

# NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

## Proposed Highly Specialised Technology Evaluation

### Cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2 ID943

#### Provisional matrix of consultees and commentators

Consultees	Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> <li>• BioMarin (cerliponase alfa)</li> </ul> <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> <li>• Action for Sick Children</li> <li>• Batten Disease Family Association</li> <li>• Brain and Spine Foundation</li> <li>• Children Living with Inherited Metabolic Diseases</li> <li>• Contact a Family</li> <li>• Council for disabled children</li> <li>• Disability Rights UK</li> <li>• Genetic Alliance UK</li> <li>• Genetic Disorders UK</li> <li>• Leonard Cheshire Disability</li> <li>• LSD Patient Collaborative</li> <li>• Muslim Council of Britain</li> <li>• National Children's Bureau</li> <li>• Neurological Alliance</li> <li>• Rare Disease UK</li> <li>• Sense</li> <li>• South Asian Health Foundation</li> <li>• Specialised Healthcare Alliance</li> <li>• Sue Ryder</li> <li>• Together for Short Lives</li> <li>• The Brain Charity</li> </ul> <p><u>Professional groups</u></p> <ul style="list-style-type: none"> <li>• Association of British Neurologists</li> <li>• Association of Genetic Nurses and Counsellors</li> <li>• British Inherited Metabolic Disease Group</li> <li>• British Neuropathological Society</li> <li>• British Society for Genetic Medicine</li> <li>• Evelina London Children's Hospital</li> <li>• Institute of Neurology</li> <li>• National Metabolic Biochemistry Network</li> <li>• Neuromodulation Society of UK and</li> </ul>	<p><u>General</u></p> <ul style="list-style-type: none"> <li>• Allied Health Professionals Federation</li> <li>• Board of Community Health Councils in Wales</li> <li>• British National Formulary</li> <li>• Care Quality Commission</li> <li>• Department of Health, Social Services and Public Safety for Northern Ireland</li> <li>• Healthcare Improvement Scotland</li> <li>• Inherited Metabolic and Lysosomal Disease Service (at the Cardiff and Vale UHB).</li> <li>• Medicines and Healthcare products Regulatory Agency</li> <li>• National Association of Primary Care</li> <li>• National Pharmacy Association</li> <li>• National Services Division Scotland</li> <li>• Neurological Alliance of Scotland</li> <li>• NHS Alliance</li> <li>• NHS Commercial Medicines Unit</li> <li>• NHS Confederation</li> <li>• Scottish Medicines Consortium</li> <li>• Wales Neurological Alliance</li> <li>• Welsh Government</li> <li>• Welsh Health Specialised Services Committee</li> </ul> <p><u>Possible comparator manufacturers</u></p> <ul style="list-style-type: none"> <li>• None</li> </ul> <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> <li>• Brain Research Trust</li> <li>• British Neurological Research Trust</li> <li>• Cochrane Cystic Fibrosis and Genetic Disorders Group</li> <li>• Cochrane Multiple Sclerosis and Rare Diseases of the Central Nervous System Group</li> <li>• MRC Clinical Trials Unit</li> <li>• National Institute for Health Research</li> <li>• Society for the Study of Inborn Errors of</li> </ul>

<b>Consultees</b>	<b>Commentators (no right to submit or appeal)</b>
<p>Ireland</p> <ul style="list-style-type: none"> <li>• Primary Care Neurology Society</li> <li>• Royal College of General Practitioners</li> <li>• Royal College of Nursing</li> <li>• Royal College of Paediatrics and Child Health</li> <li>• Royal College of Pathologists</li> <li>• Royal College of Physicians</li> <li>• Royal Pharmaceutical Society</li> <li>• Royal Society of Medicine</li> <li>• UK Clinical Pharmacy</li> <li>• UK Genetic Testing Network</li> </ul> <p><u>Others</u></p> <ul style="list-style-type: none"> <li>• Department of Health</li> <li>• NHS England</li> <li>• Addenbrooke's Lysosomal Disorders Unit</li> <li>• Birmingham Children's Hospital NHS Foundation Trust Lysosomal Storage Disorders Unit</li> <li>• Department of Endocrinology, University Hospital Birmingham Foundation Trust</li> <li>• Great Ormond Street Hospital Metabolic Unit</li> <li>• National Hospital for Neurology and Neurosurgery Charles Dent Metabolic Unit</li> <li>• Royal Free Lysosomal Storage Disorders Unit</li> <li>• Salford Royal NHS Foundation Trust Mark Holland Metabolic Unit</li> <li>• Willink Unit, Genetic Medicine, Central Manchester Foundation Trust</li> </ul>	<p>Metabolism</p> <p><u>Associated Public Health Groups</u></p> <ul style="list-style-type: none"> <li>• Public Health England</li> <li>• Public Health Wales</li> </ul>

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***

## **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 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 Determination (FAD).

All non company consultees are invited to submit a statement<sup>1</sup>, respond to consultations, nominate clinical or patient experts and have the right to appeal against the Final Appraisal Determination (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 NHS Commercial Medicines Unit, and the British National Formulary).

All non company commentators are invited to nominate clinical or patient experts.

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<sup>1</sup> Non company consultees are invited to submit statements relevant to the group they are representing.