NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Doxecitine–doxribtimine for treating thymidine kinase 2 deficiency in people of any age ID6484

Stakeholder List

Provisional Consultees	Provisional Commentators (no right to submit or appeal)
Company • UCB Pharma (doxecitine–doxribtimine) Patient/carer groups • Beacon • Gene People • Genetic Alliance UK • Metabolic Support UK • Muscular Dystrophy UK • South Asian Health Foundation • Specialised Healthcare Alliance • The Lily Foundation Healthcare professional groups • Association of British Neurologists • Association of Genetic Nurses & Counsellors • Association of Paediatric Chartered Physiotherapists • British Inherited Metabolic Disorder Group • British Myology Society • British Paediatric Neurology Association • British Society for Gene and Cell Therapy • British Society for Genetic Medicine • Neonatal and Paediatric Pharmacists Group • Royal College of General Practitioners • Royal College of Nursing • Royal College of Paediatrics & Child Health • Royal College of Pathologists	General • All Wales Inherited Metabolic Disease Service Cardiff • 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 - Northern Ireland • Healthcare Improvement Scotland • Medicines and Healthcare products Regulatory Agency • National Association of Primary Care • National Pharmacy Association • NHS Confederation • NHS Wales Joint Commissioning Committee • Scottish Medicines Consortium • Welsh Government Possible comparator companies • None Relevant research groups • Cochrane Cystic Fibrosis & Genetic Disorders Group • Genomics England • MRC Clinical Trials Unit • MRC Mitochondrial Biology Unit • National Institute for Health Research Associated Public Health groups • Public Health Wales

Provisional Consultees	Provisional Commentators (no right to submit or appeal)
• Royal College of Physicians • Royal Pharmaceutical Society • Royal Society of Medicine • The Chartered Society of Physiotherapy • UK Clinical Pharmacy Association • United Kingdom National Screening Committee <u>Others</u> • Department of Health and Social Care • Department of Paediatric Neurology, Manchester University NHS Foundation Trust • Metabolic Medicine Department, Great Ormond Street Hospital for Children NHS Foundation Trust • NHS England • NHS Rare Mitochondrial Disorders Service (Newcastle Mitochondrial Service, University College London Hospital NHS Highly Specialised Service for Rare Mitochondrial Disorders & Oxford University Hospitals Rare Mitochondrial Disorders Service) • The Charles Dent Metabolic Unit, University College London Hospital NHS Foundation Trust • The Mark Holland Metabolic Unit, Salford Royal Hospital, Northern Care Alliance NHS Foundation Trust • Willink Biochemical Genetics Unit, Manchester University NHS Foundation Trust	• 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 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:

Consultee or commentator stakeholders are provisional until a signed Confidentiality Agreement & Undertaking form is submitted to NICE at the evaluation stage. Participating stakeholders will be listed on the project information page for the evaluation.

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.

