

# **Trends Underlying Positive and Negative Decision-Making for New Treatments Targeting Rare Diseases Appraised by NICE in 2023**

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[HTA214: Supplementary Materials](#)



Table 1: Included NICE TA and HST for treatments targeting rare diseases published in 2023

Reference ID	Date guidance was last updated	Title
TA937	2023-12-20	Targeted-release budesonide for treating primary IgA nephropathy
TA755	2023-12-15	Risdiplam for treating spinal muscular atrophy
HST29	2023-12-13	Velmanase alfa for treating alpha-mannosidosis
TA927	2023-10-17	Glofitamab for treating relapsed or refractory diffuse large B-cell lymphoma after 2 or more systemic treatments
TA915	2023-10-04	Pegunigalsidase alfa for treating Fabry disease
HST28	2023-09-20	Birch bark extract for treating epidermolysis bullosa
HST27	2023-07-26	Afamelanotide for treating erythropoietic protoporphyria
TA894	2023-06-07	Axicabtagene ciloleucel for treating relapsed or refractory follicular lymphoma
TA895	2023-06-07	Axicabtagene ciloleucel for treating relapsed or refractory diffuse large B-cell lymphoma after first-line chemoimmunotherapy
TA896	2023-06-07	Bulevirtide for treating chronic hepatitis D
TA897	2023-06-06	Daratumumab with bortezomib and dexamethasone for previously treated multiple myeloma
TA891	2023-05-31	Ibrutinib with venetoclax for untreated chronic lymphocytic leukaemia
TA892	2023-05-31	Mosunetuzumab for treating relapsed or refractory follicular lymphoma
TA881	2023-05-03	Ripretinib for treating advanced gastrointestinal stromal tumour after 3 or more treatment
TA883	2023-05-03	Tafasitamab with lenalidomide for treating relapsed or refractory diffuse large B-cell lymphoma
HST25	2023-04-19	Lumasiran for treating primary hyperoxaluria type 1
HST15	2023-04-19	Onasemnogene abeparvovec for treating spinal muscular atrophy
HST26	2023-04-19	Eladocagene exuparvovec for treating aromatic L-amino acid decarboxylase deficiency

Reference ID	Date guidance was last updated	Title
HST24	2023-04-19	Onasemnogene abeparvovec for treating presymptomatic spinal muscular atrophy
HST23	2023-03-01	Asfotase alfa for treating paediatric-onset hypophosphatasia
TA872	2023-02-28	Axicabtagene ciloleucel for treating diffuse large B-cell lymphoma and primary mediastinal large B-cell lymphoma after 2 or more systemic therapies
HST22	2023-02-22	Ataluren for treating Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene
TA870	2023-02-22	Ixazomib with lenalidomide and dexamethasone for treating relapsed or refractory multiple myeloma
TA868	2023-02-15	Vutrisiran for treating hereditary transthyretin-related amyloidosis
TA863	2023-02-01	Somatrogon for treating growth disturbance in children and young people aged 3 years and over
TA864	2023-02-01	Nintedanib for treating idiopathic pulmonary fibrosis when forced vital capacity is above 80% predicted
TA860	2023-01-18	Maribavir for treating refractory cytomegalovirus infection after transplant

**Abbreviations:** HST: highly specialised technology; ID: identification; TA: technology appraisal.