Appendix C

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Highly Specialised Technology Evaluation

Ataluren for treating Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene (review of HST 3)

Final stakeholder of consultees and commentators

Consultees	**Commentators (no right to submit or appeal)**
Company(ies) • PTC Therapeutics (ataluren) Patient/carer groups • Action Duchenne • Action for Sick Children • Alex's Wish • Arthritis & Musculoskeletal Alliance • Black Health Agency • Children's Society • Contact • Disability Rights UK • DMD Pathfinders • Duchenne Family Support Group • Duchenne UK • Findacure • Gene People • Genetic Alliance UK • Harrison's Fund • Joining Jack • Muscular Dystrophy UK • South Asian Health Foundation • Specialised Healthcare Alliance • Together for Short Lives Professional groups • Association of Anaesthetists • Association of Genetic Nurses & Counsellors • Association of Respiratory Nurses • Association of Surgeons of Great Britain and Ireland • British Dietetic Association • British Institute of Musculoskeletal Medicine	General • All Wales Therapeutics and Toxicology Centre • Allied Health Professionals Federation • Board of Community Health Councils in Wales • British National Formulary • Care Quality Commission • 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 • NHS Alliance • NHS Commercial Medicines Unit • NHS Confederation • Scottish Medicines Consortium • Welsh Government • Welsh Health Specialised Services Committee Possible comparator companies • None Relevant research groups • British Myology Society • Cochrane Cystic Fibrosis and Genetic Disorders Group • Cochrane Musculoskeletal Group • Duchenne Research Fund • John Walton Centre for Muscular Dystrophy Research (Newcastle University)

Consultees	**Commentators (no right to submit or appeal)**
• British Orthopaedic Association • British Paediatric Neurology Association • British Paediatric Respiratory Society • British Society for Genetic Medicine • British Society of Rehabilitation Medicine • British Thoracic Society • Chartered Society for Physiotherapy • Physiotherapy Pain Association • Primary Care Respiratory 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 • UK Clinical Pharmacy Association • UK Genetic Testing Network <u>Others</u> • Department of Health • National Commissioning Group for Rare Neuromuscular Disorders • NHS England • MRC Centre for Neuromuscular Diseases • Queen Square Centre for Neuromuscular Diseases UCL • Great Ormond Street Hospital • Cardiff and Vale University Health Board • Abertawe Bro Morgannwg University Health Board	• MRC Centre for Neuromuscular Diseases • MRC Clinical Trials Unit • North Star Clinical Network • National Institute for Health Research • TREAT-NMD <u>Evidence Review Group</u> • National Institute for Health Research Health Technology Assessment Programme • Warwick Evidence <u>Associated Guideline Groups</u> • National Clinical Guideline Centre <u>Associated Public Health Groups</u> • Public Health Wales • UK Health security Agency

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.

PTO FOR DEFINITIONS OF CONSULTEES AND COMMENTATORS

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 relevant NHS organisations in England.

The company that markets the technology is invited to make an evidence submission, respond to consultations, nominate clinical specialists and has the right to appeal against recommendations.

All non-company consultees are invited to make an evidence submission or submit a statement[1], respond to consultations, nominate clinical specialists or patient experts and have the right to appeal against the recommendations.

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 final evaluation document for information only, without right of appeal. These organisations are: companies that market comparator technologies; Healthcare Improvement Scotland; the relevant National Collaborating Centre (a group commissioned by the Institute to develop clinical guidelines); other 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 NHS Commercial Medicines Unit, and the British National Formulary).

All non-company commentators are invited to nominate clinical specialists or patient experts.

Evidence Review Group (ERG)

An independent academic group commissioned by the National Institute for Health Research (NIHR) Health Technology Assessment Programme (HTA Programme) to assist the HST Evaluation Committee in reviewing the company evidence submission to the Institute.

[1]Non-company consultees are invited to submit statements relevant to the group they are representing.