

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Highly Specialised Technologies Evaluation

Avalglucosidase alfa for treating Pompe disease ID3737

Provisional stakeholder list of consultees and commentators

Consultees	Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> • Sanofi Genzyme (avalglucosidase alfa)) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • Arthritis and Musculoskeletal Alliance • Association for Glycogen Storage Disease (AGSD) • British Lung Foundation • Findacure • Genetic Alliance • Muscular Dystrophy UK • Muslim Council of Great Britain • NARA – The Breathing Charity • Pompe UK • 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 Inherited Metabolic Diseases Group • British Institute of Musculoskeletal Medicine • British Myology Society • British Society for Gene & Cell Therapy • British Society for Genetic Medicine • British Society for Human Genetics 	<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 Government • Welsh Health Specialised Services Committee <p><u>Possible comparator companies</u></p> <ul style="list-style-type: none"> • Sanofi Genzyme (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 Disorders Group • Cochrane Metabolic & Endocrine Disorders Group • Cochrane Musculoskeletal Group

Stakeholder list for the highly specialised technologies evaluation of avalglucosidase alfa for treating Pompe disease ID3737. Issue date: September 2021

Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • British Society of Rehabilitation Medicine • British Thoracic Society • ILD-IN: Interstitial Lung Diseases Interdisciplinary Network • National Heart & Lung Institute • National Metabolic Biochemistry Network • 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 • UK Clinical Pharmacy Association • UK Genetic Testing Network <p><u>Others</u></p> <ul style="list-style-type: none"> • Department of Health and Social Care • NHS England • Birmingham Children's Hospital NHS Foundation Trust Lysosomal storage disorder service • Cambridge University Hospitals NHS Foundation Trust Lysosomal disorder service • Great Ormond Street Hospital for Children NHS Foundation Trust Metabolic medicine department • Manchester University NHS Foundation Trust • Royal Free London NHS Foundation Trust lysosomal storage disorders • Salford Royal NHS Foundation Trust The Mark Holland metabolic unit • University College London Hospital NHS Foundation Trust Metabolic disease service • University Hospitals Birmingham NHS Foundation Trust Lysosomal storage disorders 	<ul style="list-style-type: none"> • 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

NICE is committed to promoting equality and eliminating unlawful discrimination. Please let us know if we have missed any important

organisations from the provisional stakeholder list, and which organisations we should include who have a particular focus on relevant equality issues.

PTO FOR DEFINITIONS OF CONSULTEES AND COMMENTATORS

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