

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

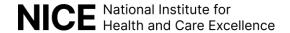
Single Technology Appraisal

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

Final Stakeholder List

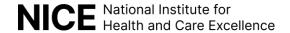
Provisional Consultees	Provisional Commentators (no right to submit or appeal)
Company	<u>General</u>
Novartis Pharmaceuticals UK	All Wales Therapeutics and Toxicology
(onasemnogene abeparvovec)	Centre
Patient/carer groups	Allied Health Professionals Federation Paged of Community Health Councils in
ACE SMA	 Board of Community Health Councils in Wales
Ally Cadence Trust for Spinal	British National Formulary
Muscular Atrophy	Care Quality Commission
Arthritis and Musculoskeletal Alliance	Department of Health - Northern Ireland
Brain and Spine Foundation	Healthcare Improvement Scotland
Brain Charity	Medicines and Healthcare products
Gene People	Regulatory Agency
Genetic Alliance UK	National Association of Primary Care
Jnetics	National Pharmacy Association
Muscular Dystrophy UK Navigala giant Allianaa	Neurological Alliance of Scotland
Neurological AlliancePathfinders Neuromuscular Alliance	NHS Confederation NHS Walsa Joint Commissioning
Rally Round Rupert	NHS Wales Joint Commissioning Committee
South Asian Health Foundation	Scottish Medicines Consortium
Specialised Healthcare Alliance	Wales Neurological Alliance
Spinal Muscular Atrophy UK	Welsh Government
The Annabelle Rose Foundation for	
Spinal Muscular Atrophy	Comparator companies
TREAT-SMA	Biogen (nusinersen)
	Novartis Pharmaceuticals UK
Healthcare professional groups	(onasemnogene abeparvovec)
Adult SMA Reach Association of Association	[intravenous infusion]
Association of AnaesthetistsAssociation of British Neurologists	Roche (risdiplam)
Association of British Neurologists Association of Chartered	Relevant research groups
Physiotherapists in Neurology	Bone Research Society
Association of Genetic Nurses and	Brain Research UK
Counsellors	Chronic Pain Policy Coalition
Association of Paediatric Chartered	Cochrane Musculoskeletal Group
Physiotherapists	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



Provisional Consultees Provisional Commentators (no right to submit or appeal) Association of Surgeons of Great John Walton Muscular Dystrophy Britain and Ireland Research Centre **British Dietetic Association** MRC Centre for Neuromuscular **British Geriatrics Society Diseases** MRC Clinical Trials Unit **British Myology Society** British Neuropathological Society National Hospital for Neurology and Neurosurgery **British Orthopaedic Association** National Institute for Health Research **British Paediatric Neurology** Orthopaedic Research UK Association British Society for Children's Associated Public Health groups Orthopaedic Surgery Public Health Wales British Society for Gene and Cell **UK Health Security Agency** 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** Others Department of Health and Social Care

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



Provisional Consultees	Provisional Commentators (no right to submit or appeal)
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.

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