

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Pegzilarginase for treating arginase-1 deficiency ID4029

Stakeholder List

Consultees	Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> • Immedica Pharma (pegzilarginase) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • Beacon • Brain and Spine Foundation • Brain Charity • British Liver Trust • Children's Liver Disease Foundation • Contact • Gene People • Genetic Alliance UK • GUTS UK • Liver4Life • Metabolic Support UK • Neurological Alliance • South Asian Health Foundation • Specialised Healthcare Alliance <p><u>Healthcare professional groups</u></p> <ul style="list-style-type: none"> • Association of British Neurologists • Association of Genetic Nurses and Counsellors • British Association for the Study of the Liver • British Association of Endocrine and Thyroid Surgeons • British Geriatrics Society • British Inherited Metabolic Disease Group • British Neuropathological Society • British Paediatric Neurology Association • British Society for Gene and Cell Therapy • British Society for Genetic Medicine • Institute of Neurology 	<p><u>General</u></p> <ul style="list-style-type: none"> • All Wales Inherited Metabolic Disease Service • 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 • Inherited Metabolic and Lysosomal Disease Services, Cardiff and Vale UHB • 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"> • Eurocept International (sodium phenylbutyrate) • Immedica (sodium phenylbutyrate) <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> • Brain Research UK • Cochrane Cystic Fibrosis and Genetic Disorders Group • Cochrane Hepato-Biliary Group

Stakeholder list for the evaluation of pegzilarginase for treating arginase-1 deficiency ID4029

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Page 1 of 3

Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • National Metabolic Biochemistry Network • National Neuroscience Advisory Group • Neonatal and Paediatric Pharmacists Group • Primary Care and Community Neurology Society • 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 Pharmaceutical Society • Royal Society of Medicine • Society for Endocrinology • UK Clinical Pharmacy Association <p><u>Others</u></p> <ul style="list-style-type: none"> • Birmingham Children’s Hospital NHS Foundation Trust, Inherited Metabolic Disorders • Department of Health and Social Care • Great Ormond Street Hospital for Children, Metabolic Medicine Department • NHS England • Queen Elizabeth Hospital, Birmingham, Adult Metabolic Unit • Royal Manchester Children’s Hospital, Metabolic Medicine Unit • Salford Royal Hospital, Mark Holland Metabolic Unit • Sheffield Children’s Hospital, Metabolic Medicine Unit • University College London Hospitals, Charles Dent Metabolic Unit 	<ul style="list-style-type: none"> • Cochrane Metabolic and Endocrine Disorders Group • Cochrane UK • Foundation for Liver Research • Genomics England • MRC Clinical Trials Unit • National Hospital for Neurology and Neurosurgery • National Institute for Health Research <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 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.