

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

**Intrathecal onasemnogene abeparvovec for treating spinal muscular atrophy
in people 2 years and over [ID6556]**

Final Stakeholder List

Provisional Consultees	Provisional Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> Novartis Pharmaceuticals UK (onasemnogene abeparvovec) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> ACE SMA Ally Cadence Trust for Spinal Muscular Atrophy Arthritis and Musculoskeletal Alliance Brain and Spine Foundation Brain Charity Gene People Genetic Alliance UK Jnetics Muscular Dystrophy UK Neurological Alliance Pathfinders Neuromuscular Alliance Rally Round Rupert South Asian Health Foundation Specialised Healthcare Alliance Spinal Muscular Atrophy UK The Annabelle Rose Foundation for Spinal Muscular Atrophy TREAT-SMA <p><u>Healthcare professional groups</u></p> <ul style="list-style-type: none"> Adult SMA Reach Association of Anaesthetists Association of British Neurologists Association of Chartered Physiotherapists in Neurology Association of Genetic Nurses and Counsellors Association of Paediatric Chartered Physiotherapists 	<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 - Northern Ireland Healthcare Improvement Scotland Medicines and Healthcare products Regulatory Agency National Association of Primary Care National Pharmacy Association Neurological Alliance of Scotland NHS Confederation NHS Wales Joint Commissioning Committee Scottish Medicines Consortium Wales Neurological Alliance Welsh Government <p><u>Comparator companies</u></p> <ul style="list-style-type: none"> Biogen (nusinersen) Novartis Pharmaceuticals UK (onasemnogene abeparvovec) [intravenous infusion] Roche (risdiplam) <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> Bone Research Society Brain Research UK Chronic Pain Policy Coalition Cochrane Musculoskeletal Group Genomics England

Final stakeholder list for the evaluation of intrathecal onasemnogene abeparvovec for treating spinal muscular atrophy in people 2 years and over [ID6556]

Issue date: November 2025

© National Institute for Health and Care Excellence 2025. All rights reserved. 1 of 3

Provisional Consultees	Provisional Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • Association of Surgeons of Great Britain and Ireland • British Dietetic Association • British Geriatrics Society • 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 Rheumatology • British Society of Physical and Rehabilitation Medicine • Chartered Society of Physiotherapy • Institute of Neurology • Institute of Neurology and Neurosurgery • National Neuroscience Advisory Group • Neonatal and 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 and Child Health • Royal College of Pathologists • Royal College of Physicians • Royal College of Surgeons • Royal Pharmaceutical Society • Royal Society of Medicine • SMA Reach UK • Society for Endocrinology • Treat-NMD • UK Clinical Pharmacy Association <p><u>Others</u></p> <ul style="list-style-type: none"> • Department of Health and Social Care 	<ul style="list-style-type: none"> • John Walton Muscular Dystrophy Research Centre • MRC Centre for Neuromuscular Diseases • MRC Clinical Trials Unit • National Hospital for Neurology and Neurosurgery • National Institute for Health Research • Orthopaedic Research UK <p><u>Associated Public Health groups</u></p> <ul style="list-style-type: none"> • Public Health Wales • UK Health Security Agency

Provisional Consultees	Provisional Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> NHS England 	

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.