Information for patients and clinicians regarding the impact of COVID-19 (coronavirus) on the elosulfase alfa (Vimizim) Managed Access Agreement

NICE and NHS England and NHS Improvement have taken advice from clinical experts to review the potential impact of COVID-19 on the delivery of the Managed Access Agreement (MAA) for elosulfase alfa (Vimizim) for treating mucopolysaccharidosis (MPS) type IVa [HST2]. This statement sets out considerations for patients who are already receiving treatment and those patients who have not yet started treatment. We have considered the unprecedented demand on the NHS in the coming months and that some patients may want to self-isolate or be required to shield.

While we would like to provide general information about likely access to treatment and suggested adjustments to clinical monitoring, some hospitals may have to take additional local decisions to further prioritise resources to tackle COVID-19. For questions about your individual circumstances and to understand what is available at your usual treatment centre, please contact the team who manage your treatment.

For existing managed access patients

1. Patients who are already receiving treatment as part of the MAA

1.1 Where safe and appropriate to do so, treatment with elosulfase alfa will continue to be delivered via homecare or by previously trained family members for those patients already receiving infusions via this method.

1.2 If you are self-isolating or shielding and have concerns about healthcare professionals entering your home, please review the UK Government advice on self-isolation and shielding, and speak with your treatment centre and homecare provider about the precautionary measures in place to protect you and others.

1.3 Homecare providers are already experiencing higher demand for their services and it is likely that existing scheduled treatments and/or the frequency of future treatments may need to change. Your homecare provider will be able to provide the most up to date information concerning their schedules.

1.4 We aim to ensure that no patient will be disadvantaged if they are unable to receive regular treatment infusions during this period. 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 be flexed to take account of each patient's personal circumstances during this period.

1.5 Patients will be allowed to restart treatment where clinically appropriate under the MAA if treatment is interrupted during this period.

2. Ongoing monitoring of existing managed access patients

2.1 If patients are unable to complete ongoing assessments as required by the MAA (e.g. because it is unsafe, or circumstances do not allow), these should be deferred until they

can be performed safely under valid, standardised conditions your treatment centre. With these measures we aim to ensure that no patient will be disadvantaged if they are unable to complete monitoring assessments as a direct result of COVID-19.

2.2 It is recommended that elosulfase alfa dose adjustments will not be considered during this period and will resume when weight can be measured under standardised conditions at your treatment centre.

2.3 Where possible Rare Disease Research Partners will continue to conduct Quality of Life assessments via telephone. We recognise that the outcomes of these assessments may be affected during this period due to heightened anxiety, school/nursery closures, the potential need to self-isolate and the impacts of potentially delayed treatments. This will be taken into account by the Managed Access Oversight Committee at subsequent clinical reviews.

For new patients starting treatment under the MAA

3. Adjustments to baseline assessments and treatment initiation

3.1 New patients starting treatment within the MAA **must** have a confirmed diagnosis of MPS type IVa (as per the diagnosis criteria recommended in Wood et al. (2012) and **must** have a uKS (urine) test collected and stored prior to the first treatment.

3.2 If it is not possible to conduct the other baseline tests required by the MAA, these can be deferred until they can be performed safely under valid, standardised conditions at your treatment centre. Patients will not be disadvantaged if their baseline tests are performed after their first treatment; the Managed Access Oversight Committee will take this into account at subsequent clinical reviews.

3.3 In consultation with their treating clinician, the patient and their family should assess whether the benefits of attending a treatment centre to perform the baseline assessments and receive first treatment outweigh the risks of potential exposure to Covid-19. In addition, the patient and their family should consider that they may need to attend face to face appointments on multiple occasions when starting on treatment, before transferring to homecare.

3.4 Some treatment centres may need to reschedule treatment slots during this period because of the limited availability of elective inpatient day-case beds for treatment initiation. You will be notified if this is the case and your new treatment date will be provided when slots are available.

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

We will regularly review this information and share updates as these become available.