

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

**Eladocagene exuparvovec for treating aromatic L-amino acid
decarboxylase deficiency ID3791**

Provisional Stakeholder List

Consultees	Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> • PTC Therapeutics (eladocagene exuparvovec) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • AADC Trust • Action for Sick Children • Brain and Spine Foundation • Brain Charity • Genetic Alliance • Metabolic Support UK • Neurological Alliance • South Asian Health Foundation • Specialised Healthcare Alliance • Sue Ryder <p><u>Professional groups</u></p> <ul style="list-style-type: none"> • Association of Anaesthetists of Great Britain & Ireland • Association of British Neurologists • Association of Genetic Nurses & Counsellors • Association of Surgeons of Great Britain and Ireland • British Dietetic Association • British Institute of Musculoskeletal Medicine • British Neuropathological Society • British Paediatric Neurology Association • British Sleep Society • British Society for Gene and 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 • Medicines and Healthcare products Regulatory Agency • National Association of Primary Care • National Pharmacy Association • NHS Alliance • NHS Confederation • Neurological Alliance of Scotland • Scottish Medicines Consortium • Wales Neurological Alliance • Welsh Health Specialised Services Committee <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 • British Myology Society • Cochrane Cystic Fibrosis & Genetic Disorders Group • Genomics England • MRC Centre for Neuromuscular Diseases • MRC Clinical Trials Unit

Provisional stakeholder list for the single technology appraisal of eladocagene exuparvovec for treating aromatic L-amino acid decarboxylase deficiency ID3791

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Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • British Society of Gastroenterology • British Society of Paediatric Gastroenterology, Hepatology and Nutrition • British Society of Rehabilitation Medicine • Chartered Society of Physiotherapy • Institute of Neurology • National Congenital Anomaly and Rare Disease Registration Service • Neonatal & Paediatric Pharmacists Group • Primary Care and Community Neurology Society • Primary Care Society for Gastroenterology • Royal College of General Practitioners • Royal College of Nursing • Royal College of Paediatrics & Child Health • Royal College of Pathologists • Royal College of Physicians • Royal College of Surgeons • Royal Pharmaceutical Society • Royal Society of Medicine • Society of Occupational Medicines • 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 • NHS Southend CCG • NHS Tameside and Glossop CCG • UCL/GOSH • Welsh Government 	<ul style="list-style-type: none"> • National Hospital for Neurology and Neurosurgery • National Institute for Health Research • TREAT-NMD <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, 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 stakeholder list, and which organisations we should include that have a particular focus on relevant equality issues.

Consultees

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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.