NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Lumacaftor with ivacaftor for treating cystic fibrosis in children aged 2 to 11 years old homozygous for the F508del mutation ID1486 Matrix of consultees and commentators

Consultees	Commentators (no right to submit or appeal)
 <u>Company</u> Vertex Pharmaceuticals (ivacaftor, lumacaftor) <u>Patient/carer group</u> Action for Sick Children British Lung Foundation Contact a Family Cystic Fibrosis Care Cystic Fibrosis Trust Genetic Alliance Genetic Disorders UK Muslim Council of Britain National Children's Bureau South Asian Health Foundation Specialised Healthcare Alliance Together for Short Lives 	 <u>General</u> 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 Confederation Scottish Medicines Consortium Welsh Health Specialised Services Committee
 Association of Respiratory Nurse Specialists British Inherited Metabolic Disease Group British Paediatric Respiratory Society British Society for Gene and Cell therapy British Society for Genetic Medicine British Society for Human Genetics British Thoracic Society Chartered Society of Physiotherapy Cystic Fibrosis Nurses Association Primary Care Respiratory Society UK Royal College of General Practitioners Royal College of Nursing 	 <u>Comparator companies</u> Accord (nebulised hypertonic sodium chloride) Essential Pharmaceuticals (pancreatin) Janssen (pancreatin) Merck (pancreatin) Mylan (pancreatin) Pari Medical (nebulised hypertonic sodium chloride) Pharmaxis (mannitol dry powder for inhalation) Roche Products (dornase alfa) Sanofi (carbocisteine) Typharm (carbocisteine)

Provisional matrix for the proposed single technology appraisal of lumacaftor with ivacaftor for treating cystic fibrosis in children aged 2 to 11 years old homozygous for the F508del mutation ID1486 Issue date: September 2018.

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Consultees	Commentators (no right to submit or appeal)
 Royal College of Paediatrics & Child Health Royal College of Pathologists Royal College of Physicians Royal Pharmaceutical Society Royal Society of Medicine UK Clinical Pharmacy Association UK Cystic Fibrosis Pharmacy Group UK Genetic Testing Network Others Department of Health and Social Care NHS Bath and North East Somerset CCG NHS England NHS Wiltshire CCG Welsh Government 	 <u>Relevant Research Groups</u> British Association for Lung Research CF Unite Cochrane Airways Group Cochrane Cystic Fibrosis and Genetic Disorders Group Genomics England MRC Clinical Trials Unit National Institute for Health Research UK Cystic Fibrosis Gene Therapy Consortium <u>Associated Public Health Groups</u> Public Health England Public Health Wales

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 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 appraisal; 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 Appraisal Document (FAD).

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 Appraisal Document (FAD).

Commentators

Organisations that engage in the appraisal process but that are not asked to prepare an evidence submission or statement, are able to respond to consultations and they receive the FAD 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.

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