NICE National Institute for Health and Care Excellence

Update on the re-evaluation of elosulfase alfa

NICE, NHS England and NHS Improvement (NHSE&I) and BioMarin have reached a commercial agreement which will enable the NICE re-evaluation of ID1643 elosulfase alfa (Vimzim) to be resumed, following the suspension of this topic in February 2020.

While the re-evaluation continues, we can also confirm that NHSE&I and BioMarin have agreed a commercial arrangement that will ensure patients can continue to access elosulfase alfa over the duration of the NICE re-evaluation (until **December 2021**).

- Because of the interim agreement reached, during this 12-month extension period:
    - Patients receiving elosulfase alfa treatment as part of the Managed Access Agreement (MAA) can continue to receive treatment
    - New patients can start receiving treatment with elosulfase alfa, subject to the criteria outlined in (1) below).

The adaptations to the MAA communicated on 22 July 2020 remain in place until further notice. These adaptations are designed to ensure that no patient is disadvantaged by the impact of COVID-19 (coronavirus) on NHS services.

These include:

1. The MAA baseline assessments for new treatment naïve patients can be deferred until they can be performed safely in hospital on the condition that:
    - The patient has a confirmed diagnosis of MPS type IVA as per the diagnosis criteria recommended in Wood et al. (2012), **AND**
    - Starting treatment with elosulfase alfa is appropriate in the opinion of the treating clinician.
2. The treating clinician will be responsible for clinical decisions concerning the safe continuation of elosulfase alfa treatment.
3. Routine clinical safety monitoring should continue, however if patients are unable to complete ongoing assessments as required by the MAA (e.g. because circumstances do not allow), these should be deferred until they can be performed safely under valid, standardised conditions.
4. The 6-monthly patient caseload review **will not** be performed, however if clinical teams would like to seek advice from the Managed Access

Oversight Committee on individual cases they are advised to contact managed.access@nice.nhs.uk to coordinate a response. **Please do not include any patient identifiable information in any correspondence to NICE, use the managed access identifier only.**

5. The treatment stopping criteria for missed treatments (missing 3 infusions in a 14-month period and/or failing to perform the managed access clinical and quality of life assessments) will continue to be flexed to take account of each patient's personal circumstances during this period.

6. It is recommended that elosulfase alfa dose adjustments are not considered unless weight can be measured under standardised conditions.

Patients and their families should be advised to contact their clinical team if they have any concerns about their treatment while these measures are in place.

NICE and NHSE&I will provide a further update to stakeholders concerning the schedule for the NICE re-evaluation and details on the terms that will be applied during the interim extension period.