03 April 2024

Sharmila Nebhrajani OBE

Non-Executive Director & Chairman

National Institute for Health and Care Excellence 2nd Floor

2 Redman Place London E20 1JQ

Dear Ms Nebhrajani,

**Re: Final Appraisal Determination –** Olipudase alfa for treating Niemann-Pick disease types A and B [ID3913]

Thank you for your letter of 26th or March in response to our appeal against the above final draft guidance.

You are not minded to refer our appeal because we are not arguing that the committee departed from NICE guidance in reaching its decision. This may be correct in a purely procedural sense, but the decision they have reached is unfair when viewed in the context of other recent NICE appraisal recommendations. As we have pointed out in our appeal, NICE are not applying the same rigorous cost- effectiveness criteria to technologies where there is an existing treatment as they are to ones where there is not. This is distorting the decision-making process to the point where some highly expensive drugs for rare diseases are being approved by a single technology appraisal due to a favourable ICER, but others are failing the highly specialised technology assessment process, which was meant to be more favourable, because it does perform a full cost-effectiveness assessment. This is clearly unfair and exposes fundamental flaws in how NICE is going about fulfilling its mission of getting ‘the best care to patients, fast, while ensuring value for the taxpayer’.

If NICE decides that an appeal of this guidance is not an appropriate forum to address these deep-seated issues, then we would ask what is the mechanism via which NICE can openly consider these iniquitous and poor value decisions?

Yours sincerely,

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on behalf of all below

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Consultant in Inherited Metabolic Disease

University College London Hospitals NHS Foundation Trust XXXXXXXXXXXX

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Consultant in Inherited Metabolic Disease

University College London Hospitals NHS Foundation Trust XXXXXXXXXXXXXXX

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Consultant in Paediatric Inherited Metabolic Disease Manchester University NHS Foundation Trust

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