

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Cipaglucosidase alfa with miglustat for treating Pompe disease ID3771

Provisional stakeholder list

Consultees	Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> • Amicus Therapeutics Europe (cipaglucosidase alfa) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • Association for Glycogen Storage Disease • Contact • Findacure • Genetic Alliance • Lysosomal Storage Disorders Collaborative • Metabolic Support UK • Muscular Dystrophy UK • Pompe Support Network • South Asian Health Foundation • Specialised Healthcare Alliance <p><u>Professional groups</u></p> <ul style="list-style-type: none"> • Association for Respiratory Technology & Physiology • Association of British Neurologists • Association of Genetic Nurses & Counsellors • Association of Respiratory Nurse Specialists • British Association of Endocrine and Thyroid Surgeons • British Cardiovascular Society • British Geriatrics Society • British Inherited Metabolic Diseases Group • British Institute of Musculoskeletal Medicine • British Myology Society • British Society for Gene & Cell Therapy 	<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 • Department of Health, Social Services and Public Safety for Northern Ireland • Healthcare Improvement Scotland • Hospital Information Services - Jehovah's Witnesses • 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 <p><u>Possible comparator companies</u></p> <ul style="list-style-type: none"> • Accord Healthcare (miglustat) • Janssen-Cilag (miglustat) • Piramal Critical Care (miglustat) • Sanofi (Alglucosidase alfa) <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> • Bone Research Society • British Association for Lung Research • Cochrane Airways Group • Cochrane Cystic Fibrosis & Genetic

Provisional stakeholder list for the single technology appraisal of cipaglucosidase alfa with miglustat for treating Pompe disease ID3771

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Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • British Society for Genetic Medicine • British Society for Human Genetics • British Society of Rehabilitation Medicine • British Thoracic Society • ILD-IN: Interstitial Lung Diseases Interdisciplinary Network • National Heart & Lung Institute • National Metabolic Biochemistry Network • Neonatal and Paediatric Pharmacists Group • Physiotherapy Pain Association • Royal College of General Practitioners • Royal College of Nursing • Royal College of Pathologists • Royal College of Physicians • Royal College of Surgeons • Royal Pharmaceutical Society • Royal Society of Medicine • UK Clinical Pharmacy Association • UK Genetic Testing Network • UK Kidney Association • UK Renal Pharmacy Group <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 • Department of Health and Social Care • Great Ormond Street Hospital for Children NHS Foundation Trust, Metabolic Unit • Manchester University NHS Foundation Trust • NHS Blackburn with Darwen CCG • NHS England • NHS Wyre Forest CCG • Royal Free London NHS Foundation Trust, Lysosomal Storage Disorders Unit • Salford Royal NHS Foundation Trust, Mark Holland Metabolic Unit 	<p>Disorders Group</p> <ul style="list-style-type: none"> • Cochrane Metabolic & Endocrine Disorders Group • Cochrane Musculoskeletal Group • Cochrane UK • Genomics England • MRC Clinical Trials Unit • National Institute for Health Research • Orthopaedic Research UK <p><u>Associated Public Health Groups</u></p> <ul style="list-style-type: none"> • Public Health England • Public Health Wales

Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • University College London Hospital NHS Foundation Trust • University Hospitals Birmingham NHS Foundation Trust Lysosomal Storage Disorders • Welsh Government 	

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 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 specialists 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 specialists or patient experts and have the right to appeal against the Final Appraisal Document (FAD).

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 FAD for information only, without right of appeal. These organisations are: companies that market comparator technologies; Healthcare Improvement Scotland; other 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 specialists or patient experts.

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