

NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Single Technology Appraisal

**Apadamtase alfa for treating congenital thrombotic thrombocytopenic
purpura caused by ADAMTS-13 deficiency ID6192**

Provisional Stakeholder List

Provisional Consultees	Provisional Commentators (no right to submit or appeal)
<p><u>Company</u></p> <ul style="list-style-type: none"> • Takeda UK Ltd (apadamtase alfa) <p><u>Patient/carer groups</u></p> <ul style="list-style-type: none"> • African Health Policy Network • AOFAC Foundation • Beacon • Black Health Agency for Equality • British Liver Trust • Children’s Liver Disease Foundation • Contact • Gene People • Genetic Alliance UK • Haemophilia Society • ITP Support Association • Liver4Life • South Asian Health Foundation • Specialised Healthcare Alliance • Thrombosis UK • TTP Network <p><u>Healthcare professional groups</u></p> <ul style="list-style-type: none"> • Anaemia Nurse Specialist Association • Association of Genetic Nurses and Counsellors • British Association for the Study of the Liver • British Blood Transfusion Society • British Geriatrics Society • British Psychological Society • British Society for Gene and Cell Therapy • British Society for Genetic Medicine • British Society for Haematology 	<p><u>General</u></p> <ul style="list-style-type: none"> • All Wales Therapeutics and Toxicology Centre • Allied Health Professionals Federation • Board of Community Health Councils in Wales • British National Formulary • Care Quality Commission • Cell and Gene Therapy Catapult • Department of Health, Social Services and Public Safety for Northern Ireland • Healthcare Improvement Scotland • Hospital Information Services - Jehovah’s Witnesses • Medicines and Healthcare products Regulatory Agency • National Association of Primary Care • National Pharmacy Association • National Services Division • NHS Confederation • NHS Wales Joint Commissioning Committee • Scottish Medicines Consortium • Welsh Government <p><u>Possible comparator companies</u></p> <ul style="list-style-type: none"> • Bio Products Laboratory Limited (dried factor VIII fraction type 8Y, factor VIII) CSL Behring UK Limited (factor VIII) • Grifols (factor VIII) • Novo Nordisk Limited (factor VIII) • Octapharma Limited (factor VIII, octaplas)

Provisional stakeholder list for the evaluation of apadamtase alfa for treating congenital thrombotic thrombocytopenic purpura caused by ADAMTS-13 deficiency ID6192

Issue date: January 2026

Provisional Consultees	Provisional Commentators (no right to submit or appeal)
<ul style="list-style-type: none"> • British Society for Haemostasis and Thrombosis • Clinical Leaders of Thrombosis • Haemophilia Nurses Association • Neonatal and Paediatric Pharmacists Group • NHS Blood and Transplant • Royal College of General Practitioners • Royal College of Nursing • Royal College of Obstetricians and Gynaecologists • Royal College of Paediatrics and Child Health • Royal College of Pathologists • Royal College of Physicians • Royal Pharmaceutical Society • Royal Society of Medicine • UK Clinical Pharmacy Association • UK Forum on Haemoglobin disorders • UK Haemophilia Centre Doctors' Organisation • UK ITP Forum • UK National Screening Committee <p><u>Others</u></p> <ul style="list-style-type: none"> • Birmingham TTP Centre • Bristol TTP Centre • Cambridge TTP Centre • Department of Health and Social Care • Leicester TTP Centre • Liverpool TTP Centre • London TTP Centre • NHS England • Nottingham TTP • Oxford TTP Centre • Sheffield TTP Centre • Southampton TTP Centre 	<p><u>Relevant research groups</u></p> <ul style="list-style-type: none"> • Foundation for Liver Research • Genomics England • MRC Clinical Trials Unit • National Institute for Health Research • Thrombosis Research Institute • UK TTP registry <p><u>Associated Public Health groups</u></p> <ul style="list-style-type: none"> • Public Health Wales • UK Health Security Agency

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 stakeholder list, and which organisations we should include that have a particular focus on relevant equality issues.

Definitions:

Consultee or commentator stakeholders are provisional until a signed Confidentiality Agreement & Undertaking form is submitted to NICE at the evaluation stage. Participating stakeholders will be listed on the project information page for the evaluation.

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 Social Care 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 Draft Guidance (FDG).

All non-company consultees are invited to submit a statement relevant to the group they are representing, respond to consultations, nominate clinical or patient experts and have the right to appeal against the Final Draft Guidance (FDG).

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 FDG 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]); other groups (for example, the NHS Confederation and the British National Formulary).

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