

## NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

### Highly Specialised Technology Evaluation

#### Givosiran for treating acute hepatic porphyria [ID1549]

#### Final 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"> <li>• Alnylam (givosiran)</li> </ul> <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> <li>• Addenbrookes Liver Transplant Association</li> <li>• British Liver Trust</li> <li>• British Porphyria Association</li> <li>• Findacure</li> <li>• Genetic Alliance</li> <li>• Global Porphyria Advocacy Coalition</li> <li>• International Porphyria Patient Network IPPN</li> <li>• Liver4Life</li> <li>• Metabolic Support UK</li> <li>• South Asian Health Foundation</li> <li>• Specialised Healthcare Alliance</li> <li>• The Brain Charity</li> <li>• The Neurological Alliance</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 and Irish Porphyria Network</li> <li>• British Association for the Study of the Liver</li> <li>• British Inherited Metabolic Diseases Group</li> <li>• British Liver Nurses Forum</li> <li>• British Neuropathological Society</li> <li>• British Society for Gene and Cell Therapy</li> <li>• British Society for Genetic Medicine</li> <li>• British Society for Human Genetics</li> <li>• British Society of Gastroenterology</li> <li>• British Transplantation Society</li> </ul>	<p><u>General</u></p> <ul style="list-style-type: none"> <li>• All Wales Therapeutics and Toxicology Centre</li> <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>• Hospital Information Services – Jehovah’s Witnesses</li> <li>• Inherited Metabolic and Lysosomal Disease Service, 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</li> <li>• Neurological Alliance of Scotland</li> <li>• NHS Alliance</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>Comparator companies</u></p> <ul style="list-style-type: none"> <li>• Recordati Rare Diseases (haem arginate)</li> </ul> <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> <li>• Brain Research UK</li> <li>• Cochrane Cystic Fibrosis and Genetic Disorders Group</li> </ul>

Final stakeholder list for the highly specialised evaluation of givosiran for treating acute hepatic porphyria [ID1549]

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<ul style="list-style-type: none"> <li>• Institute of Neurology</li> <li>• NHS Blood and Transplant</li> <li>• Primary Care &amp; Community Neurology Society</li> <li>• Primary Care Society for Gastroenterology</li> <li>• Royal College of General Practitioners</li> <li>• Royal College of Nursing</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 Association</li> <li>• UK Genetic Testing Network</li> </ul> <p><u>Others</u></p> <ul style="list-style-type: none"> <li>• Department of Health and Social Care</li> <li>• National Acute Porphyria Service, Cardiff and Vale UHB</li> <li>• National Acute Porphyria Service, King's College Hospital NHS Foundation Trust</li> <li>• Mark Holland Metabolic Unit, Salford Royal NHS Foundation Trust</li> <li>• NHS England</li> </ul>	<ul style="list-style-type: none"> <li>• Cochrane Hepato-Biliary Group</li> <li>• European Porphyria Network</li> <li>• Foundation for Liver Research</li> <li>• Genomics England</li> <li>• MRC Clinical Trials Unit</li> <li>• National Hospital for Neurology and Neurosurgery</li> <li>• National Institute for Health Research</li> </ul> <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>
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NICE is committed to promoting equality, eliminating unlawful discrimination and fostering good relations between people who share a protected characteristic and those who do share it. Please let us know if we have missed any important organisations from the lists in 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 manufacturer(s) or sponsor(s) of the technology; national professional organisations; national patient organisations; the Department of Health and Social Care and relevant NHS organisations in England.

The company that markets the technology is invited to prepare a submission dossier, can 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 prepare a submission dossier respond to consultations on the draft scope, the Assessment Report and the Appraisal Consultation Document. They can nominate clinical or patient experts and have the right to appeal against the Final Appraisal Document (FAD).

### 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 final evaluation documentation for information only, without right of appeal. These organisations are: manufacturers of comparator technologies; Healthcare Improvement Scotland; the relevant National Collaborating Centre (a group commissioned by the Institute to develop clinical guidelines); other related research groups where appropriate (for example, the Medical Research Council [MRC], other groups (for example, the NHS Confederation, NHS Alliance, and the British National Formulary).

All non-company organisations can nominate clinical or patient experts to present their personal views to the Appraisal Committee.