

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Sapropterin for treating phenylketonuria [ID1475]

Stakeholder list of consultees and commentators

Consultees	Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> • BioMarin (sapropterin) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • Genetic Alliance UK • Metabolic Support UK • Muslim Council of Britain • PHED UP • South Asian Health Foundation • Specialised Healthcare Alliance • The National Society for Phenylketonuria <p><u>Professional groups</u></p> <ul style="list-style-type: none"> • Association of Genetic Nurses & Counsellors • British Dietetic Association • British Geriatrics Society • British Inherited Metabolic Disease Group • British Psychoanalytic Council • British Society for Genetic Medicine • British Society for Human Genetics • 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 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 • NHS Hastings & Rother CCG • NHS West Hampshire CCG • Welsh Government 	<p><u>General</u></p> <ul style="list-style-type: none"> • Allied Health Professionals • All Wales Therapeutics and Toxicology Centre • 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 <p><u>Possible comparator companies</u></p> <p>None</p> <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> • Cochrane Cystic Fibrosis & Genetic Disorders Group • Cochrane Metabolic & Endocrine Disorders Group • 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 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

Stakeholder list for the technology appraisal of sapropterin for treating phenylketonuria ID1475. Issue date: May 2020

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 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.