



1 April 2019

Prof T Irish
Vice Chair
National Institute for Health and Care Excellence
10 Spring Gardens
London SW1A 2BU

Dear Prof Irish

Re: Final Evaluation Determination – Cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2

Thank you for your letter of 18 March in response to the Batten Disease Family Association's appeal against the Final Evaluation Determination (FED) for Cerliponase alfa, the contents of which have been noted.

Since the NICE FED has been in consultation we observe that another country, Australia, has agreed that children with CLN2 should be treated with Cerliponase alfa and the treatment is being funded by their health service. The BDFFA is delighted for the children of Australia and saddened that the children with CLN2 in England should be less valued by our national health system.

We are disappointed with your initial consideration of the grounds put forward to challenge the FED. Separately, the grounds may not seem of consequence but taken together we believe that they show that the process has not fully considered the benefit of the treatment on the children with CLN2 and their families, which is unfair. However, we acknowledge that your final decision about our appeal is likely to remain the same.

Yours sincerely

████████████████████

Chair of Trustees

████████████████████

Chief Executive

Batten Disease Family Association
209-211 City Road London EC1V 1JN
For more information on supporting our work visit www.bdfa-uk.org.uk
Tel: 07876 682589 Email admin@bdfa-uk.org.uk
Registered Charity No: England: 1084908 Scotland: SCO47408