

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Highly Specialised Technologies Evaluation

Olipudase alfa for treating acid sphingomyelinase deficiency (Niemann-Pick disease type B and AB)

Stakeholder List

Consultees	Commentators (no right to submit or appeal)
<p><u>Companies</u></p> <ul style="list-style-type: none"> • Sanofi (olipudase alfa) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • Brain Charity • Findacure • Genetic Alliance UK • Gene People • Metabolic Support UK • Neurological Alliance • Niemann-Pick UK • South Asian Health Foundation • Specialised Healthcare Alliance • UK LSD Patient Collaborative Group <p><u>Professional groups</u></p> <ul style="list-style-type: none"> • Association of British Neurologists • Association of Genetic Nurses and Counsellors • British Association of Occupational Therapists and College of Occupational Therapists • British Association for the Study of the Liver • British Geriatrics Society • British Inherited Metabolic Disease Group • British Neuropathological Society • British Paediatric Neurology Association • British Society for Gene and Cell Therapy • British Society for Genetic Medicine • Institute of Neurology 	<p><u>General</u></p> <ul style="list-style-type: none"> • All Wales Therapeutics and Toxicology Centre • Allied Health Professionals Federation • Board of Community Health Councils in Wales • British National Formulary • Care Quality Commission • Cell and Gene Therapy Catapult • Department of Health, Social Services and Public Safety for Northern Ireland • Healthcare Improvement Scotland • Inherited Metabolic and Lysosomal Disease Service, Cardiff and Vale UHB • Medicines and Healthcare products Regulatory Agency • National Association of Primary Care • National Pharmacy Association • National Services Division • NHS Alliance • NHS Confederation • Scottish Medicines Consortium • Welsh Government • Welsh Health Specialised Services Committee <p><u>Possible comparator companies</u></p> <ul style="list-style-type: none"> • None <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> • Brain Research UK • Cochrane Cystic Fibrosis & Genetic Disorders Group • Cochrane Hepato-Biliary Group

Stakeholder list for the highly specialised evaluation of olipudase alfa for treating acid sphingomyelinase deficiency (Niemann-Pick disease type B and AB) ID3913

Issue date: June 2022

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Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • National Metabolic Biochemistry Network • Neonatal and Paediatric Pharmacists Group • Royal College of General Practitioners • Royal College of Nursing • Royal College of Paediatrics & Child Health • Royal College of Pathologists • Royal College of Physicians • Royal Pharmaceutical Society • Royal Society of Medicine • UK Clinical Pharmacy Association • UK Genetic Testing Network <p><u>Others</u></p> <ul style="list-style-type: none"> • Addenbrooke's Lysosomal Disorders Unit • Birmingham Children's Hospital NHS Foundation Trust Lysosomal Storage Disorders Unit • Department of Health and Social Care • Great Ormond Street Hospital Metabolic Unit • NHS England • Royal Free Lysosomal Storage Disorders Unit • Royal Manchester Children's Hospital • Salford Royal NHS Foundation Trust Mark Holland Metabolic Unit • University College London Hospitals NHS Foundation Trust Lysosomal Disorders Unit • University Hospitals Birmingham, Inherited Metabolic Diseases team • Willink Unit, Genetic Medicine, Central Manchester Foundation Trust 	<ul style="list-style-type: none"> • Cochrane Metabolic & Endocrine Disorders Group • Cochrane UK • Genomics England • MRC Clinical Trials Unit • National Institute for Health Research <p><u>Associated Public Health Groups</u></p> <ul style="list-style-type: none"> • Public Health Wales • UK Health Security Agency

NICE is committed to promoting equality, eliminating unlawful discrimination and fostering good relations between people who share a protected characteristic and those who do share it. Please let us know if we have missed any important organisations from the provisional stakeholder list, and which organisations we should include that have a particular focus on relevant equality issues.

Definitions:Consultees

Organisations that accept an invitation to participate in the evaluation; the manufacturer(s) or sponsor(s) of the technology; national professional organisations; national patient organisations; the Department of Health and Social Care and relevant NHS organisations in England.

The company that markets the technology is invited to prepare a submission dossier, can respond to consultations, nominate clinical experts and has the right to appeal against the Final Appraisal Document (FAD).

All non- company consultees are invited to prepare a submission dossier respond to consultations on the draft scope, the Assessment Report and the Appraisal Consultation Document. They can nominate clinical or patient experts and have the right to appeal against the Final Appraisal Document (FAD).

Commentators

Organisations that engage in the evaluation process but that are not asked to prepare an evidence submission or statement, are able to respond to consultations and they receive the final evaluation documentation for information only, without right of appeal. These organisations are: manufacturers of comparator technologies; Healthcare Improvement Scotland; the relevant National Collaborating Centre (a group commissioned by the Institute to develop clinical guidelines); other related research groups where appropriate (for example, the Medical Research Council [MRC], other groups (for example, the NHS Confederation, NHS Alliance, and the British National Formulary).

All non-company organisations can nominate clinical or patient experts to present their personal views to the Appraisal Committee.