

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Exagamglogene autotemcel for treating transfusion-dependent beta-thalassaemia [ID4015]

Final Stakeholder List

Consultees	Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> • Vertex (exagamglogene autotemcel) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • Anthony Nolan • Black Health Agency • Gene People • Genetic Alliance UK • Immunodeficiency UK • Sickle Cell Society • South Asian Health Foundation • Specialised Healthcare Alliance • The Essenelle Foundation • UK Thalassaemia Society <p><u>Healthcare professional groups</u></p> <ul style="list-style-type: none"> • Association of Genetic Nurses & Counsellors • British Blood Transfusion Society • British Committee for Standards in Haematology • British Society for Gene and Cell Therapy • British Society for Genetic Medicine • British Society for Haematology • British Society for Human Genetics • British Society for Blood and Bone Marrow Transplantation and Cellular Therapy • NHS Genomic Medicine Service Alliance • Neonatal Paediatric Pharmacist Group • NHS Blood & Transplant • Royal College of General Practitioners • Royal College of Nursing 	<p><u>General</u></p> <ul style="list-style-type: none"> • 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 • Hospital Information Services – Jehovah’s Witnesses • Medicines and Healthcare products Regulatory Agency • National Association of Primary Care • National Pharmacy Association • NHS Confederation • Scottish Medicines Consortium • Welsh Government • Welsh Health Specialised Services Committee <p><u>Possible comparator companies</u></p> <ul style="list-style-type: none"> • Accord Health care Limited (deferasirox) • Chiesi Ltd (deferiprone) • Dr Reddy’s Laboratories (UK) Ltd (deferasirox) • Glenmark Pharmaceuticals Europe Ltd (deferasirox) • Hospira UK Ltd (desferrioxamine Mesilate) • Lipomed GmbH (deferiprone)

Provisional stakeholder list for the evaluation of CTX001 for treating transfusion-dependent beta-thalassaemia ID4015

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Page 1 of 3

Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • Royal College of Pathologists • Royal College of Physicians • Royal College of Surgeons of England • Royal Pharmaceutical Society • Royal Society of Medicine • Sickle cell and thalassaemia nurses, midwives and allied health care professionals (STANMAP) • UK Clinical Pharmacy Association • UK Forum on Haemoglobin Disorders • UK National Haemoglobinopathy Panel • UK National Screening Committee <p><u>Others</u></p> <ul style="list-style-type: none"> • Department of Health and Social Care • NHS England 	<ul style="list-style-type: none"> • MSN Laboratories Europe Limited (deferasirox) • Mylan (deferasirox) • Novartis Pharmaceuticals UK Ltd (deferasirox, desferrioxamine Mesilate) • Sandoz Limited (deferasirox) • Teva UK (deferasirox) • Zentiva (deferasirox) <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> • Cochrane Cystic Fibrosis & Genetic Disorders Group • Cochrane UK • Genomics England • MRC Clinical Trials Unit • National Institute for Health Research <p><u>Associated Public Health groups</u></p> <ul style="list-style-type: none"> • 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 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 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 statement¹, 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], 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.

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