

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Olezarsen for treating familial chylomicronaemia syndrome ID6585

Final Stakeholder List

Provisional Consultees	Provisional Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> • Sobi (olezarsen) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • Action FCS • Beacon • Gene People • Genetic Alliance UK • Metabolic Support UK • South Asian Health Foundation • Specialised Healthcare Alliance <p><u>Healthcare professional groups</u></p> <ul style="list-style-type: none"> • Association of Genetic Nurses and Counsellors • British Dietetic Association • British Geriatrics Society • British Inherited Metabolic Disease Group • British Society for Gene and Cell Therapy • British Society for Genetic Medicine • HEART UK • National Metabolic Biochemistry Network • Royal College of General Practitioners • Royal College of Nursing • Royal College of Pathologists • Royal College of Physicians • Royal Pharmaceutical Society • Royal Society of Medicine • Society for Endocrinology • UK Clinical Pharmacy Association <p><u>Others</u></p> <ul style="list-style-type: none"> • All Wales Inherited Metabolic Disease Service 	<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 - Northern Ireland • Healthcare Improvement Scotland • Medicines and Healthcare products Regulatory Agency • National Association of Primary Care • National Pharmacy Association • National Services Division • NHS Confederation • NHS Wales Joint Commissioning Committee • Scottish Medicines Consortium • Welsh Government <p><u>Comparator companies</u></p> <ul style="list-style-type: none"> • Akcea Therapeutics (volanesorsen) • Arrowhead Pharmaceuticals (plozasiran) <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> • Cochrane Cystic Fibrosis and Genetic Disorders Group • Cochrane Metabolic and Endocrine Disorders Group • Genomics England • MRC Clinical Trials Unit • National Institute for Health Research • Society for The Study of Inborn Errors of Metabolism

Final stakeholder list for the evaluation of olezarsen for treating familial chylomicronaemia syndrome ID6585

Issue date: November 2025

© National Institute for Health and Care Excellence 2025. All rights reserved. 1 of 3

Provisional Consultees	Provisional Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • Central Manchester Foundation Trust, Willink Unit, Genetic Medicine • Department of Health and Social Care • Great Ormond Street Hospital Metabolic Unit • Guys and St Thomas' NHS Trust, Adult Inherited Metabolic Diseases • Leeds Teaching Hospitals NHS Trust, St James' Hospital, Diabetes • NHS England • NHS England National Programmes of Care, Internal Medicine: Hepatobiliary and Pancreas Clinical Reference Group (CRG) • NHS Genomic Medicine Service • Salford Royal NHS Foundation Trust Mark Holland Metabolic Unit • Specialised Endocrinology CRG • University Hospital Birmingham Foundation Trust, Department of Endocrinology • University Hospitals of Bristol NHS Foundation Trust, Clinical Biochemistry 	<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:

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.