Summary of operational arrangements for Cipaglucosidase alfa /miglustat in NHS Scotland. EAMS number 50636/0001

The aim of the MHRA Early Access to Medicines Scheme (EAMS) is to provide earlier availability of promising new unlicensed and ‘off label’ medicines to UK patients that have a high unmet clinical need. The medicinal products included in the scheme are those that are intended to treat, diagnose or prevent seriously debilitating or life threatening conditions where there are no adequate treatment options. The MHRA EAMS positive opinion should not be regarded as a medicine licensed by the MHRA or a future commitment by the MHRA to license such a medicine. The opinion and EAMS documentation published by the MHRA are intended only to inform clinicians’ decision making and not to recommend use. Under EAMS the risk and legal responsibility for prescribing an EAMS medicines remains with the prescribing clinician. More information about the scheme can be found here: http://www.mhra.gov.uk/Howweregulate/Innovation/EarlyaccesstomedicinesschemeEAMS/index.htm

Cipaglucosidase alfa /miglustat is made available free of charge for patients via EAMS during the EAMS period. This document summarises how the medicine can be used in Scotland.

**Medicine**

Cipaglucosidase alfa 105mg of powder for concentrate for solution of infusion, miglustat 65mg hard capsules

**Indication and patient population**

Cipaglucosidase alfa in conjunction with miglustat is indicated in the long-term treatment of adult symptomatic patients with confirmed diagnosis of late-onset Pompe disease (acid α-glucosidase [GAA] deficiency) who have received enzyme replacement therapy with alglucosidase alfa for ≥ 2 years.

**Conditions for entry to EAMS**

The EAMS makes free-of-charge cipaglucosidase alfa/miglustat for use in Pompe disease available to Health Boards (subject to approval by Amicus Therapeutics) provided that the following conditions are met

I. The patient (who is the subject of each order for EAMS cipaglucosidase alfa/miglustat) is an adult patient with Pompe disease.

II. The Health Board does not have any policies, guidelines or procedures in place which prohibit the acceptance of EAMS medicines

III. While a valid positive EAMS opinion is in place (Marketing Authorisation expected Q3 2022)
Supply arrangements

1. Clinicians wishing to access treatment for their patients with cipaglucosidase alfa / miglustat need to contact the Amicus Therapeutics Medical team by completing a request for expanded access at https://www.amicusrx.com/advocacy/expanded-access. If further information is required, please contact EAMS@amicusrx.com

2. As for all unlicensed medicines and off-label uses of licensed medicines, individual health boards will also have local governance arrangements in place to authorise medicines supplied via EAMS. This review should be expedited as a rigorous risk/benefit assessment has already been conducted by MHRA and can be accessed in the Public Assessment Report (PAR), link appended.

3. Other aspects of the supply arrangements can be accessed via EAMS documentation.

Practical considerations

Pompe disease is a rare inherited metabolic disorder and it is advised that the management of patients is discussed with the Scottish Inherited Metabolic Disorders Service.

Cipaglucosidase alfa / miglustat recommended frequency of administration is once every other week.

a) Miglustat should be taken 1 hour (between 50 to 90 minutes) before the start of the cipaglucosidase alfa infusion. In the event of cipaglucosidase alfa infusion delay, the start of infusion should not exceed 3 hours from the oral administration of miglustat.

b) Cipaglucosidase alfa should be administered as an intravenous infusion using an infusion pump. The patient should be observed during and until infusion is complete.

The requirement for an oral medicine in advance of the enzyme replacement therapy is not a part of the current standard of care treatment with alglucosidase alfa, and may increase clinic time.

Infusion of cipaglucosidase alfa should be administered incrementally as determined by patient response and comfort over approximately 4 hours.

Monitoring requirements for cipaglucosidase alfa are similar to alglucosidase alfa. There are no additional monitoring requirements for miglustat.

Cipaglucosidase alfa should be stored in a refrigerator between 2°C to 8°C and protected from light. The powder requires reconstitution followed by further dilution, in a two-step process, prior to administration.

Miglustat does not require special storage conditions.

Homecare can be made available on the NHS Commissioned Framework (NP45418). Please contact nss.pchc@nhs.scot to arrange. The relevant home health board will have to agree to fund the homecare package for eligible patients.

Pharmacovigilance and data collection

Clinicians are required to report to Amicus Therapeutics using specific documentation and recording mechanisms.
EAMS termination arrangements/exit strategy

Following Marketing Authorisation of Cipaglucosidase alfa /miglustat for the treatment of patients with Pompe disease, the EAMS scheme will close in line with the MHRA regulations and no new patients will be allowed to enrol onto the scheme to access free of charge supply.

Access to treatment for new patients would be via local board processes from the point of licensing until Scottish Medicines Consortium (SMC) accepted advice is issued or, where relevant, until availability via the ultra-orphan pathway is confirmed by Scottish Government.

The provision of Cipaglucosidase alfa /miglustat, free of charge via EAMS for any one patient enrolled in the EAMS shall end, on the earliest of the following to occur:

A. In the event that SMC accepted advice is issued for this medicine and indication, 30 days after the publication of SMC accepted advice on their website [www.scottishmedicines.org.uk](http://www.scottishmedicines.org.uk).

OR

B. In the event that SMC not recommended advice is issued:
   I. until such time as Amicus Therapeutics may receive SMC accepted advice for this medicine and indication (e.g. after a resubmission).
   II. until such time that the patient:
      a. Completes the defined course length of treatment
      b. No longer derives clinical benefit (e.g. disease progression or unacceptable toxicity)

OR

C. In the event that Marketing Authorisation is not granted, until such time as the patient no longer derives clinical benefit (disease progression or unacceptable toxicity).

OR

D. In the event of availability through the ultra-orphan pathway, 30 days after Scottish Government confirmation

Supporting documents
- Public Assessment Report
- Treatment Protocol – Information for Healthcare Professionals
- Treatment Protocol – Information for Patients
- Treatment Protocol – Information on the Pharmacovigilance System
- Information for NHS Medical Directors