

# NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

## Proposed Highly Specialised Technology Evaluation

### Elosulfase alfa for treating mucopolysaccharidosis type IVa (review of HST2)

#### 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 Pharmaceuticals (elosulfase alfa)</li> </ul> <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> <li>• Carol Ann Foundation/The International Morquio Organisation</li> <li>• Contact a Family</li> <li>• Findacure</li> <li>• Genetic Alliance UK</li> <li>• Jnetics</li> <li>• Metabolic Support UK</li> <li>• MPS Society</li> <li>• Muslim Council of Britain</li> <li>• South Asian Health Foundation</li> <li>• Specialised Healthcare Alliance</li> </ul> <p><u>Professional groups</u></p> <ul style="list-style-type: none"> <li>• Association of Genetic Nurses and Counsellors</li> <li>• Association of Surgeons of Great Britain and Ireland</li> <li>• British Inherited Metabolic Disease Group</li> <li>• British Society for Genetic Medicine</li> <li>• National Metabolic Biochemistry Network</li> <li>• Royal College of Anaesthetists</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 College of Surgeons</li> <li>• Royal Pharmaceutical 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>• Medicines and Healthcare products Regulatory Agency</li> <li>• National Association of Primary Care</li> <li>• National Pharmacy Association</li> <li>• National Services Division (specialised services in Scotland)</li> <li>• NHS Alliance</li> <li>• NHS Confederation</li> <li>• Scottish Medicines Consortium</li> <li>• Welsh Government</li> <li>• Welsh Health Specialised Services Committee</li> <li>• Welsh Government</li> </ul> <p><u>Possible comparator companies</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>• Cochrane Cystic Fibrosis and Genetic Disorders Group</li> <li>• Genomics England</li> <li>• MRC Clinical Trials Unit</li> <li>• National Institute for Health Research</li> <li>• Society for the Study of Inborn Errors of Metabolism</li> </ul>

Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> <li>• Royal Society of Medicine</li> <li>• UK Genetic Testing Network</li> <li>• United Kingdom Clinical Pharmacy Association</li> </ul> <p><u>Others</u></p> <ul style="list-style-type: none"> <li>• Addenbrooke's Lysosomal Disorders Unit</li> <li>• Birmingham Children's Hospital NHS Foundation Trust</li> <li>• Charles Dent Metabolic Unit, UCLH</li> <li>• Department of Endocrinology, Queen Elizabeth Hospital, UHBFT</li> <li>• Department of Health and Social Care</li> <li>• Great Ormond Street Hospital</li> <li>• Inherited Metabolic and Lysosomal Disease Service, Cardiff and Vale UHB</li> <li>• Manchester Foundation NHS Trust</li> <li>• Mark Holland Metabolic Unit for Adult Inherited Metabolic Disorders, SRFT</li> <li>• Metabolic Medicine Unit, GOSH</li> <li>• NHS England</li> <li>• Royal Free Lysosomal Storage Disorders Unit</li> <li>• Salford Royal NHS Foundation Trust</li> <li>• University College London Hospital NHS Foundation Trust</li> <li>• University Hospital Birmingham NHS Foundation Trust</li> <li>• Willink Unit, Royal Manchester Children's Hospital, CMFT</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>

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 specialists 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 specialists 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; other 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 specialists 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.