

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Elamipretide for treating Barth syndrome in people of any age ID6545

Provisional Stakeholder List

Provisional Consultees	Provisional Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> • Pharmanovia (Elamipretide) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • Arrhythmia Alliance • Atrial Fibrillation Association • Barth Syndrome UK • Beacon for Rare Diseases • Cardiac Risk in the Young • Cardiomyopathy UK • Cardiovascular Care Partnership • Children’s Heart Federation • Circulation Foundation • Gene People • Genetic Alliance UK • HEART UK • Metabolic Support UK • Pumping Marvellous • Somerville Foundation • 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 Association for Nursing in Cardiovascular Care • British Cardiovascular Intervention Society • British Cardiovascular Society • British Heart Foundation • British Nuclear Cardiology Society • British Paediatric and Adolescent Bone Group • British Society for Children's Orthopaedic Surgery 	<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 • NHS Confederation • NHS Wales Joint Commissioning Committee • Scottish Medicines Consortium • Welsh Government <p><u>Possible comparator companies</u></p> <p>None</p> <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> • British Society for Cardiovascular Research • Cochrane Heart, Stroke and Circulation • European Council for Cardiovascular Research • Genomics England • Heart Research UK • MRC Clinical Trials Unit • National Centre for Cardiovascular Preventions and Outcomes • National Institute for Health Research • Wellcome Trust

Provisional stakeholder list for the evaluation of elamipretide for treating Barth syndrome in people of any age ID6545

Issue date: March 2026

Provisional Consultees	Provisional Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • British Society for Gene and Cell Therapy • British Society for Genetic Medicine • British Society for Heart Failure • British Society of Cardiovascular Imaging • National Heart and Lung Institute • Primary Care Cardiovascular Society • 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 Cardiological Science and Technology • Society for Vascular Technology of Great Britain and Ireland • UK Clinical Pharmacy Association • Vascular Society of Great Britain and Ireland <p><u>Others</u></p> <ul style="list-style-type: none"> • University Hospitals Bristol and Weston NHS Foundation Trust • Cardiac and Cardiology Research Dept, Barts • Department of Health and Social Care • NHS England 	<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.