Patient/parent questions on the managed access agreement for cerliponase alfa (January 2022)

Education
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The re-evaluation process

What is the timeline of NICE decision-making process concerning the managed access agreement (MAA)?

The NICE evaluation using the new data collected during the MAA is expected to start around the end of year 4, which is November 2023.

The timelines for the NICE evaluation will be confirmed during the summer of 2023. Those timelines will be developed so that the NICE evaluation will be complete before the end of the 5-year MAA, that is by November 2024.

How can patients and families get involved in the NICE evaluation?

By participating in the MAA, with the associated activity and the data collection patients and their families are already making an important contribution to the NICE evaluation. This is vital in ensuring complete data is available on all patients, which BioMarin can use as part of their submission to NICE.

NICE will invite patient organisation stakeholders to provide written submissions and nominate patient experts. Patient experts can also provide written submissions in advance of the committee meeting. Patient experts are invited to attend the committee to share their experience on receiving or caring for someone who receives treatment with cerliponase alfa and to address issues that arise during the committee process that are relevant to patients and their families.

While randomised controlled trials are the best way to assess the comparative effectiveness of a treatment, NICE recognises that there are other sources of data which are needed to complement these complex studies, especially in rarer conditions where when it is often not possible or unethical to conduct a randomised controlled trial. This is why the collection of quality-of-life data is an important element of the MAA for cerliponase alfa. Additionally, the committee would welcome information from patients and families about their lived experience. The best way to contribute to the guidance update is to have this information collated by a patient organisation stakeholder who can present the individual stories, as well as general points about the experience of patients and families, as a written submission in advance of the committee meeting.

NICE's patient involvement team is also able to provide further information, support and advice to patients and their families before and during the evaluation process.

Will the evidence review group be involved again in the re-evaluation?

NICE aims to involve the same evidence review group (ERG) primarily because they are familiar with the topic.

How can you be sure that the correct data is being collected for cerliponase alfa?

The MAA outlines the uncertainties that the NICE committee identified and the outcomes that need to be collected to provide new evidence when cerliponase alfa is evaluated again at the end of the data collection period. NICE is hosting regular MAA Oversight Group meetings with the company, NHS England, BDFA, clinicians and the Rare Diseases Research Partnership to review the data collection and to confirm that the relevant clinical and patient reported outcome data is being collected. Any issues that are identified will be addressed during these meetings and we will provide short updates on the progress with these meetings.

What other research projects could we do to strengthen the evidence at the end of the MAA?

The NICE FED outlines the uncertainties the NICE committee had at the original evaluation and as outlined above, the MAA has been designed to collect the data required to address these uncertainties. Whilst it may not be necessary to do anything more than to deliver those analyses the NICE committee will consider all available evidence when it updates the guidance.

Additionally, the school reports study (coordinated by BDFA) was designed to provide a complementary assessment of patient outcomes, beyond the clinical setting. This along with any information that families are able to provide about their child's progress on treatment should be collated along with the school reports study and submitted when the guidance is updated.

How will NICE's decision be communicated to patients and their families?

NICE's recommendation will be shared in confidence with stakeholders who are involved in the evaluation process (including patient organisations who have registered). It will then be published on the NICE website 5 days later.

Questions relating to a successful outcome

Would there be any changes in how I receive my treatment?

Whether and how your treatment might continue to be provided will depend on the evidence presented and the recommendation of the NICE committee. Should there be any changes to how patients receive their treatment, this would be communicated to patients and their families via the patient groups and treatment centres once NICE's decision has been published.

Would patient data continue to be collected on the impact of cerliponase alfa?

NICE's requirement for data collection would cease once the evaluation has been completed. However, the NHS and clinicians may wish to continue to collect data on the impact of cerliponase alfa to continue to monitor patient outcomes and other factors related to the service.

How would new patients be able to access the drug?

Information for new patients and how they will receive their treatment would be communicated via patient organisations and treatment centres once NICE's decision has been published. Patients with a confirmed diagnosis will be able to access treatment in line with the NICE guidance from the services commissioned to provide cerliponase alfa.

Questions concerning the possibility of a negative recommendation

Could NICE make a negative recommendation on the grounds of cost, even if the data collection is correct and the evidence provided proves the treatment is effective?

The committee originally made a recommendation to ensure that patients have access to this promising treatment while further evidence is generated to address the significant evidential uncertainties that were identified. When the guidance is updated, the committee will need to assess all the available evidence and consider whether cerliponase alfa can be recommended for routine use in the NHS. The NICE Highly Specialised Technology committee considers a range of factors when recommending treatments for use in the NHS and cost-effectiveness, which considers both the clinical effectiveness and costs associated with the treatment, is one of these considerations.

Will there be an appeal process if cerliponase alfa is not approved?

Yes, in line with NICE's process for evaluations, all registered stakeholders which includes patient organisations, will have the opportunity to appeal the final recommendation. The NICE Patient Involvement team will also be able to provide support and guidance on the appeal process (but not the content of any appeal) for patient organisations.

Monitoring the MAA

How can we be assured that the uncertainties NICE identified are being addressed?

NICE is convening regular MAA Oversight Group meetings with the company, NHS England, BDFA, clinicians and the Rare Diseases Research Partnership to review the data collection process and to confirm that the relevant patient data is being collected and will be available for analysis at the end of the MAA. NICE will provide brief updates from these meetings, when they occur, to update on progress. Patients on the MAA are reviewed by the cerliponase alfa clinical panel, chaired by NHS England.

How will patients and their families be kept informed, will there be an opportunity to see reports?

NICE will provide brief progress updates following each MAOG meeting. The BDFA and Rare Diseases Research Partnership are also members of the MAOG.

Delivery of clinical assessments to ensure the best evidence is collected

Who has developed the assessments that are part of the MAA data collection?

The assessments are designed collaboratively by researchers and clinicians in the UK and internationally. They are intended to be used as tools to objectively monitor the progress of patients receiving treatment. The assessments used were included in the data collection plan because of their ability to collect the information needed, based on insight from clinicians and patients about the appropriateness of using these tools.

Can the scheduling of assessments be adjusted so they are not done within hours of the brain infusion which, alongside travel to London, is tiring for the child?

Families can express their preferences here. It may be possible to arrange the psychology assessments on another day if families are prepared to come for 2 or 3 days.

Can videos be used, for example a video of a parent carrying out the assessment so that the child is more at ease?

Psychologists can consider the role of videos in their assessments. Families know their children better than anyone, so talking to clinicians about adjustments is perfectly reasonable.