NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Highly Specialised Technology Evaluation

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

Final stakeholder list

Consultees	Commentators (no right to submit or appeal)
Company • BioMarin (cerliponase alfa) Patient/carer groups • Batten Disease Family Association • Beacon • Contact • Genetic Alliance UK • Neurological Alliance • South Asian Health Foundation • Specialised Healthcare Alliance • Together for Short Lives Professional groups • Association of British Neurologists • Association of Genetic Nurses and Counsellors • British Inherited Metabolic Disease Group • British Neuropathological Society • British Paediatric Neurology Association • British Society for Genetic Medicine • Institute of Neurology • National Metabolic Biochemistry Network • Neuromodulation Society of UK and Ireland • Primary Care and Community Neurology Society • 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 • UK Clinical Pharmacy Association	General • All Wales Therapeutics and Toxicology Centre • Allied Health Professionals Federation • Board of Community Health Councils in Wales • British National Formulary • Care Quality Commission • Department of Health, Social Services and Public Safety for Northern Ireland • Healthcare Improvement Scotland • Inherited Metabolic and Lysosomal Disease Services, Cardiff and Vale UHB • Medicines and Healthcare products Regulatory Agency • National Association of Primary Care • National Pharmacy Association • National Services Division • Neurological Alliance of Scotland • NHS Alliance • NHS Confederation • Scottish Medicines Consortium • Wales Neurological Alliance • Welsh Government • Welsh Health Specialised Services Committee Comparator manufacturers • None Relevant research groups • Brain Research UK • Cochrane Cystic Fibrosis and Genetic Disorders Group • Cochrane Multiple Sclerosis and Rare Diseases of the Central Nervous System Group • MRC Clinical Trials Unit • National Institute for Health Research

Consultees	Commentators (no right to submit or appeal)
Others • Birmingham Children's Hospital NHS Foundation Trust Lysosomal Storage Disorders Unit - • Bristol Royal Hospital for Children • Cambridge University Hospitals NHS Trust – Addenbrooke's Lysosomal Disorders Unit • Department of Health and Social Care • Great Ormond Street Hospital Metabolic Unit • Manchester NHS Foundation Trust • Mark Holland Metabolic Unit, Salford Royal NHS Foundation Trust • National Hospital for Neurology and Neurosurgery Charles Dent Metabolic Unit, UCLH • Newcastle upon Tyne Hospitals NHS Foundation Trust, Great North Children's Hospital • NHS England • Royal Free London NHS Foundation Trust, Lysosomal Storage Disorders Unit • University Hospital Birmingham Foundation Trust, Department of Endocrinology • Willink Unit, Genetic Medicine, Central Manchester Foundation Trust	• Rare Disease Research Partners • Society for the Study of Inborn Errors of Metabolism Associated Public Health Groups • 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:

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 Draft Guidance (FDG).

All non-company consultees are invited to submit a statement[1], respond to consultations, nominate clinical or patient experts and have the right to appeal against the Final Draft Guidance (FDG).

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 FDG 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.