

## NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

## Single Technology Appraisal

## Risdiplam for treating spinal muscular atrophy in children and adults [ID1631]

## Final stakeholder list of consultees and commentators

Consultees	Commentators (no right to submit or appeal)
<p><u>Company</u></p> <p>Roche (risdiplam)</p> <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> <li>• Action on Pain</li> <li>• Action for Sick Children</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> <li>• Contact a Family</li> <li>• Disability Rights UK</li> <li>• Genetic Alliance UK</li> <li>• Jnetics</li> <li>• Leonard Cheshire Disability</li> <li>• Muscular Dystrophy UK</li> <li>• National Children's Bureau</li> <li>• Neurological Alliance</li> <li>• South Asian Health Foundation</li> <li>• Specialised Healthcare Alliance</li> <li>• Spinal Muscular Atrophy UK (SMA UK)</li> <li>• Together for Short Lives</li> <li>• Treat SMA</li> </ul> <p><u>Professional groups</u></p> <ul style="list-style-type: none"> <li>• Association of British Neurologists</li> <li>• Association of Genetic Nurses and Counsellors</li> <li>• Brain and Spine Foundation</li> <li>• British Dietetic Association</li> <li>• British Geriatrics Society</li> <li>• British Institute of Musculoskeletal Medicine</li> <li>• British Myology Society</li> </ul>	<p><u>General</u></p> <ul style="list-style-type: none"> <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>• Cell and Gene Therapy Catapult</li> <li>• Department of Health, Social Services and Public Safety for Northern Ireland</li> <li>• Healthcare Improvement Scotland</li> <li>• Medicines and Healthcare Products Regulatory Agency</li> <li>• National Association of Primary Care</li> <li>• National Pharmacy Association</li> <li>• Neurological Alliance of Scotland</li> <li>• NHS Alliance</li> <li>• NHS Confederation</li> <li>• Scottish Medicines Consortium</li> <li>• Wales Neurological Alliance</li> <li>• Welsh Health Specialised Services Committee</li> </ul> <p><u>Comparator companies</u></p> <p>None</p> <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> <li>• Bone Research Society</li> <li>• Brain Research UK</li> <li>• Chronic Pain Policy Coalition</li> <li>• Cochrane Cystic Fibrosis &amp; Genetic Disorders Group</li> <li>• Cochrane Musculoskeletal Group</li> <li>• Cochrane Neuromuscular</li> <li>• Genomics England</li> <li>• John Walton Muscular Dystrophy</li> </ul>

Final stakeholder list for the single technology appraisal of risdiplam for treating spinal muscular atrophy in children and adults [ID1631]. Issue date: August 2020

Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> <li>• British Neuropathological Society</li> <li>• British Orthopaedic Association</li> <li>• British Paediatric Neurology Association (BPNA)</li> <li>• British Society for Children's Orthopaedic Surgery</li> <li>• British Society for Gene and Cell Therapy</li> <li>• British Society for Genetic Medicine</li> <li>• British Society for Human Genetics</li> <li>• British Society for Paediatric and Adolescent Rheumatology</li> <li>• British Society of Rehabilitation Medicine</li> <li>• Chartered Society for Physiotherapy</li> <li>• Institute of Neurology</li> <li>• Neonatal &amp; Paediatric Pharmacists Group</li> <li>• Primary Care and Community Neurology Society</li> <li>• Royal College of General Practitioners</li> <li>• Royal College of Nursing</li> <li>• Royal College of Paediatrics &amp; Child Health</li> <li>• Royal College of Pathologists</li> <li>• Royal College of Physicians</li> <li>• Royal Pharmaceutical Society</li> <li>• Royal Society of Medicine</li> <li>• SMA Reach</li> <li>• Society for Endocrinology</li> <li>• Treat-NMD</li> <li>• UK Clinical Pharmacy Association</li> <li>• UK Genetic Testing Network</li> </ul> <p><u>Others</u></p> <ul style="list-style-type: none"> <li>• Department of Health and Social Care</li> <li>• NHS England</li> <li>• NHS Newcastle Gateshead CCG</li> <li>• NHS West Cheshire CCG</li> <li>• Welsh Government</li> </ul>	<p>Research Centre</p> <ul style="list-style-type: none"> <li>• MRC Centre for Neuromuscular Diseases</li> <li>• MRC Clinical Trials Unit</li> <li>• National Hospital for Neurology and Neurosurgery</li> <li>• National Institute for Health Research</li> <li>• Orthopaedic Research UK</li> </ul> <p><u>Associated Public Health Groups</u></p> <ul style="list-style-type: none"> <li>• Public Health England</li> <li>• Public Health Wales</li> </ul>

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.

***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 experts and has the right to appeal against the Final Appraisal Document (FAD).

All non-company consultees are invited to submit a statement<sup>1</sup>, respond to consultations, nominate clinical 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; 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 or patient experts.

---

<sup>1</sup> Non company consultees are invited to submit statements relevant to the group they are representing.