

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Leniolisib for untreated activated phosphoinositide 3-kinase delta syndrome in people 12 years and over ID6130

Provisional Stakeholder List

Consultees	Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> • Pharming Group N.V (leniolisib) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • Anthony Nolan • Beacon • Contact • Gene People • Genetic Alliance UK • Immunodeficiency UK • South Asian Health Foundation • Specialised Healthcare Alliance • UKPIPS <p><u>Healthcare professional groups</u></p> <ul style="list-style-type: none"> • Association of Genetic Nurses & Counsellors • British Geriatrics Society • British Society for Gene and Cell Therapy • British Society for Genetic Medicine • British Society for Immunology • British Society of Blood and Marrow Transplantation and Cellular Therapy • Neonatal and Paediatric Pharmacists Group • NHS Blood and Transplant • Royal College of General Practitioners • Royal College of Nursing • Royal College of Paediatrics & Child Health • Royal College of Pathologists • Royal College of Physicians • Royal Society of Medicine • United Kingdom Primary 	<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 • 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"> • None <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> • Cochrane Cystic Fibrosis & Genetic Disorders Group • Cochrane UK • Genomics England • MRC Clinical Trials Unit • National Institute for Health Research

Provisional stakeholder list for the evaluation of leniolisib for untreated activated phosphoinositide 3-kinase delta syndrome in people 12 years and over ID6130
Issue date: November 2022

Consultees	Commentators (no right to submit or appeal)
<p>Immunodeficiency Network</p> <ul style="list-style-type: none"> • UK Clinical Pharmacy Association <p><u>Others</u></p> <ul style="list-style-type: none"> • Cambridge University Hospitals, NHS Foundation Trust • Department of Health and Social Care • NHS England • Royal Papworth Hospital, NHS 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

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

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

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.