

## NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

## Proposed Highly Specialised Technologies Evaluation

## OTL-101 for treating adenosine deaminase deficiency–severe combined immunodeficiency ID1152

## 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>• Orchard Therapeutics (OTL-101)</li> </ul> <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> <li>• Action for Sick Children</li> <li>• Anthony Nolan</li> <li>• Findacure</li> <li>• Friends, Families and Travellers</li> <li>• Genetic Alliance UK</li> <li>• Genetic Disorders UK</li> <li>• Metabolic Support UK</li> <li>• Midaye</li> <li>• Muslim Council of Britain</li> <li>• National Children's Bureau</li> <li>• Ocean Somali Community Association</li> <li>• Paveepoint Traveller and Roma Centre</li> <li>• Primary Immunodeficiency UK</li> <li>• Purine Metabolic Patients' Association</li> <li>• South Asian Health Foundation</li> <li>• Specialised Healthcare Alliance</li> <li>• The National Federation of Gypsy Liaison Groups</li> <li>• The Travellers Movement</li> <li>• Together for Short Lives</li> <li>• UK Primary Immune-deficiency Patient Support</li> <li>• UK Primary Immunodeficiency Network</li> </ul> <p><u>Professional groups</u></p> <ul style="list-style-type: none"> <li>• Association of Genetic Nurses &amp; Counsellors</li> <li>• Association of Paediatric Emergency Medicine</li> <li>• British Association of Neurologists</li> <li>• British Inherited Metabolic Disease Group</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 Transplantation Society</li> <li>• Genomics England</li> <li>• Royal College of General Practitioners</li> <li>• Royal College of Nursing</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>• British Society of Blood and Marrow Transplantation</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>• 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 Blood and Transplant</li> <li>• NHS Confederation</li> <li>• Scottish Medicines Consortium</li> <li>• United Kingdom National Screening Committee</li> <li>• Welsh Blood Service</li> <li>• Welsh Government</li> <li>• Welsh Health Specialised Services Committee</li> </ul> <p><u>Possible comparator companies</u></p> <ul style="list-style-type: none"> <li>• Orchard Therapeutics (Strimvelis)</li> </ul> <p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> <li>• Cell and Gene Therapy Catapult</li> <li>• Cochrane Cystic Fibrosis and Genetic Disorders Group</li> <li>• Gene and Cell Therapy Group - UCL</li> <li>• Medical Research Council - Human Genetics Unit</li> <li>• MRC Clinical Trials Unit</li> <li>• National Institute for Health Research</li> </ul>

Consultees	Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> <li>• Royal College of Paediatrics &amp; 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>• Society for the Study of Inborn Errors of Metabolism</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>• Great Ormond Street Hospital – Immunology Department</li> <li>• Newcastle upon Tyne Hospitals NHS Foundation Trust</li> <li>• NHS England</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 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.