# NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

## Highly Specialised Technologies Evaluation (HST)

### Strimvelis for treating severe combined immunodeficiency caused by adenosine deaminase deficiency [ID926]

Consultees	Commentators (no right to submit or
	appeal)
Company	General
GlaxoSmithKline (Strimvelis)	<ul> <li>Allied Health Professionals Federation</li> <li>Board of Community Health Councils in</li> </ul>
Patient/carer groups	Wales
<ul><li>Action for Sick Children</li><li>Anthony Nolan</li></ul>	British Society of Blood and Marrow Transplantation
Climb (Children Living with Inherited	British National Formulary
Metabolic Diseases)	Care Quality Commission
• Friends, Families and Travellers	• Department of Health, Social Services
Genetic Alliance UK	and Public Safety for Northern Ireland
Genetic Disorders UK	Healthcare Improvement Scotland
<ul><li>Jnetics</li><li>Midaye</li></ul>	Medicines and Healthcare Products     Regulatory Agency
Muslim Council of Britain	National Association of Primary Care
National Children's Bureau	National Pharmacy Association
Ocean Somali Community Association	-
<ul> <li>Paveepoint Traveller and Roma</li> </ul>	NHS Blood and Transplant
Centre	NHS Commercial Medicines Unit
Primary Immunodeficiency UK	NHS Confederation
<ul> <li>Purine Metabolic Patients' Association</li> </ul>	
<ul> <li>South Asian Health Foundation</li> </ul>	United Kingdom National Screening
Specialised Healthcare Alliance	Committee
The National Federation of Gypsy	Welsh Blood Service
Liaison Groups	Welsh Health Specialised Services
The Travellers Movement	Committee
Together for Short Lives	
UK Primary Immunodeficiency	Comparator companies
Network	Orchard Therapeutics (ex-vivo
UK Primary Immune-deficiency	autologous gene therapy)
Patient Support	Sigma-Tau Pharmaceuticals (pegylated)
	adenosine deaminase enzyme)
Professional groups	
Association of Genetic Nurses &	Relevant research groups
Counsellors	Cell and Gene Therapy Catapult
Association of Paediatric Emergency	Cochrane Cystic Fibrosis and Genetic
Medicine	Disorders Group
British Inherited Metabolic Disease	Gene and Cell Therapy Group - UCL

### Matrix of consultees and commentators

National Institute for Health and Care Excellence

Matrix for the highly specialised technologies evaluation of Strimvelis for treating severe combined immunodeficiency caused by adenosine deaminase deficiency [ID926] Issue date: May 2017

Consultees	Commentators (no right to submit or appeal)
<ul> <li>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 Paediatrics &amp; Child Health</li> <li>Royal College of Pathologists</li> <li>Royal College of Physicians</li> <li>Royal College of Physicians</li> <li>Royal College of Physicians</li> <li>Royal College 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> Others <ul> <li>Department of Health</li> <li>Great Ormond Street Hospital – Immunology Department</li> <li>Newcastle upon Tyne Hospitals NHS Foundation Trust</li> <li>NHS England</li> <li>Welsh Government</li> </ul>	<ul> <li>Medical Research Council - Human Genetics Unit</li> <li>MRC Clinical Trials Unit</li> <li>National Institute for Health Research</li> </ul> <u>Associated Public Health Groups</u> <ul> <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 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 Evaluation Determination (FED).

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 Evaluation Determination (FED).

#### 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 FED for information only, without right of appeal. These organisations are: companies that market 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], 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

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

National Institute for Health and Care Excellence

Matrix for the highly specialised technologies evaluation of Strimvelis for treating severe combined immunodeficiency caused by adenosine deaminase deficiency [ID926] Issue date: May 2017