

## NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

## Single Technology Appraisal

## Vamorolone for treating inflammation associated with Duchenne muscular dystrophy ID4024

## Provisional Stakeholder List

Consultees	Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> <li>• Santhera (vamorolone)</li> </ul> <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> <li>• Action on Pain</li> <li>• Action Duchenne</li> <li>• Alex's Wish</li> <li>• Arthritis &amp; Musculoskeletal Alliance</li> <li>• Beacon</li> <li>• Contact</li> <li>• DMD Pathfinders</li> <li>• Duchenne Family Support Group</li> <li>• Duchenne Research Fund</li> <li>• Duchenne UK</li> <li>• Gene People</li> <li>• Genetic Alliance UK</li> <li>• Harrison's Fund</li> <li>• Joining Jack</li> <li>• Muscular Dystrophy UK</li> <li>• Pathfinders Neuromuscular Alliance</li> <li>• South Asian Health Foundation</li> <li>• Specialised Healthcare Alliance</li> <li>• Together for Short Lives</li> </ul> <p><u>Healthcare professional groups</u></p> <ul style="list-style-type: none"> <li>• Association of Anaesthetists of Great Britain &amp; Ireland</li> <li>• Association of British Neurologists</li> <li>• Association of Chartered Physiotherapists in Respiratory Care</li> <li>• Association of Genetic Nurses &amp; Counsellors</li> <li>• Association of Respiratory Nurse Specialists</li> <li>• Association of Surgeons of Great Britain and Ireland</li> </ul>	<p><u>General</u></p> <ul style="list-style-type: none"> <li>• All Wales Inherited Metabolic Disease Service Cardiff</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, 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>• National Services Division</li> <li>• NHS Commercial Medicines Unit</li> <li>• NHS Confederation</li> <li>• Scottish Medicines Consortium</li> <li>• Welsh Government</li> <li>• Welsh Health Specialised Services Committee</li> </ul> <p><u>Possible comparator companies</u></p> <ul style="list-style-type: none"> <li>• Accord-UK Ltd (prednisone)</li> <li>• Advanz Pharma (dexamethasone, prednisone)</li> <li>• Aspen (dexamethasone)</li> <li>• Aspire Pharma Ltd (dexamethasone)</li> <li>• Phoenix Labs (prednisone)</li> <li>• Cheplapharm (deflazacort)</li> <li>• Wockhardt UK Ltd (prednisone)</li> <li>• Zentiva (prednisone)</li> </ul>

Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> <li>• British Cardiovascular Society</li> <li>• British Dietetic Association</li> <li>• British Institute of Musculoskeletal Medicine</li> <li>• British Orthopaedic Association</li> <li>• British Paediatric Neurology Association</li> <li>• British Paediatric Respiratory Society</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 of Rehabilitation Medicine</li> <li>• British Thoracic Society</li> <li>• Chartered Society of Physiotherapy</li> <li>• Neonatal and Paediatric Pharmacists Group</li> <li>• Primary Care Respiratory Society UK</li> <li>• Royal College of General Practitioners</li> <li>• Royal College of Nursing</li> <li>• Royal College of Paediatrics and Child Health</li> <li>• Royal College of Pathologists</li> <li>• Royal College of Physicians</li> <li>• Royal College of Surgeons</li> <li>• Royal Pharmaceutical Society</li> <li>• Royal Society of Medicine</li> <li>• The National Congenital Anomaly and Rare Disease Registration Service</li> <li>• The North Star Clinical Network</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>• Alder Hey Children's Hospital NHS Foundation Trust, Liverpool</li> <li>• Department of Health and Social Care</li> <li>• Dubowitz Neuromuscular Centre (DNC)</li> <li>• MRC Centre for Neuromuscular Diseases</li> <li>• NHS England</li> </ul>	<p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> <li>• British Myology Society</li> <li>• Cochrane Cystic Fibrosis and Genetic Disorders Group</li> <li>• Cochrane Musculoskeletal Group</li> <li>• Cochrane Neuromuscular Group</li> <li>• Cochrane UK</li> <li>• MRC Clinical Trials Unit</li> <li>• National Institute for Health Research</li> <li>• TREAT-NMD</li> </ul> <p><u>Associated Public Health groups</u></p> <ul style="list-style-type: none"> <li>• Public Health Wales</li> <li>• UK Health Security Agency</li> </ul>

Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> <li>• Queen Square Centre for Neuromuscular Diseases UCL</li> <li>• Royal Manchester Children's Hospital NHS Foundation Trust, Manchester</li> <li>• Ryegate Centre, Sheffield Children's NHS Foundation Trust, Sheffield</li> <li>• The Addenbrooke's Neuromuscular Service, Cambridge</li> <li>• The John Walton Muscular Dystrophy Research Centre, Newcastle</li> <li>• The National Hospital for Neurology and Neurosurgery, London</li> <li>• The Walton Centre, Liverpool</li> <li>• Wessex Neurological Centre, Southampton General Hospital, Southampton</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 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.

### Definitions:

#### 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 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 Draft Guidance (FDG).

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 Draft Guidance (FDG).

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

### 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], 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.