

### NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

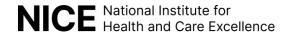
# **Single Technology Appraisal**

# Givinostat for treating Duchenne muscular dystrophy in people 6 years and over ID6323

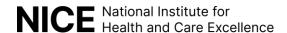
### **Provisional Stakeholder List**

Consultees	Commentators (no right to submit or
	appeal)
Company	<u>General</u>
Italfarmaco (givinostat)	<ul> <li>All Wales Inherited Metabolic Disease Service</li> </ul>
Patient/carer groups	All Wales Therapeutics and Toxicology
Action Duchenne	Centre
Alex's Wish	Allied Health Professionals Federation
Arthritis & Musculoskeletal Alliance	Board of Community Health Councils in
Beacon	Wales
Contact	British National Formulary
Duchenne Family Support Group	Care Quality Commission
Duchenne Now	Cell and Gene Therapy Catapult
Duchenne Research Fund	Department of Health, Social Services
Duchenne UK	and Public Safety for Northern Ireland
Gene People	Healthcare Improvement Scotland
Genetic Alliance UK	<ul> <li>Medicines and Healthcare products</li> </ul>
Harrison's Fund	Regulatory Agency
Joining Jack	<ul> <li>National Association of Primary Care</li> </ul>
Muscular Dystrophy UK	<ul> <li>National Pharmacy Association</li> </ul>
Pathfinders Neuromuscular Alliance	<ul> <li>National Services Division</li> </ul>
South Asian Health Foundation	NHS Confederation
Specialised Healthcare Alliance	Scottish Medicines Consortium
Together for Short Lives	Welsh Government
	Welsh Health Specialised Services
Healthcare professional groups	Committee
Association of Anaesthetists of Great	Desaible semenanten semenanias
Britain & Ireland	Possible comparator companies
Association of British Neurologists	Roche (delandistrogene moxeparvovec,     subject to NICE evaluation)
Association of Chartered	subject to NICE evaluation)  • PTC Therapeutics (ataluren)
Physiotherapists in Respiratory Care	PTC Therapeutics (ataluren)
Association of Genetic Nurses &	Relevant research groups
Counsellors	British Myology Society
Association of Respiratory Nurse     Specialists	Cochrane UK
Specialists  Association of Surgeons of Great	Cochrane Cystic Fibrosis and Genetic
Association of Surgeons of Great     Britain and Ireland	Disorders Group
British Cardiovascular Society	Cochrane Musculoskeletal Group

Provisional stakeholder list for the evaluation of givinostat for treating Duchenne muscular dystrophy in people 6 years and over ID6323



## Consultees Commentators (no right to submit or appeal) **British Dietetic Association** Genomics England MRC Clinical Trials Unit British Institute of Musculoskeletal Medicine National Institute for Health Research **British Orthopaedic Association** TREAT-NMD British Paediatric and Adolescent Bone Group Associated Public Health groups • Public Health Wales **British Paediatric Neurology** Association UK Health Security Agency British Paediatric Respiratory Society British Society for Children's Orthopaedic Surgery British Society for Gene and Cell Therapy British Society for Genetic Medicine British Society for Paediatric **Endocrinology and Diabetes** British Society of Rehabilitation Medicine **British Thoracic Society** Chartered Society of Physiotherapy Neonatal and Paediatric Pharmacists Group Primary Care Respiratory Society UK 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 The National Congenital Anomaly and Rare Disease Registration Service The North Star Clinical Network **UK Clinical Pharmacy Association Others** Alder Hey Children's Hospital NHS Foundation Trust, Liverpool Bristol Royal Hospital for Children, Bristol Department of Health and Social Care **Dubowitz Neuromuscular Centre** (DNC)



Consultees	Commentators (no right to submit or appeal)
<ul> <li>MD UK Oxford Neuromuscular Centre, Oxford</li> <li>MRC Centre for Neuromuscular Diseases</li> <li>NHS England</li> <li>Queen Square Centre for Neuromuscular Diseases UCL</li> <li>Robert Jones and Agnes Hunt Orthopaedic Hospital, Oswestry</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 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:**

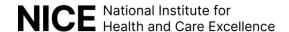
#### Consultees

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).

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#### 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.