

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Highly Specialised Technologies Evaluation

Onasemnogene abeparvovec for treating pre-symptomatic spinal muscular atrophy (MAA partial review of HST 15) [ID4051]

Final stakeholder list

Consultees	Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> • Novartis Gene Therapies (onasemnogene abeparvovec) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • Ally Cadence Trust for Spinal Muscular Atrophy • Arthritis and Musculoskeletal Alliance • Beacon • Brain and Spine Foundation • Brain Charity • Contact • Disability Rights UK • Genetic Alliance UK • Leonard Cheshire Disability • Muscular Dystrophy UK • National Children’s Bureau • Neurological Alliance • South Asian Health Foundation • Specialised Healthcare Alliance • Spinal Muscular Atrophy UK (SMA UK) • The Annabelle Rose Foundation for Spinal Muscular Atrophy • Together for Short Lives • TreatSMA <p><u>Professional groups</u></p> <ul style="list-style-type: none"> • Association of Anaesthetists • Association of British Neurologists • Association of Genetic Nurses and Counsellors • Association of Paediatric Anaesthetists of Great Britain and Ireland • Association of Surgeons of Great Britain and Ireland 	<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 • National Services Division • Neurological Alliance of Scotland • NHS Alliance • NHS Confederation • Scottish Medicines Consortium • Wales Neurological Alliance • Welsh Government • Welsh Health Specialised Services Committee <p><u>Comparator companies</u></p> <ul style="list-style-type: none"> • None <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> • Cochrane Cystic Fibrosis & Genetic Disorders Group • Cochrane Neuromuscular • Genomics England • John Walton Muscular Dystrophy Research Centre • MRC Centre for Neuromuscular Diseases

Final matrix for the highly specialised technologies evaluation of onasemnogene abeparvovec for treating pre-symptomatic spinal muscular atrophy (MAA partial review of HST 15) [ID4051].

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Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • British Dietetic Association • British Institute of Musculoskeletal Medicine • British Myology Society • British Neuropathological Society • British Orthopaedic Association • British Paediatric Neurology Association • British Society for Children's Orthopaedic Surgery • British Society for Gene and Cell Therapy • British Society for Genetic Medicine • British Society for Human Genetics • British Society of Rehabilitation Medicine • Chartered Society for Physiotherapy • Institute of Neurology • Neonatal & Paediatric Pharmacists Group • Primary Care and Community Neurology Society • Royal College of Anaesthetists • 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 • SMA Reach • Treat-NMD • 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 • UK SMA Newborn Baby Screening Alliance 	<ul style="list-style-type: none"> • MRC Clinical Trials Unit • National Institute for Health Research <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 share it. 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.

PTO FOR DEFINITIONS OF CONSULTEES AND COMMENTATORS

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.