

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Maralixibat for treating 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 • Beacon • British Liver Trust • Children’s Liver Disease Foundation • Contact a Family • 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 • British Society of Paediatric Gastroenterology, Hepatology and Nutrition 	<p><u>General</u></p> <ul style="list-style-type: none"> • 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, 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 • National Services Division • 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"> • Albireo Pharma (odevixibat) <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> • Cochrane Cystic Fibrosis & Genetic Disorders Group • Cochrane Hepato-Biliary Group • Cochrane UK • Foundation for Liver Research • Genomics England • MRC Clinical Trials Unit • National Institute for Health Research

Provisional stakeholder list for the evaluation of maralixibat for treating progressive familial intrahepatic cholestasis ID3818

Issue date: March 2023

© National Institute for Health and Care Excellence 2023. All rights reserved.

Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • 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 <p><u>Others</u></p> <ul style="list-style-type: none"> • Addenbrooke's Lysosomal Disorders Unit • Birmingham Children's Hospital NHS Foundation Trust Lysosomal Storage Disorders Unit • Department of Endocrinology, University Hospital Birmingham Foundation Trust • Department of Health and Social Care • Great Ormond Street Hospital Metabolic Unit • NHS England • Royal Free Lysosomal Storage Disorders Unit • Salford Royal NHS Foundation Trust Mark Holland Metabolic Unit • Willink Unit, Genetic Medicine, Central Manchester Foundation Trust 	<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

Provisional stakeholder list for the evaluation of maralixibat for treating progressive familial intrahepatic cholestasis ID3818

Issue date: March 2023

© National Institute for Health and Care Excellence 2023. All rights reserved.

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.