BATTEN DISEASE FAMILY ASSOCIATION
Together we WILL make a difference
Registered Charity in England and Wales 1205650

BDFA response to extension of Brineura Managed Access Agreement for the treatment of CLN2

The National Institute for Health and Care Excellence (NICE) has extended the Managed Access Agreement (MAA) for cerliponase alfa (Brineura®) for the treatment of neuronal ceroid lipofuscinosis type 2 (CLN2) by six months so that it can remain available through the NHS for that period and to allow the manufacturer (BioMarin) and NHS England (NHSE) to negotiate a long-term commercial agreement.

Read full statement from NICE here https://www.nice.org.uk/guidance/indevelopment/gid-hst10061

The Batten Disease Family Association CIO (BDFA) expresses deep dismay that after five years of the MAA, characterised by rigorous clinical assessments and burdensome data collection for families affected by CLN2, and two evaluation meetings held on 12th June and 5th September 2024, NICE has failed to issue any guidance about the outcome of its evaluations. This raises serious concerns about the efficacy of the NICE process, the purpose of the MAA, and the overall impact of the data collection and clinical assessments.

Given the circumstances, while an MAA extension may be helpful, we fear it may merely prolong the agonising wait for families seeking clarity about their children's futures.
The CLN2 community demands assurance that this extension is not a delaying tactic and that NICE, BioMarin, and NHS England will use this time effectively to reach a permanent access agreement for Brineura® as a matter of urgency.

Moreover, with data collection no longer required, we insist that rigorous clinical assessments also cease immediately.

Brineura® is a groundbreaking treatment and its availability over the last five years under an MAA between NHSE and BioMarin has been a lifeline to families who, before then, had no other treatment options.

There is no cure for this neurodegenerative disease and without treatment, children living with CLN2 risk deteriorating very rapidly.

Withdrawing access from the NHS treatment could lead to a situation where the future of a child's access to Brineura® is uncertain, and where one child with Batten disease has access and another does not.

Liz Brownnutt, Chief Executive of the BDFA:

> *"We do not want the MAA extension to lessen any sense of urgency for the parties involved. Coming to a long-term agreement to allow continued funding of this life-saving treatment will provide security to the patients and families who so desperately need it and will put an end to their agonising wait. We will not stop fighting until all eligible CLN2 patients in the UK are able to access Brineura®."*

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Further information

Batten Disease and CLN2

Batten disease is a group of 13 ultra-rare genetic disorders that affect the brain and nervous system.[i] CLN2 is a form of Batten disease which is manifested by language delay, seizures, rapid decline in a child's ability to walk, speak and see, alongside progressive dementia and untimely death. [ii] Children diagnosed with CLN2 have a greatly shortened life expectancy and require extensive care from their parents and wider family. There are currently 36 children on Brineura® throughout the whole of the UK.

The Batten Disease Family Association CIO

The BDFA is a national charity which aims to support families, raise awareness and facilitate research into Batten disease. The organisation works with children, young people, families and professionals across the UK. The BDFA was formed in 1998 and granted Registered Charity status in 2001. The BDFA's mission *"is to enable children and adults who are affected by Batten disease to live life to the full and to provide families with the care and support they need so that they do not walk this path alone"*. It is the only dedicated Batten disease charity in the UK and is run by a small, dedicated team and led by the Chief Executive Officer Liz Brownnutt.

Treatment for CLN2

Currently, there is no cure and few treatment options available for children living with CLN2 in the UK. Brineura®, an enzyme replacement therapy, is the only treatment that helps treat CLN2 disease.[iii] Brineura®, which is infused directly into the brain via an implanted device, has been shown to restore enzyme activity and slow the onset of disability. Clinical trials have demonstrated that patients receiving Brineura® showed 80% less decline in patients' motor and language skills, when compared to the natural progression of the disease, alongside reduced loss of brain tissue.[iv]

NICE re-evaluation of Brineura®

Following a NICE Highly Specialised Technology appraisal, a Managed Access Agreement was established in November 2019 to enable access to Brineura® and collect further information on its benefits before deciding on its routine availability through the NHS. Instead of issuing formal guidance, on 9th October 2024, NICE recommended the six-month extension of the MAA to allow for negotiations between BioMarin and NHSE.

References

[i] Children's Hospital, n.d. Batten Disease. Available at: Batten Disease | Boston Children's Hospital (childrenshospital.org) Accessed August 2024.

[ii] Great Ormond Street Hospital for Children, 2022. Children with rare genetic disease get world-first treatment to save sight. Available at: Children with rare genetic disease get world-first treatment to save sight | Great Ormond Street Hospital (gosh.nhs.uk) Accessed August 2024.

[iii] BioMarin, n.d. What is Brineura? Available at: About Brineura - Brineura Accessed August 2024.

[iv] BDFA. Brineura for CLNS2. Available at: http://www.bdfa-uk.org.uk/research-and-resources/brineura-for-cln2/ Access August 2024.