2nd Floor 2 Redman Place

London E20 1JQ

United Kingdom

+44 (0)300 323 0140

Sent by e-mail only: XXXXXXXXXXXXXXX[;](mailto:r.lachmann@nhs.net) XXXXXXXXXXXXXXXXX; XXXXXXXXXXXXXXX;

British Inherited Metabolic Diseases Group (BIMDG) Tuesday 26 March 2024

Dear XXXXXXXXXXXXXXX, XXXXXXXXXX, and XXXXXXXXX.

# Re: Final Draft Guidance – Olipudase alfa for treating acid sphingomyelinase deficiency (Niemann-Pick disease) type AB and type B [ID3913]

Thank you for your letter of 18 March 2024, lodging an appeal against the above Final Draft Guidance (FDG). Dr Chakravarty is temporarily unavailable and so in accordance with paragraph 3.1 of NICE's Guide to the technology appraisal and highly specialised technologies appeal process, I am conducting initial scrutiny on this occasion.

Introduction

The Institute's appeal procedures provide for an initial scrutiny of points that an appellant wishes to raise, to provide an initial view on whether they are within the permitted grounds of appeal ("valid") and are at least arguable. The permitted grounds of appeal are:

* 1(a) NICE has failed to act fairly, or
* 1(b) NICE has exceeded powers;
* (2) the recommendation is unreasonable in the light of the evidence submitted to NICE.

This letter sets out my initial view of the point of appeal you have raised: principally whether it falls within any of the grounds of appeal, or whether further clarification is required of any point. Only if I am satisfied that your point contains the necessary information, is arguable, and falls within any one of the grounds will your appeal be referred to the Appeal Panel.

You have the opportunity to comment on this letter in order to elaborate on or clarify any of the points raised before I will make my final decision as to whether your appeal point should be referred on to the Appeal Panel.

Initial View

***Ground 1(a): In making the assessment that preceded the recommendation, NICE has failed to act fairly***

# Appeal point 1(a).1: The decision not to recommend treatment with olipudase alfa is unfair on patients with Niemann-Pick disease type B because similar enzyme replacement therapies for

DOCUMENT3 Page 1 of 3



**other lysosomal storage disorders which are less cost effective have received positive recommendations. We believe that NICE is using entirely different criteria to assess novel treatments for diseases where no disease modifying therapy is available, to those criteria they are using to assess new therapies for diseases where other licensed treatment options already exist.**

I understand your appeal point, in substance, to be a challenge to NICE's processes for highly specialised technology evaluation. You argue that other drugs for different conditions have been approved, despite being less clinically effective than olipudase alfa is for Niemann-Pick disease type B. You argue that it is harder for olipudase alfa to be considered cost effective than those other drugs, because the incremental costs of those drugs were measured against existing standard of care therapies; whereas olipudase alfa is measured against best supportive care, because no other disease modifying therapy is currently available. You say that this is unfair, because the incremental cost of a new technology, measured against no existing technology, is likely to be much higher than the incremental cost of a new technology measured against an existing technology used for that disease.

I am not minded to refer this appeal point to the Appeal Panel. You have not argued that the Committee has departed from *NICE health technology evaluations: the Manual*, which sets out the methods and processes by which NICE committees are obliged to carry out technology evaluations. A challenge to the contents of the Manual itself cannot be brought by way of an appeal against a particular evaluation. In the absence of any argument that the Committee departed from NICE's established processes, I do not see a basis for a claim that the Committee has acted unfairly.

In respect of your challenge to the recommendations made for other technologies based on their existing comparators, you may be reassured by paragraphs 6.2.3 and 6.2.4 of the Manual, which set out in some detail the bases on which committees identify comparators, including mitigations if the evaluation suggests that established practice may not be considered a good use of NHS resources relative to another available treatment. It also explains that the committee will consider whether that other technology is so embedded that its use will continue unless it is replaced by a new technology. I would also note that the full complement of QALYs did accrue to the technology in the current evaluation precisely because there was no comparator treatment.

Conclusion

The above sets out my initial views on your appeal point.

You are entitled to submit further clarification and/or evidence to me within the next 10 working days, and I will then give a final decision on whether your appeal point is to be put before an appeal panel.

Once I have made my final decision, and where there is more than one appellant, each appellant will receive the valid appeal points of the other appellants and their redacted appeal letter. This is to enable appellants to avoid duplication at the hearing where there are overlapping appeal points. If the appeal letter and/or responses to scrutiny contain confidential information please ensure you have provided a version with this information redacted by 18 April 2024.

Ordinarily appeals are conducted on the basis of the appellants’ written appeal letters, and the material generated during the appraisal process. Use of additional written material is discouraged, and the panel cannot receive any new evidence. If, exceptionally, you feel there is written material that will not be before the panel that you would wish to rely on you must let the NICE Appeal team know by return of letter, indicating what the material is, why it is desirable to submit it, and when it will be available, by no later than 3 May 2024. Please note that the appeal panel cannot accept papers that are tabled late or ad hoc, as this affects the preparation of the panel and other parties for the appeal.

Yours sincerely

XXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXX

Sharmila Nebhrajani OBE

Non-Executive Director & Chairman

National Institute for Health and Care Excellence