

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Highly Specialised Technology

**Maralixibat for treating type 2 progressive familial intrahepatic
cholestasis ID3818**

Provisional stakeholder list

Consultees	Commentators (no right to submit or appeal)
<p><u>Company</u> Mirum Pharmaceuticals (maralixibat)</p> <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • Addenbrookes Liver Transplant Association • British Liver Trust • Children’s Liver Disease Foundation • Contact a Family • Findacure • Genetic Alliance UK • Liver4Life • Metabolic Support UK • South Asian Health Foundation • Specialised Healthcare Alliance <p><u>Professional groups</u></p> <ul style="list-style-type: none"> • Association of Anaesthetists • Association of Genetic Nurses & Counsellors • Association of Surgeons of Great Britain and Ireland • British Association for the Study of the Liver • British Blood Transfusion Society • British Committee for Standards in Haematology • British Geriatrics Society • British Society for Gene and Cell Therapy • British Society for Genetic Medicine • British Society for Haematology • British Society for Human Genetics • British Society of Gastroenterology 	<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 • Inherited Metabolic and Lysosomal Disease Service, Cardiff and Vale UHB • Medicines and Healthcare products Regulatory Agency • National Association of Primary Care • National Pharmacy Association • National Services Division • NHS Alliance • NHS Confederation • Scottish Medicines Consortium • Welsh Health Specialised Services Committee <p><u>Possible comparator companies</u> None</p> <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> • Cochrane Cystic Fibrosis & Genetic Disorders Group • Cochrane Hepato-Biliary Group • Foundation for Liver Research • Genomics England

Provisional stakeholder list for the evaluation of maralixibat for treating type 2 progressive familial intrahepatic cholestasis ID3818 Issue date: January 2021

Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • British Society of Paediatric Gastroenterology, Hepatology and Nutrition • British Transplantation Society • Neonatal and Paediatric Pharmacists Group • NHS Blood and Transplant • 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 <p><u>Others</u></p> <ul style="list-style-type: none"> • Department of Health and Social Care • NHS England • Addenbrooke's Lysosomal Disorders Unit • Birmingham Children's Hospital NHS Foundation Trust Lysosomal Storage Disorders Unit • Department of Endocrinology, University Hospital Birmingham Foundation Trust • Great Ormond Street Hospital Metabolic Unit • Royal Free Lysosomal Storage Disorders Unit • Salford Royal NHS Foundation Trust Mark Holland Metabolic Unit • Willink Unit, Genetic Medicine, Central Manchester Foundation Trust • Welsh Government 	<ul style="list-style-type: none"> • 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 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 stakeholder list, and which organisations we should include that have a particular focus on relevant equality issues.

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 specialists 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 specialists 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; 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 the British National Formulary).

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

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