	Infants to Adults: **$300,000 to $8,000,000+**	Stem Cell Transplant	Gene Therapies: Strimvelis® and Simoladagene Autotemcel
Alpha Mannosidosis[11]	E77.1	None at this time	TBD	TBD	Stem Cell Transplant	ERT: Lamzede® (Velmanase alfa)
Fabry Disease[12,13]	E75.21	Fabrazyme® (Agalsidase beta)	J0180	Infants to Adults: **$70,000 to $600,000+**	Galafold®	ERT: Agalsidase alfa and Pegunigalsidase alfa
Gaucher Disease Type 1[14,15,16,17,18]	E75.22	Cerezyme® (Imiglucerase)	J1786	Toddlers to Adults: **$100,000 to $600,000**	Cerdelga® Zavesca®	Gene Therapies: AVR-RD-02
		Elelyso® (Taliglucerase alfa)	J3060			
		Vpriv® (Velaglucerase alfa)	J3385			
Hypophosphatasia[19]	E83.31 E83.39	Strensiq® (Asfotase alfa)	J3590 J3490	Newborns to Adults: **$40,000 to $2,000,000+**	Stem Cell Transplant	None
Lysosomal Acid Lipase Deficiency[20]	E75.5	Kanuma® (Sebelipase alfa)	J2840	Children to Adults: **$300,000 to $3,000,000+**	Stem Cell Transplant	None
MPS* 1 (Hurler Syndrome)[21]	E76.0	Aldurazyme® (Laronidase)	J1931	Infants to Adults: **$120,000 to $800,000+**	Stem Cell Transplant	Gene Therapy

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Disease	ICD 10 Code(s)	ERT	HCPCS Code(s)	Estimated Current Cost Per Plan Year	Additional Treatments	Pipeline Possibilities
MPS* 2 (Hunter Syndrome)[22]	E76.1	Elaprase® (Idursulfase)	J1743	Toddlers to Adults: **$160,000 to $1,000,000+**	Stem Cell Transplant	Gene Therapy & ERT
MPS* 4A (Morquio A syndrome)[23]	E76.211	Vimizim® (Elosulfase alfa)	J1322	Children to Adults: **$500,000 to $2,000,000+**	Stem Cell Transplant	None
MPS* 6 (Maroteaux-Lamy syndrome)[24]	E76.29	Naglazyme® (Galsulfase)	J1458	Infants to Adults: **$150,000 to $1,600,000+**	Stem Cell Transplant	Gene Therapy
MPS* 7[25]	E76.29	Mepsevii® (Vestronidase alfa)	J3590	Infants to Adults: **$200,000 to $950,000+**	Stem Cell Transplant	ERT
Neuronal Ceroid Lipofuscinosis Type 2 (NCL-2; Batten Disease type)[26]	E75.4	Brineura® (Cerliponase alfa)	J0567	Children to Adults: (One Dose): **$800,000**	None	Gene Therapy
Niemann Pick Type B	E75.241	None at This Time	TBD	N/A	None	ERT: Olipudase Alfa; Currently Available for Compassionate Use
Pompe Disease[27,28]	E74.02	Lumizyme®	J0221	Toddlers to Adults: **$300,000 to $700,000+**	None	Gene Therapy & ERT
		Nexviazyme™ (Avalglucosidase Alfa)	J3590		None	

Cost Containment Considerations

As part of its HMConnects™ cost containment program, HM Insurance Group (HM) works to support cost management opportunities around the use of gene and cell therapies and other high-cost pharmaceutical treatment options that can impact our clients' bottom line. The Pharmacy Operations (RxOps) team watches the market – and our book of business – to anticipate how current and future advancements will impact financial risk levels for HM's client base. Standard practices include reviewing, auditing and collaborating on the content of current policies, monitoring trends and implementing appropriate cost savings techniques, all of which are provided to HM's clients at no additional cost to them.

Pharmacy Focus provides valuable information about pharmaceutical industry developments and their associated costs that can impact the growing claims trend in the self-funded insurance market. Be aware of influences and gain insight into approaches that may help to contain costs. Please share topic suggestions or feedback with **HMPharmacyServices@hmig.com**.

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