

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

Ganaxolone for treating seizures caused by CDKL5 deficiency disorder in people 2 years and over ID3988

Final Stakeholder List

Consultees	Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> • Orion Pharma (ganaxolone) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • Brain and Spine Foundation • Brain Charity • CDKL5 UK • Epilepsy Action • Epilepsy Society • Findacure • Gene People • Genetic Alliance UK • Neurological Alliance • South Asian Health Foundation • Specialised Healthcare Alliance • Young Epilepsy <p><u>Professional groups</u></p> <ul style="list-style-type: none"> • Association of British Neurologists • Association of Genetic Nurses and Counsellors • British Association of Neurologists • British Geriatrics Society • British Neuropathological Society • British Paediatric Epilepsy Group • British Paediatric Neurology Association • British Society for Cell and Gene Therapy • British Society for Genetic Medicine • British Society for Human Genetics • Epilepsy Nurses Association • Epilepsy Society • Institute of Neurology • International League Against Epilepsy 	<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 • Inherited Metabolic and Lysosomal Disease Service, Cardiff and Vale UHB • Medicines and Healthcare Products Regulatory Agency • National Association of Primary Care • National Pharmacy Association • National Services Division • Neurological Alliance of Scotland • NHS Alliance • NHS Confederation • Scottish Medicines Consortium • Wales Neurological Alliance • 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"> • Brain Research UK • British Neurological Research Trust • Cochrane Cystic Fibrosis & Genetic Disorders Group

Stakeholder list for the health technology appraisal of ganaxolone for treating seizures caused by CDKL5 deficiency disorder in people 2 years and over ID3988

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Consultees	Commentators (no right to submit or appeal)
<p>UK</p> <ul style="list-style-type: none"> • Neonatal and Paediatric Pharmacists Group • Neuromodulation Society of UK and Ireland • Primary Care and Community Neurology Society • Primary Care Neurology Society • Royal College of General Practitioners • Royal College of Nursing • Royal College of Paediatrics & Child Health • Royal College of Pathologists • Royal College of Physicians • Royal Pharmaceutical Society • Royal Society of Medicine • UK Clinical Pharmacy Association • UK Genetic Testing Network <p><u>Others</u></p> <ul style="list-style-type: none"> • Central Manchester Foundation Trust, Willink Unit, Genetic Medicine • Department of Health and Social Care • Manchester centre for genomic medicine • NHS England • Rare Disease Collaborative Network (RDCN) for CDKL5 Bristol Royal Hospital for Children • Welsh Government 	<ul style="list-style-type: none"> • Cochrane Epilepsy Group • Cochrane UK • Epilepsy Research UK • 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 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 experts 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 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; 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.