

## NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

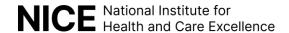
# **Single Technology Appraisal**

## Intrathecal onasemnogene abeparvovec for treating spinal muscular atrophy ID6556

## **Provisional Stakeholder List**

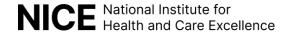
Provisional Consultees	Provisional Commentators (no right to submit or appeal)
<ul> <li>Company</li> <li>Novartis Pharmaceuticals UK (onasemnogene abeparvovec)</li> <li>Patient/carer groups</li> <li>Ally Cadence Trust for Spinal Muscular Atrophy</li> <li>Annabelle Rose Foundation for Spinal Muscular Atrophy</li> <li>Arthritis and Musculoskeletal Alliance</li> <li>Brain and Spine Foundation</li> <li>Brain Charity</li> </ul>	<ul> <li>submit or appeal)</li> <li>General</li> <li>All Wales Therapeutics and Toxicology Centre</li> <li>Allied Health Professionals Federation</li> <li>Board of Community Health Councils in Wales</li> <li>British National Formulary</li> <li>Care Quality Commission</li> <li>Department of Health - Northern Ireland</li> <li>Healthcare Improvement Scotland</li> <li>Medicines and Healthcare products Regulatory Agency</li> </ul>
<ul> <li>Gene People</li> <li>Genetic Alliance UK</li> <li>Jnetics</li> <li>Muscular Dystrophy UK</li> <li>Neurological Alliance</li> <li>South Asian Health Foundation</li> <li>Specialised Healthcare Alliance</li> <li>Spinal Muscular Atrophy UK</li> <li>Treat SMA</li> </ul>	<ul> <li>National Association of Primary Care</li> <li>National Pharmacy Association</li> <li>Neurological Alliance of Scotland</li> <li>NHS Confederation</li> <li>NHS Wales Joint Commissioning Committee</li> <li>Scottish Medicines Consortium</li> <li>Wales Neurological Alliance</li> <li>Welsh Government</li> </ul>
<ul> <li>Healthcare professional groups</li> <li>Adult SMA Reach</li> <li>Association of Anaesthetists</li> <li>Association of British Neurologists</li> <li>Association of Genetic Nurses and Counsellors</li> <li>Association of Surgeons of Great Britain and Ireland</li> <li>British Dietetic Association</li> <li>British Geriatrics Society</li> <li>British Myology Society</li> <li>British Neuropathological Society</li> <li>British Orthopaedic Association</li> </ul>	<ul> <li>Possible comparator companies</li> <li>Biogen (nusinersen)</li> <li>Novartis Pharmaceuticals UK (Onasemnogene abeparvovec [intravenous infusion])</li> <li>Roche Products (risdiplam)</li> <li>Relevant research groups</li> <li>Bone Research Society</li> <li>Brain Research UK</li> <li>Chronic Pain Policy Coalition</li> <li>Cochrane Musculoskeletal Group</li> <li>Genomics England</li> </ul>
<ul> <li>British Orthopaedic Association</li> <li>British Paediatric Neurology</li> </ul>	

Provisional stakeholder list for the evaluation of intrathecal onasemnogene abeparvovec for treating spinal muscular atrophy ID6556



#### **Provisional Consultees Provisional Commentators (no right to** submit or appeal) Association Research Centre British Society for Children's MRC Centre for Neuromuscular Orthopaedic Surgery Diseases British Society for Gene and Cell • MRC Clinical Trials Unit Therapy National Hospital for Neurology and British Society for Genetic Medicine Neurosurgery British Society for Rheumatology • National Institute for Health Research British Society of Physical and Orthopaedic Research UK Rehabilitation Medicine Chartered Society of Physiotherapy Associated Public Health groups Public Health Wales Institute of Neurology UK Health Security Agency 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 **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.