Society for Mucopolysaccharide Diseases
MPS House, Repton Place
White Lion Road, Amersham
Bucks, HP7 9LP

0345 389 9901
mps@mpssociety.org.uk
www.mpssociety.org.uk

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

22 March 2017

Dear Andy,

Thank you for your response to the Society's appeal against the FED for Sebelipase alfa. Please find below our observations for further consideration against the appeal points that were not upheld.

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

I am not minded to consider this a valid appeal point. The Committee clearly recognised the severity of the disease in the infant population, noting in paragraph 4.1 that, for infants, survival is less than 12 months and the median life expectancy for those with rapid progression of the disease is 3.7 months. It is however an absolute requirement of highly specialised technology evaluations that NICE consider both costs and effectiveness and come to a balanced judgement, bearing in mind the resources available.

You refer to treatment for Hypophosphatasia. Although NICE should be consistent between appraisals, each appraisal is so dependent on the precise evidence for the costs and benefits of each treatment that it is very difficult to make out an arguable case for inconsistency. For there to be a valid appeal under Ground 1(a) there would need to be an aspect of the process in reaching that decision that was unfair, or a clear and unexplained inconsistency with a truly parallel past appraisal, not simply that the recommendation was disadvantageous to a particular group.

MPS Society's observation.
It is our opinion that paragraph 4.1 reflects the summary of the company's submission and not the view of the committee. We also believe that the committee's decision not to approve treatment for infants was not balanced but biased towards cost rather than the effectiveness and lifesaving properties of Sebelipase alfa.

We maintain that the committee's decision in relation to the infant population was unreasonable, given their recent recommendation for the use of treatment for the perinatal and infant population for Hypophosphatasia and the fact that the current and projected number of infants for LAL D is small compared to other patient populations who have access to approved treatments for rare diseases.

We would therefore ask that this point and our further response be viewed under ground 2 and upheld.

Ground 2

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

I am not minded to consider this a valid appeal point. There is no suggestion in the FED that the Committee felt that patient expert views were not given transparently and honestly, or that the Committee was critical of them. They did not use the term "biased". 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.

MPS Society's observation
In response to the above, we would like to again point out that for the infant population there is no spectrum of disease and therefore the only view the parent expert could give is that of the severest form of the disease where quality of life is not existent without access to this life saving treatment.

We thank you for upholding point 2.3 and hope that you reconsider the validity of our response to the above points.

Yours sincerely

Advocacy Support Team Manager