

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

**Rebisufligene etisparvovec for treating mucopolysaccharidosis type IIIA
ID6540**

Provisional Stakeholder List

Provisional Consultees	Provisional Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> • Ultragenyx (rebisufligene etisparvovec) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • Beacon • Brain and Spine Foundation • Brain Charity • Child Growth Foundation • Gene People • Genetic Alliance UK • Jnetics • Metabolic Support UK • MPS Society • Neurological Alliance • Restricted Growth Association • South Asian Health Foundation • Specialised Healthcare Alliance <p><u>Healthcare professional groups</u></p> <ul style="list-style-type: none"> • Association of British Neurologists • Association of Genetic Nurses & Counsellors • British Inherited Metabolic Disease Group • British Neuropathological Society • British Paediatric Neurology Association (BPNA) • British Society for Gene and Cell Therapy • British Society for Genetic Medicine • British Society for Paediatric Endocrinology & Diabetes • National Metabolic Biochemistry Network 	<p><u>General</u></p> <ul style="list-style-type: none"> • All Wales Inherited Metabolic Disease Service • 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 - Northern Ireland • Healthcare Improvement Scotland • Medicines and Healthcare products Regulatory Agency • National Association of Primary Care • National Pharmacy Association • National Services Division • Neurological Alliance of Scotland • NHS Confederation • NHS Wales Joint Commissioning Committee • Scottish Medicines Consortium • Welsh Government • Wales Neurological Alliance <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"> • Brain Research UK • Genomics England • Institute of Neurology • MRC Clinical Trials Unit • National Institute for Health Research

Provisional Consultees	Provisional Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • National Neurosciences Advisory Group • Neonatal and Paediatric Pharmacy Group • 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 <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 and Social Care • Great Ormond Street Hospital Metabolic Unit • NHS England • 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:

Consultee or commentator stakeholders are provisional until a signed Confidentiality Agreement & Undertaking form is submitted to NICE at the evaluation stage. Participating stakeholders will be listed on the project information page for the evaluation.

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 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 relevant to the group they are representing, 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]); other groups (for example, the NHS Confederation and the British National Formulary).

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