

Highly Specialised Technology Committee Interests Register

Topic: Cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2 (review of HST12) [ID6145]

Publication Date: TBC

Name	Role with NICE	Type of interest	Description of interest	Interest declared	Comments
Dr Paul Arundel	HST Committee Member	Non-Financial Professional and Personal Interests	I am or have been principal investigator for BMN111-901, -301, -302, -206, -208, -209 - natural history study and clinical trials of vosoritide in achondroplasia - sponsored by BioMarin pharma - BMN111-302, -208, and -209 are the studies which are ongoing. These are all conducted within the NHS and costs are paid to employing NHS Trust according to contracting and not to me. I am a joint author (along with other investigators) of various research papers and abstracts describing the methods and results of these projects.	12/06/2024 22/08/2024 03/04/2025 24/06/2025	It was agreed that Paul's declaration would not prevent them from participating in discussions on this evaluation.
Dr Paul Arundel	HST Committee Member	Financial Interest	Received honorarium (and travel and accommodation) from BioMarin pharma to act as chair for satellite symposium at International Conference on Children's Bone Health on 22nd June 2019 in Salzburg, Austria entitled "The role	12/06/2024 22/08/2024 03/04/2025	It was agreed that Paul's declaration would not prevent them from participating in discussions on this evaluation.

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			of C-type natriuretic peptide in bone development". Interest ceased on that date.	24/06/2025	
Stuart Mealing	HST Committee Member	Non-Financial Professional Interests	I undertook work for BioMarin in 2019 and 2020 to support a NICE submission for their product Kuvan in Phenylketonuria. I have not worked with them since.	12/06/2024 23/08/2024 08/07/2025	It was agreed that Stuart's declaration would not prevent them from participating in discussions on this evaluation.
Emtiyaz Chowdhury	HST Committee Member	Non-Financial Professional Interests	I am employed by Parexel. Parexel is a CRO that offers services to both manufacturers. I am not aware of and have not been involved with any activities related to the technologies under discussion.	12/06/2024 31/08/2024 03/04/2025 30/06/2025	It was agreed that Emityaz's declaration would not prevent them from participating in discussions on this evaluation.
Liz Brownnutt	Clinical expert	Organisational interests (BDFA)	The BDFA is a member of the LSD (Lysosomal Storage Disorder) Collaborative and the Newborn Screening Collaborative and as part of my role as Interim CEO, I attend all these meetings on behalf of the BDFA. I also attend meetings for the Specialist Healthcare Alliance and the Genetic Alliance Rare Disease Action Plan Patient Advisory Group, also their Patient Empowerment	12/06/2024 03/04/2025 10/07/2025	It was agreed that Ms Brownnutt's declaration would not prevent them from providing expert advice to the committee.

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			<p>group. The BDFA also has membership for the Disabled Children's Partnership, Together for Short Lives and Eurodis</p> <p>BioMarin grants to the BDFA March 2023:</p> <ul style="list-style-type: none"> • Patient Travel Support for treatment -£10,000 • BDFA-led Research Study - £10,000 • Regional work supporting the development of new treatment centres across the UK - £4,000 • BDFA Conference, later redirected to family support services due to conference being cancelled - £15,000. 		
Dipak Ram	Clinical Expert	Professional Interests	Ongoing liaison with BioMarin regarding MAA and educational events hosted by BioMarin.	12/06/2024 03/04/2025	It was agreed that Mr Ram's declaration would not prevent them from providing expert advice to the committee.

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Paul Gissen	Clinical Expert	Professional Interests	<ul style="list-style-type: none"> • I am an investigator on clinical trials in CLN2 disease. • I am part of the scientific and medical committee for BDFA. • I have published papers on cerliponase alfa treatment of CLN2 patients. • I have received research grants related to the CLN2 patient treatment. 	12/06/2024 03/04/2025 10/07/2025	It was agreed that Mr Gissen's declaration would not prevent them from providing expert advice to the committee.
Dr Natalia Kunst	HST Committee Member	Non-Financial Professional Interests	I worked as part of the EAG for Cerliponase alfa.	18/03/2025 20/06/2025	It was agreed that Natalia's declaration would prevent them from participating in discussions on this evaluation.