

# **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 protoporphyrina   |
| 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                     | Somatrogan 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.