

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Highly Specialised Technologies Evaluation

Sebelipase alfa for treating Wolman disease ID3995

Provisional Stakeholder List

Consultees	Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> • Alexion AstraZeneca Rare Disease (sebelipase alfa) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • Anthony Nolan • Black Health Agency for Equality • British Liver Trust • Children's Liver Disease Foundation • Contact a Family • Gene People • Genetic Alliance UK • Heart UK • Jnetics • Liver4Life • Metabolic Support UK • MPS Society • South Asian Health Foundation • Specialised Healthcare Alliance <p><u>Professional groups</u></p> <ul style="list-style-type: none"> • Association of Genetic Nurses and Counsellors • Association of Surgeons of Great Britain and Ireland • British Association for the Study of the Liver • British Inherited Metabolic Disease Group • British Society for Gene and Cell Therapy • British Society for Genetic Medicine • British Society for Human Genetics • British Society of Blood and Marrow Transplantation • British Transplantation Society • European Lysosomal Storage Disorder Nurses Group • National Metabolic Biochemistry Network • Neonatal and Paediatric Pharmacists Group • NHS Blood and Transplant 	<p><u>General</u></p> <ul style="list-style-type: none"> • Allied Health Professionals Federation • All Wales Therapeutics and Toxicology Centre • Association of Renal Industries • 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 Commercial Medicines Unit • NHS Confederation • Scottish Medicines Consortium • Welsh Health Specialised Services Committee • Welsh Government <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"> • Cochrane Cystic Fibrosis and Genetic Disorders Group • Cochrane Hepato-Biliary Group • Cochrane UK • Foundation for Liver Research • Health Research Authority • MRC Clinical Trials Unit • National Institute for Health Research • Society for the Study of Inborn Errors of Metabolism

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Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • Royal College of General Practitioners • Royal College of Nursing • Royal College of Paediatrics and Child Health • Royal College of Pathologists • Royal College of Physicians • Royal Pharmaceutical Society • Royal Society of Medicine • Society for DGH Nephrologists • UK Clinical Pharmacy Association • UK Genetic Testing Network <p><u>Others</u></p> <ul style="list-style-type: none"> • Birmingham Children’s Hospital NHS Foundation Trust, Lysosomal Storage Disorders Unit • Cambridge University Hospitals NHS Foundation Trust, Addenbrooke's Lysosomal Disorders Unit • Central Manchester Foundation Trust, Willink Unit, Genetic Medicine Department of Health • Evelina London Children’s Hospital, St Thomas’ Hospital • Great Ormond Street Hospital, Metabolic Unit • London Guy’s Hospital Genetic Centre • Manchester Centre for Genomic Medicine • NHS England • Queen Elizabeth Hospital, London • Royal Free Lysosomal Storage Disorders Unit • Salford Royal NHS Foundation Trust, Mark Holland Metabolic Unit • University College London Hospital, Charles Dent Metabolic Unit • University Hospital Birmingham Foundation Trust, Department of Endocrinology 	<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 not. Please let us know if we have missed any important organisations from the stakeholder list, and which organisations we should include that have a particular focus on relevant equality issues.

Definitions:

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Consultees

Organisations that accept an invitation to participate in the evaluation; the company that markets the technology; national professional organisations; national patient organisations; the Department of Health and Social Care and the Welsh Government and relevant NHS organisations in England.

The company that markets the technology is invited to make an evidence submission, 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 submit a statement¹, respond to consultations, 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 FAD for information only, without right of appeal. These organisations are: companies that market comparator technologies; Healthcare Improvement Scotland; related research groups where appropriate (for example, the Medical Research Council [MRC], National Cancer Research Institute); other groups (for example, the NHS Confederation, NHS Alliance, and the British National Formulary).

All non-company commentators are invited to nominate clinical or patient experts.

¹ Non company consultees are invited to submit statements relevant to the group they are representing.