Sent by email to: XXXXXXXXXXXX

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Society for Mucopolysaccharide Diseases

MPS House

Repton Place

White Lion Road

Amersham

Buckinghamshire, HP7 9LP

31 March 2017

Dear XX XXXXX

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

Thank you for your letter of 22 March in response to my initial scrutiny letter of 9 March. I can now give you my final view on your appeal points.

Ground 1 (a)

**1.1(a) The Committee have failed to understand the superiority of sebelipase alfa, compared to other approved ERT treatments**

You offered no further comments on my preliminary view that this was not a valid appeal point. I confirm for the reasons set out in my letter of 9 March that this is not a valid appeal point.

**1.2(a) The Committee have failed to recognise the severity of the disease in the infant population.**

I have further considered this issue in light of the points set out in your letter of 22 March. I now consider this a valid appeal point under ground 2 as you have requested.

Ground 2

**2.1 The Committee’s statement relating to infants coming off treatment reflects their lack of understanding and compassion for a fatal disease.**

I confirm that this is not a valid appeal point for the reasons set out in my letter of 9 March.

**2.2 The Committee’s statement relating to patient representation was untrue and inappropriate.**

I have further considered this issue in light of the points you make in your letter of 22 March. However, I am not persuaded that this is a valid appeal point. It is clear in paragraph 5.2 that the Committee fully accepted the testimony of patient experts in respect of babies presenting with rapidly progressive disease. Their subsequent comments in that paragraph were as I noted in my earlier letter - the Committee did consider that the evidence of the patient experts reflected their own or their child’s experience of having the more severe or severest forms of the disease because they had taken part in the clinical trials but this did not lead the Committee to discount their evidence, simply to note that the quality of life effects of symptoms in the less severe forms of the disease were less clear.

**2.3 The Committee’s reservations on the long-term health benefits of sebelipase alfa not being achieved and the benefits being highly uncertain due to the limited data available (para 5.22) to be invalid and subjective.**

I noted in my letter of 9 March that this was a valid appeal point.

In summary therefore, my final view is that 1.2(a) (now reassigned to ground 2) and 2.3 are valid appeal points. There will be an oral hearing and the Secretariat will have been in touch about the arrangements.

Yours sincerely

Andy McKeon

Vice-Chair

National Institute for Health and Care Excellence