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Cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2 [ID943]

Evaluation Committee Meeting – Thursday 29 August 2019
5th Committee meeting

The following documents are made available to the consultees and commentators:

1. **Draft managed access agreement (MAA) proposal prepared by BioMarin**

2. **BDFA letter to NICE HST Committee**

Any information supplied to NICE which has been marked as confidential, has been redacted. All personal information has also been redacted.

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Managed Access Agreement

Cerliponase alfa for treating CLN2 Disease

Date of Agreement	TBC
NHS England	John Stewart
BioMarin	James Lennertz
BDFA	Harriet Lunnemann
Clinical lead	Professor Paul Gissen
NICE	Sir Andrew Dillon

1 Purpose of agreement

1.1 The objectives of the document as a whole are to embody a set of auditable measures that will be used to assess the compliance of this "Managed Access Agreement" in England and to ensure that all relevant stakeholders have a common understanding that such measures have the agreement and backing of all involved and will therefore be enforced.

1.2 This Managed Access Agreement has been drawn up by NHS England, BioMarin International Limited (the "Market Authorisation Holder" or "MAH"), BioMarin United Kingdom Limited (the "authorised seller") and NICE with the engagement of patient community experts and clinicians, and seeks to satisfy the requirements outlined below.

1.3 For the avoidance of doubt, the parties intend this Managed Access Agreement to be legally enforceable between them. The patient organisation and clinician signatories will use their best endeavours to commend the Agreement to their patients and colleagues and encourage compliance with the Agreement.

1.4 A Commercial in confidence ancillary agreement containing certain terms relating to the supply of Brineura® (cerliponase alfa) agreed between the licensed owner of cerliponase alfa (BioMarin Europe Limited) and NHS England is appended to this Agreement at Appendix B).

2 Background

2.1 The NICE evaluation has developed positive recommendations conditional on a Managed Access Agreement (MAA) being developed and agreed by key stakeholders in the use of cerliponase alfa in the NHS in England.

2.2 This MAA includes the following:

- A statement that sets out the clinical criteria for starting and stopping treatment with cerliponase alfa.
- Agreement between the MAH and NHS England on a financial arrangement for the total costs of cerliponase alfa throughout the duration of the managed access agreement.
- Agreement between the MAH and NHS England that ensures patients started on cerliponase alfa during the term of the Managed Access Agreement should be able to continue treatment until they and their NHS clinicians consider it appropriate to stop.

3 Commencement and period of agreement

3.1 This agreement shall take effect on the date of publication of the Guidance. It will remain in force until the earlier of: (i) publication of a reissued NICE HST guidance for cerliponase alfa or; (ii) for a maximum of 5 years. The MAH will provide the relevant data required for the review of the guidance on the product performance during the fourth year of the agreement. NICE will reissue guidance to the NHS in England based on a review of the data during the fifth year of the agreement.

4 Patient eligibility

4.1 To receive treatment, patients or their guardians must sign up to the 'Managed Access Patient Agreement' included in Appendix A to this Managed Access Agreement.

4.2 Patients are required to attend their clinics two times a year for assessment.

4.3 Children under the age of 3 will be excluded from the stopping criteria as natural decline in functional endpoints is not seen at this point. However, it is important to collect data in this population to support the future evaluation and research in children under 3.

4.4 Patients must be made aware of the start and stop criteria for receiving cerliponase alfa treatment:

4.5 Start Criteria[1]

All of the following are required before treatment is started:

- All patients must have a confirmed diagnosis of CLN2 on the basis of clinical information and enzymatic activity test
- The patient is not diagnosed with an additional progressive life limiting condition where treatment would not provide long term benefit e.g.; cancer or multiple sclerosis;
- The patient has a CLN2 Rating Scale ML Score of 2 or above.
- The patient is willing to comply with the associated monitoring criteria
- In addition, all patients can only start once a complete set of baseline assessments has been obtained, and they have signed the Managed Access Patient Agreement.

4.6 Stop Criteria [1]

Patients will cease therapy with cerliponase alfa, if any of the following apply:

- The Patient is non-compliant with assessments for continued therapy (non-compliance is defined as fewer than two attendances for assessment in any 14 month period excluding medical reasons for missed dosages);
- The Patient meets the stopping criteria as defined below in sections 4.7. and 4.8.

[1] The start and stop criteria have been independently developed by clinical experts and patient advocacy group in discussions with patient families. BioMarin have not endorsed these criteria

- The Patient is unable to tolerate infusions due to infusion related severe adverse events or other clinical concerns that cannot be resolved.
- The patient is diagnosed with an additional progressive life limiting condition where treatment would not provide long term benefit e.g.; cancer or multiple sclerosis

4.7 Stopping criteria for new patients (those who have never received treatment)[2]

This section applies only to those who start treatment at the age of 3 or more and who have not received treatment prior to the time at which this agreement comes into effect. The criteria for which new patients should be stopped from treatment due to non-response to treatment are:-

- A loss of more than two points (i.e. 3 or more points) on the CLN2 Rating Scale ML Score from baseline, during the first eighteen months of treatment and a total CLN2 rating scale score of less than 2;
    - A loss is defined as a decline in CLN2 rating scale ML score that has persisted for 3 or more infusions (i.e. after 6 weeks).

AND

- During the first eighteen months of treatment, a reduction in proxy reported patient quality of life of
    - ≥ 15 points on the PedsQL total score (which is three times the minimal clinically important difference[3]); and

[2] The start and stop criteria have been independently developed by clinical experts and patient advocacy group in discussions with patient families. BioMarin have not endorsed these criteria

[3] The accepted minimal clinically important difference (MCID) is 4.5 points for the PedsQL™ [Varni et al. Ambul Pediatr. 2003 Nov-Dec; 3(6):329-41]

0.2[4] drop in utility as measured by the EQ5D-5L and decline in CLN2 quality of life assessment of ≥ 15 points.

In the case of temporary illness, patients should be retested twice within 12 weeks to ensure that the decline is not as a result of temporary illness

4.8 Stopping criteria for Patients who are currently on treatment[5]

Patients who are 'currently on treatment' are defined as: (i) clinical trial patients; (ii) extension study; (iii) patients otherwise already receiving treatment for more than 12 months and have become a commissioning responsibility of NHS England; and (iv) patients who started on treatment during the term of the Managed Access Agreement and have been receiving treatment for over 18 months.

The criteria for which patients "currently on treatment" should be stopped from treatment due to non-response are:-

- A loss of more than one point (i.e. 2 or more points) on the CLN2 Rating Scale ML Score, in the previous twelve months treatment window and a total CLN2 rating scale score of less than 2;
    - A loss is defined as a decline in CLN2 rating scale ML score that has persisted for 3 or more infusions (i.e. after 6 weeks)
- OR

[4] A minimal clinically important difference of 0.24 based on distribution methods has been estimated. Walters SJ, Brazier JE. Qual Life Res. 2005;14:1523–32.

[5] The start and stop criteria have been independently developed by clinical experts and patient advocacy group in discussions with patient families. BioMarin have not endorsed these criteria

- Progression to an unreversed score of 0 on the CLN2 Rating Scales ML Score
    - Patients with a score of 0, should be retested twice within 12 weeks to ensure that decline is not as a result of temporal illness.

AND

- A reduction in proxy reported patient quality of life in the previous twelve month treatment window of
    - ≥ 15 points on the PedsQL total score (which is three times the minimal clinically important difference[6]); and
    - 0.2[7] drop in utility as measured by the EQ5D-5L and
    - Decline in CLN2 quality of life assessment of ≥ 15 points

4.9 If a patient is ill prior to an assessment, then the patient needs to be reassessed within 12 weeks and subsequent measures need to be considered from this point.

4.10 Patients who are taken off treatment will continue to be monitored for disease deterioration and supported with other clinical measures. These patients should continue to be assessed to allow gathering of important information.

5 Data collection and monitoring

5.1 Data will be collected from all patients who start during the term of this Managed Access Agreement. [redacted]

[6] The accepted minimal clinically important difference (MCID) is 4.5 points for the PedsQL™ [Varni et al. Ambul Pediatr. 2003 Nov-Dec; 3(6):329-41]

[7] A minimal clinically important difference of 0.24 based on distribution methods has been estimated. Walters SJ, Brazier JE. Qual Life Res. 2005;14:1523–32.

[redacted].

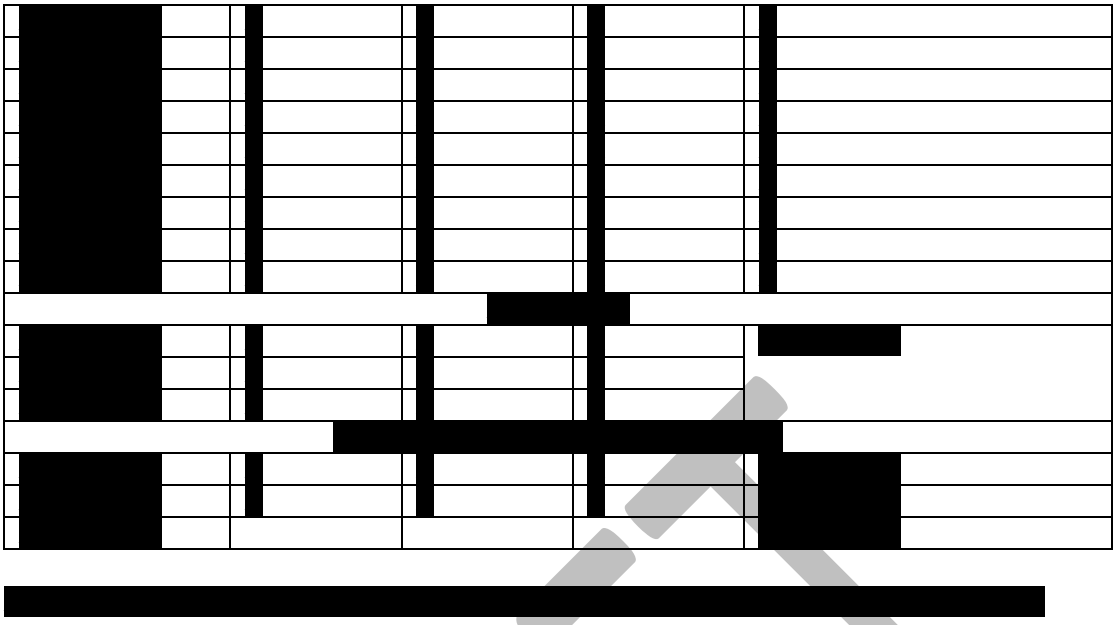
5.1.1 [redacted]. The treating clinician will be responsible for the timely collection of the anonymized clinical outcome and quality of life data and disseminate to NHS England.

5.1.2 The data will then be collated and anonymized by NHS England and sent to the MAH for analysis every six (6) months.

5.1.3 The MAH will be responsible for the analysis of the collated data and will provide access for NHS England to the results to assist it in assessing the clinical impact of cerliponase alfa on CLN2 disease.

5.2 [redacted]

[redacted]



6 Patient Appeal Process

6.1 If a patient (or patient's parents or person responsible for a patient) feels the assessments have been performed incorrectly or information not gathered appropriately, they have the right for a repeat set of assessments to be carried out at another LSD reference centre in England. Travel and associated costs will be at the patient's expense.

6.2 Reasonable adjustments will be made for patients who are unable to comply with the assessment by reasons of challenges completing assessments. These patient's stop criteria will be defined by individual agreement between the clinician and NHS England. .

7 Ownership of the data

7.1 By agreeing to take part in the Managed Access Agreement patients will be asked to consent to have their demographic and clinical data collected by their treating clinician. The MAH will be responsible for the timely analysis of the data and submitting the relevant

reassessment report to NICE in the fourth year of this agreement. The Analysed data will be owned by the MAH but shared with NHS England and NICE for the purpose of assessing the benefit of the treatment.

7.2 The data will be collected by the clinicians at the expert centres who have undertaken the relevant training prescribed by NHS England.

8 Funding

8.1 The treatment will be funded by NHS England from publication of the NICE guidance and the start of this Managed Access Agreement.

8.2 The MAH has registered a confidential patient access price with the Department of Health, and has agreed further commercial arrangements with NHS England. These confidential arrangements, set out in the ancillary agreement (Appendix B), apply for the duration of the MAA. [redacted].

8.3 The Managed Access Agreement and therefore funding for cerliponase alfa expires after 5 years. At year four a comprehensive review will look at the benefits of cerliponase alfa, collectively. The MAH, NHS England and NICE then have the opportunity to renegotiate terms for another Managed Access Agreement as an option if a NICE positive evaluation cannot be given.

8.4 Patients will be informed about the duration of this Managed Access Agreement in the Managed Access Patient Agreement.

9 Exit strategy

9.1 If at the end of the 5 year Managed Access Agreement NICE does not recommend cerliponase alfa for NHS funding, NHS England funding for cerliponase alfa will cease to be available for all patients.

10 Ongoing Review of this Agreement

10.1 The measures determined to be used are based on best current information. It would be expected that more knowledge will be gained over the next few years; hence a reassessment of the criteria by all signatories to this agreement will be reassessed three years from the start of this agreement and adjusted accordingly.

10.2 A body of NHS England, the MAH, clinical experts and patient organization representatives will meet annually to consider how the prescribed criteria are working. They will meet under the chairmanship of NICE.

Signed by	**NHS England** **John Stewart**
Signed by	**Company** **James Lennertz, VP & General Manager**
Signed by	**Patient organisation** **Harriet Lunnemann**
Signed by	**Clinical Experts Group** **Professor Paul Gissen**
Signed by	**NICE** **Professor Carole Longson / Sir Andrew Dillon**

Appendix A

Enzyme Replacement Therapy (ERT) Cerliponase alfa (Brineura) for CLN2 Managed Access Patient Agreement

NICE have approved reimbursement of Cerliponase Alfa, licensed as Brineura®, subject to the collection of auditable measures that will be used to assess the compliance of a Managed Access Agreement in England and to ensure that all relevant stakeholders have a common understanding that such measures have the agreement and backing of all involved and will therefore be enforced.

The NICE Managed Access Agreement includes:-

- A protocol that sets out the clinical criteria for starting and stopping treatment with cerliponase alfa.
- Assurance from BioMarin International limited (the "Marketing Authorisation Holder" or "MAH"), and BioMarin United Kingdom Limited ("BUKL" – the authorized seller), that it will collaborate with the BDFA and NHS England to collect your pseudonymized data. The data will be used by NICE to inform a review no more than 5 years after publication of the guidance.
- Agreement between the MAH and NHS England on a financial arrangement for the total costs of cerliponase alfa throughout the duration of the managed access agreement.
- Agreement between the MAH and NHS England that ensures patients started on cerliponase alfa during the term of the Managed Access Agreement period should be able to continue treatment until they and their NHS clinicians consider it appropriate to stop.

1. Patient Eligibility

The clinical community and BDFA feel it is appropriate and right that all patients have access to cerliponase alfa (Brineura®) in England.

- Cerliponase alfa will be added to existing standard treatment.
- Patients must be made aware of the start and stop criteria for receiving cerliponase alfa treatment and are required to attend their clinics 2 times for assessment within a 14 month period.
- All patients or their guardians must sign up to the 'Managed Access Patient Agreement' to receive treatment.

2. Access to treatment and data collection

- The criteria in this Managed Access Agreement have been used because they formed part of the phase III clinical trial and have been the basis on which the European licence for Brineura was granted.
- A distinction has been made between those patients who are new to treatment and the group of patients who have been on treatment in England prior to the commencement of this managed access agreement
- Allowance is also made for children under the age of 3, as natural decline in functional endpoints is not evaluable at this point. Children initiated on cerliponase alfa therapy before the age of 3 will be excluded from the stopping criteria mentioned until they attain the age of 3, wherein the stopping criteria for patients "currently on treatment" will apply.

3. Start Criteria[8]

- Patients must have a confirmed diagnosis of CLN2 on the basis of clinical information and enzymatic activity tests;
- The patient is not diagnosed with an additional progressive life limiting condition where treatment would not provide long term benefit e.g.; cancer or multiple sclerosis;
- The patient has a CLN2 Rating Scale ML Score of 2 or above.
- Patients can only start once a full set of baseline criteria has been obtained, and they have signed the Managed Access Patient Agreement.
- Patients / Parents will be expected to attend their clinic two times a year for assessment within a 14 month period.
- Patients / Parents will be informed about the strict requirement for attendance as set out in this patient agreement document, an appendix to the Managed Access Agreement.
- The patient is willing to comply with the associated monitoring criteria

In the event of the patient being unable to maintain the above criteria, the implementation of the stop criteria will be discussed with the Patient / Parent.

[8] The start and stop criteria have been independently developed by clinical experts and patient advocacy group in discussions with patient families. BioMarin have not endorsed these criteria

4. Stop Criteria[9]

4.1 Stopping criteria for new patients (those who have never received treatment)

This section applies only to those who start treatment at the age of 3 or more. The criteria for which new patients should be stopped from treatment due to non-response to treatment is:-

- A loss of more than two points (i.e. 3 or more points) on the CLN2 Rating Scale ML Score from baseline, during the first eighteen months of treatment and a total CLN2 rating scale score of less than 2;
    - A loss is defined as a decline in CLN2 rating scale ML score that has persisted for 3 or more infusions (i.e. after 6 weeks)

AND

- During the first eighteen months of treatment, a reduction in proxy reported patient quality of life of
    - ≥ 15 points on the PedsQL total score (which is three times the minimal clinically important difference[10]); and
    - 0.2[11] drop in utility as measured by the EQ5D-5L and
    - Decline in CLN2 quality of life assessment of ≥ 15 points

[9] The start and stop criteria have been independently developed by clinical experts and patient advocacy group in discussions with patient families. BioMarin have not endorsed these criteria

[10] The accepted minimal clinically important difference (MCID) is 4.5 points for the PedsQL™ [Varni et al. Ambul Pediatr. 2003 Nov-Dec; 3(6):329-41]

[11] A minimal clinically important difference of 0.24 based on distribution methods has been estimated. Walters SJ, Brazier JE. Qual Life Res. 2005;14:1523–32.

In the case of temporary illness, patients should be retested twice within 12 weeks to ensure that the decline is not as a result of temporary illness

4.2 Stopping criteria for Patients who are currently on treatment

Patients who are 'currently on treatment' are defined as: (i) clinical trial patients; (ii) extension study participants; (iii) patients otherwise already receiving treatment and have become a commissioning responsibility of NHS England; and (iv) patients who started on treatment during the term of the Managed Access Agreement and have been receiving treatment for over 18 months.

The criteria for which patients "currently on treatment" should be stopped from treatment due to non-response are:-

- A loss of more than one point (i.e. 2 or more points) on the CLN2 Rating Scale ML Score, in a twelve months treatment window and a total CLN2 rating scale score of less than 2;
    - A loss is defined as a decline in CLN2 rating scale ML score that has persisted for 3 or more infusions (i.e. after 6 weeks)
- OR
- Progression to an unreversed score of 0 on the CLN2 Rating Scales ML Score
    - Patients with a score of 0, should be retested twice within 12 weeks to ensure that decline is not as a result of temporal illness.

AND

- A reduction in proxy reported patient quality of life in a twelve month treatment period of

- ≥ 15 points on the PedsQL total score (which is three times the minimal clinically important difference[12]); and
- 0.2[13] drop in utility as measured by the EQ5D-5L and
- Decline in CLN2 quality of life assessment of ≥ 15 points

Patients will cease to quality for treatment if they miss more than 2 infusions in any 14 month period, excluding medical reasons for missing dosages.

If a patient is ill prior to an assessment, then the patient needs to be reassessed within 12 weeks and subsequent measures need to be considered from this point.

If you feel that you or your child will be able to comply with the above please fill in your details below and sign for reimbursed treatment to begin.

If you meet the start criteria for cerliponase alfa and choose to receive cerliponase alfa your clinician will be monitoring you or your child for demonstrable benefit.

The Managed Access Agreement (and therefore agreed funding for cerliponase alfa) expires after 5 years. At year four a comprehensive review will look at the benefits of cerliponase alfa, collectively. Any funding beyond such 5-year term will be conditional on NHS England agreeing the terms of such funding with BioMarin, the manufacturer of cerliponase alfa.

Accordingly, there are currently no arrangements to enable access to cerliponase alfa to be available as part of standard NHS care following the expiry of the MAA.

[12] The accepted minimal clinically important difference (MCID) is 4.5 points for the PedsQL™ [Varni et al. Ambul Pediatr. 2003 Nov-Dec; 3(6):329-41]

[13] A minimal clinically important difference of 0.24 based on distribution methods has been estimated. Walters SJ, Brazier JE. Qual Life Res. 2005;14:1523–32.

Any continued access to cerliponase alfa beyond this point will be subject to consideration by NICE and publication of further recommendations. If NICE does not recommend cerliponase alfa in its further review at that time patients will discontinue NHS treatment with cerliponase alfa.

You or the parents of the child must sign this Patient Managed Access Agreement as part of the start criteria for treatment.

5. Data Protection

[redacted]. as part of this Managed Access Agreement and you consent to all collected data from your monitoring visits to the hospital, including [redacted], to be entered and stored into a database, for no longer than necessary for the purpose of the Agreement and in any case no more than 5 years after publication of the guidance.

The data will be entered into a commercial database. If you object to your data being collected into this database your treating clinician may be able to offer an alternative non-commercial database.

Although researchers hope the data collected will lead to better future patient outcomes, it is your right to opt out from the data collection

By agreeing to your information being entered into the database you also explicitly consent to that information being used to fulfil the purposes of the database as described below. Patient or the respective guardian can revoke their consent by informing their treating physician and this will result in them being taken off treatment

The purposes of the database are to: (i) characterise and describe the CLN2 population as a whole, including the heterogeneity, progression and natural history of CLN2; (ii) to evaluate the long-term effectiveness and safety of Brineura (cerliponase alfa): (iii) to help the CLN2 medical community with the development of recommendations for monitoring subjects and reports on

subject outcomes to optimise subject care; (iv) to collect data on other treatment paradigms, evaluate the prevalence of their use and their effectiveness; (v) to characterise the effects of long term treatment of cerliponase alfa treatment in subjects; and (vi) to collect additional data to: (a) help broaden knowledge of identified and potential risks of cerliponase alfa, as well as increase the size of the safety database and possibly provide new information on use in identified subgroups (pregnancy, hepatic and renal impairment, cardiac impairment); and (b) to help evaluate long-term effectiveness of cerliponase alfa.

Data collected will be shared with NHS England, NICE and the MAH and may be stored on static databases and portable devices, both inside and outside of the EEA, including in the United States of America, in countries which may not provide a level of data protection equivalent to countries in the EEA. NHS England, NICE and the MAH will take the necessary steps to ensure the safety of the data when transferred or stored outside of the EEA.

Research papers and other scientific findings may be developed and published based on information provided in the registry and by signing below you understand and consent to your data being used anonymously for such scientific and academic purposes.

If you feel that you and/or your child will be able to comply with the above please fill in your details below and sign for reimbursed treatment to begin.

Patient Name: _____

Parent/Carer Name: _____

Signature: _____

Date: _____

Name of Clinician: _____

Signature of Clinician: _____

Name of Clinician: _____

Date: _____

Appendix B

Ancillary Agreement between BioMarin International Limited and NHS England

(The ancillary agreement contains commercial-in-confidence information and has been redacted from the managed access agreement)

BATTEN DISEASE FAMILY ASSOCIATION

Together we WILL make a difference

Registered Charity In England and Wales 1084908 Scotland SCO47408

Sheela Upadhyaya
Associate Director Highly Specialised Technologies
National Institute for Health and Care Excellence
10 Spring Gardens
London
SW1A 2BU

29 April 2019

Dear Sheela

Brineura (Cerliponase alfa)

We are writing to confirm some points that were made our last meeting on 17 April 2019. The points fall into two categories: 1) context, and 2) potential support.

1) *Context*

- Treatment take up: not all families have chosen to take up places on the clinical trial or compassionate use schemes; we are certain that not all families would opt for treatment when it is available for a wide range of personal reasons, including recognising that the treatment regime of fortnightly infusions in hospital is not feasible for all families.

- Parental consideration of risk: parents – with support from clinicians – carefully consider whether treatment is suitable for them. The treatment requires neurosurgery, which is itself a risky procedure, that parents have to consider carefully.

- Gene therapy: the BDFA and the Batten disease community are aware of a number of gene therapies for a range of the CLNs, including CLN2. While these are not yet in clinical trial, when these are developed and if successful, they will give parents and clinicians a choice. Not everyone will choose Brineura as their treatment option. It was noted during the meeting that the exit clauses in any Managed Access Agreement would need to take into account that some families may wish to transfer their child onto gene therapy, when available, from Brineura. We are aware that companies have raised the possibility of conducting a gene therapy trial on children who are receiving enzyme replacement therapy.

- Long term data: families are fully aware of the need to collect further data. They are committed to doing this and are willing to facilitate access to supplementary professional data, such as school reports, that would otherwise be confidential to the families.

- Eligibility criteria: families and clinicians are aware of how it could be not in a child's best interests if a child has passed beyond the point of meeting eligibility criteria for treatment. The parent experts are clear that parents are unlikely to undertake a course of action that would be detrimental to their child. One family has made the decision to remove their child from the trial for personal reasons, which demonstrates that parents will act in the best interests of their child. Parents are likely to recognise the time to stop treatment as they can see the quality of their child's life clearly.

2) *Potential support*

- Education of professionals to assist with earlier diagnosis: as well as any company run education programmes for professionals, especially pediatricians, that will enable earlier diagnosis, the BDFA is able to support this aim with a number of items. We hold regular Professionals' Days and will continue to do so. We are happy to explore webinars and other communication means to educate professionals. We already undertake regular visits to a range of hospitals as part of the LSD Collaborative as well as the BDFA and meet with clinical teams at their request. Education and diagnosis serves to raise awareness within a community and the system, which in turn can speed future diagnoses.

- School reports and other real world evidence: the BDFA is committed to exploring options about how to collect data from school report and other non-clinical sources, such as local physiotherapists, speech and language therapists and occupational therapists, as well as extra curricula activities to supplement clinical data collected as part of any Managed Access Agreement. The BDFA would like to explore how to best demonstrate quality of life in the evidence captured from a variety of sources, and the use of photographs and videos. The BDFA and parents would like to request that non-clinical data is included in any Managed Access Agreement.

- Patient diaries: the current draft Managed Access Agreement includes a reference to a seizure diary, however, it is now recognized that the content of the diaries should and could be wider in order to capture as complete picture of the impact of Brineura as possible. The BDFA is committed to working with parents and NICE to create a template for this diary. Areas to be covered could include events and activities that might not have been possible prior to treatment, demonstrating the wider impact on

BDFA
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other family members, activities the child participates in, enjoys and engages with, and skills gained.

- NICE guidelines: the BDFA, clinicians and parents are willing to input into the creation of NICE guidelines and/or be consulted on draft guidelines. We believe that such guidelines would help general paediatricians to request testing sooner that would reduce the diagnostic odyssey.

- Managed Access Agreement implementation: the BDFA is committed to assisting with the implementation of any Managed Access Agreement. Our team of two support officers would liaise with clinical teams to assist with any transfer of patients to any new site at which infusions will take place. Parents are also keen to be involved in ensuring there is a wide geographical spread of sites where the treatment is available.

We hope that this letter confirms what was discussed and the level of consideration given by parents about taking up the treatment for their child. We also hope that our stated level of support for the potential Managed Access Agreement for parents, clinicians and the BDFA will show the commitment of all patient parties. That commitment is based on the knowledge of how transformational Brineura is for some children with CLN2.

We look forward to hearing from you.

Kind regards

Samantha Barber
CEO, BDFA

Dr Paul Gissen
Consultant Paediatric Metabolic Diseases, Great Ormond Street Hospital

Lucy Carroll
Patient expert

Gail Rich
Patient expert