List of medicines funded through the risk sharing arrangement for inherited metabolic disorders (October 2024)

The medicines listed below are only included in the risk share for the specific indications listed and only in the specific circumstances described within this document.

List of medicines and indications

Medicines included in the risk share arrangements prior to establishment of Scottish Medicines Consortium (SMC):

- **Agalsidase alpha (Replagal®)** for the treatment of Fabry Disease
- **Agalsidase beta (Fabrase/Fabrazyme®)** for the treatment of Fabry Disease
- **Imiglucerase (Cerezyme®)** for the treatment of Type 1 Gaucher Disease

Medicines accepted for use by SMC:

- **Miglustat (Zavesca/Vevesca®)** for the treatment of Type 1 Gaucher Disease.
- **Velaglucerase (Vpriv®)** for the treatment of Type 1 Gaucher Disease.
- **Eliglustat (Cerdelga®)** for the long-term treatment of adult patients with Gaucher disease type 1 (GD1) who are CYP2D6 poor metabolisers, intermediate metabolisers or extensive metabolisers
- **Avalglucosidase alfa (Nexviadyme®)** for the treatment of Pompe disease.

IMD Medicines available through Ultra Orphan Pathway:

- **Cerliponase alfa (Brineura®)** for the treatment of neuronal ceroid lipofuscinosis type 2 (CLN2) disease, also known as tripeptidyl peptidase 1 (TPP1) deficiency.
- **Olipudase alfa (Xenpozyme®)** for the treatment of non-Central Nervous System (CNS) manifestations of Acid Sphingomyelinase Deficiency (ASMD) in paediatric and adult patients with type A/B or type B.
- **Velmanase alfa (Lamzede®)** enzyme replacement therapy for the treatment of non-neurological manifestations in patients with mild to moderate alpha-mannosidosis.

New high cost IMD medicines are added automatically to the scheme if they are approved through the ultra-orphan pathway.

Medicines accepted for restricted use by SMC:

- **Carbaglu (N-carbamoyl-L-glutamic acid)** for the treatment of hyperammonaemia due to N-acetylglutamate synthase deficiency.
- **Migalastat (Galafold®)** is indicated for the long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (α-galactosidase A deficiency) and who have an amenable mutation.

The below medicines are not recommended by SMC but included in the risk share if there has been a recommendation following Individual Patient Treatment Request (IPTR)/Peer Approved Clinical System (PACS) within the NHS Board of residence of the patient

- **Alglucosidase alfa (Myozyme)** for the treatment of Pompe disease (acid maltase deficiency).
- **Laronidase (Aldurazyme)** for the treatment of MPS 1, Hurler-Scheie or Scheie syndrome.
- **Idursulfase (Elaprase)** for the treatment of MPS II (Hunters syndrome)

List of excluded medicines and indications

The following orphan drug therapies are not recommended by the SMC for these indications or have not been assessed and therefore are excluded from the risk share arrangements:

- **Miglustat (Zavesca/Vevesca®)** for the treatment of progressive neurological manifestations in adult patients and paediatric patients with Niemann-Pick type C disease.
- **Kuvan (Sapropterin®)** for the treatment of PKU
- **Elosulfrase alpha (Vimizim®)** for the treatment of mucopolysaccharidosis, type IVA (Morquio A Syndrome, MPS IVA) in patients of all ages
- **Galsulfase (Naglazyme®)** for the treatment of MPS VI (Maroteaux-Lamy syndrome).

Reimbursement criteria

New registrations are only accepted from the Scottish Inherited Metabolic Disorders Service. Prescription arrangements for patients who are already covered by the scheme are not affected.[1]

[1] It is envisaged that these prescriptions will be migrated to the Scottish Inherited Metabolic Disorders Service. Details of this process still have to be worked out. The IMD service will contact prescribers.