NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Delandistrogene moxeparvovec for treating Duchenne muscular dystrophy in children aged 4 to 7 years ID3897

Provisional Stakeholder List

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

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

Consultees	Commentators (no right to submit or appeal)
• Dubowitz Neuromuscular Centre (DNC) • MRC Centre for Neuromuscular Diseases • NHS England • Queen Square Centre for Neuromuscular Diseases UCL • Royal Manchester Children's Hospital NHS Foundation Trust, Manchester • Ryegate Centre, Sheffield Children's NHS Foundation Trust, Sheffield • The Addenbrooke's Neuromuscular Service, Cambridge • The John Walton Muscular Dystrophy Research Centre, Newcastle • The National Hospital for Neurology and Neurosurgery, London • The Walton Centre, Liverpool • Wessex Neurological Centre, Southampton General Hospital, Southampton • MD UK Oxford Neuromuscular Centre, Oxford • Robert Jones and Agnes Hunt Orthopaedic Hospital, Oswestry • Bristol Royal Hospital for Children, Bristol	

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).

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.