NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Pegunigalsidase alfa for treating Fabry disease ID3904

Provisional Stakeholder List

Consultees	**Commentators (no right to submit or appeal)**
Company • Chiesi (pegunigalsidase alfa) Patient/carer groups • Action for Sick Children • Contact • Findacure • Gene People • Genetic Alliance UK • LSD Patient Collaborative • Metabolic Support UK • MPS Society • National Children's Bureau • South Asian Health Foundation • Specialised Healthcare Alliance Professional groups • Association of Genetic Nurses and Counsellors • British Inherited Metabolic Disease Group • British Society for Gene and Cell Therapy • British Society for Genetic Medicine • British Society for Paediatric Endocrinology and Diabetes • 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 Others	General • 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 Health Specialised Services Committee Possible comparator companies • Amicus Therapeutics (migalastat) • Sanofi Genzyme (agalsidase beta) • Takeda (agalsidase alfa) Relevant research groups • Cochrane Cystic Fibrosis & Genetic Disorders Group • Cochrane Metabolic and Endocrine Disorders Group • Cochrane UK • Genomics England • MRC Clinical Trials Unit

Consultees	Commentators (no right to submit or appeal)
• Birmingham Childrens 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 and Social Care • Great Ormond Street Hospital Metabolic Unit • National Hospital for Neurology and Neurosurgery Charles Dent Metabolic Unit • NHS England • NHS North East Essex CCG • NHS Wigan Borough CCG • Royal Free Lysosomal Storage Disorders Unit • Salford Royal NHS Foundation Trust Mark Holland Metabolic Unit • University Hospital Birmingham Foundation Trust, Department of Endocrinology • Welsh Government	• National Institute for Health Research <u>Associated Public Health Groups</u> • 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 lists in the matrix, 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 appraisal; 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.

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.

Commentators

Organisations that engage in the appraisal process but that are not asked to prepare an evidence submission or statement, are able to respond to consultations and they receive the final draft guidance 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.

