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NHS Foundation Trust

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Mr Andy McKeon
Vice-Chair
National Institute for Health and Care Excellence
10 Spring Gardens
London
SW1A 2BU

24 March 2017

Dear Mr McKeon

Final Evaluation Determination: Sebelipase alfa for treating lysosomal acid lipase deficiency (LAL D)

is currently on leave and I am writing on behalf of all four paediatric metabolic centres in England that manage infants and children with LAL-D.

I would like to thank you for your response dated the 09 Mar 2017, in which you upheld points 2.1, 2.2 and 2.4 in our appeal.

In addition to the above points that have been already upheld, we kindly request you to reconsider point 2.6 on ground (2) at the appeal hearing on the 25 Apr 2017.

We only intend to impress upon the appeal panel the similarities in the clinical spectrum of Hypophosphatasia and LAL-D and the strong evidence for efficacy of the new technologies in consideration to significantly and favourably alter the natural history of patients with neonatal/ infantile onset forms in particular. We don't seek to comment on the intricacies of the appraisal process of Asfotase Alfa or draw any comparisons to the appraisal of Sebelipase Alfa. In our view, the guidance for the two technologies in question is incongruent and inconsistent, from the point of view of similarities in the conditions and the known outcome of the novel treatments in question.

I would like to apologise for the delayed reply and thank the appeal panel for considering our belated response.

Yours Sincerely

Consultant in Clinical Inherited Metabolic Disorders, Birmingham Children's Hospital

And on behalf of:

Consultant in Clinical Inherited Metabolic Disorders, Birmingham Children's Hospital

Consultant in Clinical Inherited Metabolic Disorders, Birmingham Children's Hospital

Consultant in Clinical Inherited Metabolic Disorders, Royal Manchester Children's Hospital

Consultant in Clinical Inherited Metabolic Disorders, Royal Manchester Children's Hospital

Consultant in Clinical Inherited Metabolic Disorders, Evelina Children's Hospital, London

Consultant in Clinical Inherited Metabolic Disorders, Great Ormond Street Hospital, London