

Specialty	NICE / AWMSG Reference	Medicine Generic Name	Indication	Age Range	Automatic Approval YES/ NO	Continuation Form YES/ NO/ N/A	Treatment Providers	Commission Policy / Policy Position Statement
Metabolic/ Lysosomal Storage Disorder	AWMSG 1107	Agalsidase alfa	Treatment of Fabry disease	(0-99)	YES	YES	Cardiff and Vale University Health Board Salford Royal NHS Foundation Trust, Queen Elizabeth Hospital Birmingham Manchester University Hospital	CP55
Metabolic/ Lysosomal Storage Disorder	AWMSG 12	Agalsidase beta	Treatment of Fabry disease	(8-99)	YES	YES	Cardiff and Vale University Health Board Salford Royal NHS Foundation Trust Queen Elizabeth Hospital Birmingham Manchester University Hospital	CP55
Metabolic/ Lysosomal Storage Disorder	AWMSG17	Alglucosidase alfa	Treatment of Pompe disease	(16-99)	YES	YES	Cardiff and Vale University Health Board Salford Royal NHS Foundation Trust Queen Elizabeth Hospital Birmingham Manchester University Hospital	CP55
Rheumatology	TA685	Anakinra	Treatment of Systemic juvenile idiopathic arthritis (JIA)	(0-17)	YES	YES	Cardiff and Vale University Health Board Alder Hey Childrens' Hospital, Liverpool University Hospitals Bristol Birmingham Women's & Children Hospital Royal Free Hospital Guy's and St. Thomas Hospital NHS Foundation Trust Great Ormond Street Hospital for Children NHS Foundation Trust	In development
Metabolic Bone Disorder/Endocrinology	HST23	Asfotase alfa	Treatment of Paediatric-onset hypophosphatasia	(0-99)	YES	NO	Cardiff and Vale University Health Board Birmingham Childrens Hospital Sheffield Royal Infirmary	PP156
Musculoskeletal	HST22	Ataluren	Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene	(2-99)	YES	NO	Cardiff and Vale University Health Board Alderhey Childrens' Hospital Liverpool Birmingham Womens & Childrens Hospital University Hospitals Birmingham	CP118
Metabolic/ Lysosomal Storage Disorder	HST18	Atidarsagene autotemcel	Treatment of metachromatic leukodystrophy	(0-17)	YES	NO	St Mary's Hospital, Manchester (treatment centre) Birmingham Childrens Hospital (referring centre) Great Ormond Street Hospital (referring centre)	PP257
Metabolic/ Lysosomal Storage Disorder	TA821	Avalglucosidase alfa	Treatment of Pompe disease	(0-99)	YES	YES	Cardiff and Vale University Health Board Salford Royal NHS Foundation Trust Queen Elizabeth Hospital Birmingham Manchester University Hospital	CP55
Oncology	TA895	Axicabtagene ciloleucel	Treatment of relapsed or refractory diffuse large B-cell lymphoma after first-line chemoimmunotherapy	(18-99)	YES	YES	Cardiff and Vale University Health Board University Hospital Bristol NHS Foundation Trust University Hospital Birmingham NHS Foundation Trust Kings College Hospital NHS Foundation Trust Christie Hospital NHS Foundation Trust Manchester University NHS Foundation Trust	In development - PP282

Oncology	TA559	Axicabtagene ciloleuce	Treatment of Diffuse large B-cell lymphoma, mediastinal B-cell lymphoma and follicular lymphoma	(16-99)	YES	YES	Cardiff and Vale University Health Board University Hospital Bristol NHS Foundation Trust University Hospital Birmingham NHS Foundation Trust Kings College Hospital NHS Foundation Trust Christie Hospital NHS Foundation Trust Manchester University NHS Foundation Trust	CP175
Immunology/ Rheumatology	TA752	Belimumab	Treatment of active autoantibody-positive systemic lupus erythematosus	(0-17)	YES	YES	Cardiff and Vale University Health Board Alder Hey Children's Hospital Bristol Royal Hospital for Children	N
Cardiology	TA738	Berotralstat	Prevention of recurrent attacks of hereditary angioedema	(12-99)	YES	YES	Cardiff and Vale University Health Board Salford , University Hospitals Queen Elizabeth Hospital Birmingham Birmingham Women's and Children Alder Hey Childrens Hospital	PP236
Oncology	TA893	Brexucabtagene autoleuce	Treatment of relapsed or refractory B-cell acute lymphoblastic leukaemia in people 26 years and over	(26-99)	YES	N/A	Kings College Hospital NHS Foundation Trust Christie Hospital NHS Foundation Trust University Hospital Bristol NHS Foundation Trust	CP175
Metabolic Bone Disorder/Endocrinology	HST8	Burosumab	Treatment of X-linked hypophosphataemia in children and young people	(0-17)	YES	NO	Cardiff and Vale University Health Board Bristol Children's Hospital Alder Hey Childrens' Hospital Birmingham Childrens' Hospital University Hospitals Bristol	PP177
Rheumatology/ Immunology	N/A	Canakinumab	Treatment of periodic fever syndromes: TRAPS, HIDS/MKD and FMF in ages 2 and older	(2-99)	YES	YES	Cardiff and Vale University Health Board Alder Hey Children's Hospital Birmingham Children's Hospital Bristol Children's Hospital Manchester Children's Hospital Leeds Teaching Hospitals NHS Trust	PP228
Neurology	TA614	Cannabidiol	Adjuvant treatment of seizures associated with Dravet syndrome	(2-17)	YES	YES	Cardiff and Vale University Health Board The Walton Centre, Liverpool Alderhey Childrens' Hospital Birmingham Womens & Children Hospital	PP203
Neurology	TA615	Cannabidiol	Adjuvant treatment of seizures associated with Lennox-Gastaut syndrome	(2-17)	YES	YES	Cardiff and Vale University Health Board The Walton Centre, Liverpool Alderhey Childrens' Hospital Liverpool Birmingham Womens & Children Hospital Bristol Children's Hospital	In development - PP272
Neurology	TA873	Cannabidiol	Adjunctive therapy of seizures associated with tuberous sclerosis complex (TSC) for patients 2 years of age and older	(2-17)	YES	YES	Cardiff and Vale University Health Board The Walton Centre, Liverpool Alder Hey Children's Hospital	In development PP262
Metabolic/ Lysosomal Storage Disorder	HST12	Cerliponase alfa	Treatment of neuronal ceroid lipofuscinosis type 2	(0-17)	YES	YES	Cardiff and Vale University Health Board Alderhey Childrens' Hospital, Liverpool Birmingham Womens & Children Hospital University Hospitals Bristol	CP55

Metabolic/ Lysosomal Storage Disorder	TA912	Cipaglucosidase & miglustat	Treatment of late-onset Pompe disease	(18-99)	Yes	YES	Cardiff and Vale University Health Board Betsi Cadwaladr University Health Board Salford Royal NHS Foundation Trust	CP55
Oncology	PP253 WHSSC Policy	Dexrazoxane	To prevent cardiotoxicity in children (0-15 years) receiving high-dose anthracyclines or related drugs for the treatment of cancer	(0-15)	YES	NO	Cardiff and Vale University Health Board Velindre NHS Trust Swansea University Health Board Betsi Cadwaladr University Health Board Alder Hey Children's Hospital Birmingham Childrens Hospital Royal Manchester Children's Hospital Great Ormond Street Hospital	PP253
Haematology	HST1	Eculizumab	Treatment of Atypical haemolytic uraemic syndrome in adults and children.	(16-99)	YES	NO	Betsi Cadwaladr University Health Board Hywel Dda University Health Board Swansea Bay University Health Board Cardiff and Vale University Health Board Sciensus (Healthcare at Home) Royal Liverpool & Broadgreen Hospital Manchester University Hospital Leeds Teaching Hospital Kings College Hospital NHS Foundation Trust Alderhey Childrens' Hospital, Liverpool	PP237
Metabolic Disorder	HST26	Eladocagene exuparvovec	Treatment of aromatic L-amino acid decarboxylase deficiency	(18 months-99)	YES	N/A	Great Ormond Street Hospital	In development - PP281
Metabolic/ Lysosomal Storage Disorder	HST5	Eliglustat	Treatment of Type 1 Gaucher disease	(18-99)	YES	YES	Cardiff and Vale University Health Board Salford Royal NHS Foundation Trust Birmingham Manchester University Hospital	CP55
Metabolic Disorder	HST 19	Elosulfase alfa	Treatment of Mucopolysaccharidosis type IVa	(0-99)	YES	YES	Cardiff and Vale University Health Board	CP55
Neurology	AWMSG 2142	Everolimus	Adjunctive treatment of patients aged 2 years and older whose refractory partial-onset seizures, with or without secondary generalisation, are associated with tuberous sclerosis complex (TSC).	(2-17)	YES	YES	Betsi Cadwaladr University Health Board Cardiff and Vale University Health Board Alder Hey Childrens' Hospital, Liverpool The Walton Centre, Liverpool	PP250 (in development)
Neurology	AWMSG 1156	Everolimus	Treatment of adult patients with renal angiomyolipoma associated with tuberous sclerosis complex who are at risk of complications (based on factors such as tumour size or presence of aneurysm, or presence of multiple or bilateral tumours) but who do not require immediate surgery	(18-99)	YES		Betsi Cadwaladr University Health Board Cardiff and Vale University Health Board	PP274 (in development)
Neurology	AWMSG 1156	Everolimus	Treatment of patients with subependymal giant cell astrocytoma associated with tuberous sclerosis complex who require therapeutic intervention but are not amenable to surgery	(2-99)	YES		Betsi Cadwaladr University Health Board Cardiff and Vale University Health Board Alder Hey Childrens' Hospital, Liverpool The Walton Centre, Liverpool	PP273 (in development)

Neurology	TA808	Fenfluramine	Treatment of seizures associated with Dravet syndrome	(2-17)	YES	YES	Cardiff and Vale University Health Board The Walton Centre, Liverpool Alderhey Childrens' Hospital, Liverpool Birmingham Womens & Children Hospital	In developemnt
Hepatology	HST16	Givosiran	Treatment of Acute hepatic porphyria	(12-99)	YES	YES	Cardiff and Vale University Health Board King's College Hospital NHS Foundation Trust Salford Royal NHS Foundation Trust University Hospitals Birmingham Cambridge University Hospitals NHS Foundation Trust	Y - PP252
Neurology	HST9	Inotersen	Treatment of hereditary transthyretin-related amyloidosis	(16-99)	YES	YES	NHS National Amyloidosis Centre Division of Medicine (Royal Free Campus) University College London Hospital	PP187
Oncology	TA677	KTE-X19 (Autologous anti-CD19-transduced CD3+ cells)	Treatment of Relapsed or refractory mantle cell lymphoma	(16-99)	YES	N/A	Cardiff and Vale University Health Board University Hospital Bristol NHS Foundation Trust University Hospital Birmingham NHS Foundation Trust Kings College Hospital NHS Foundation Trust Christie Hospital NHS Foundation Trust Manchester University NHS Foundation Trust	PP185
Metabolic Disorder	AWMSG 180	Laronidase	Enzyme replacement therapy in patients with mucopolysaccharidosis I (MPS I)	(0-99)	YES	YES	Cardiff and Vale University Health Board Salford Royal NHS Foundation Trust Cambridge University Hospital NHS Foundation Trust	CP55
Infection	TA591	Letermovir	For preventing cytomegalovirus disease after a stem cell transplant	(0-17)	YES	NO	Cardiff and Vale University Health Board Swansea University Health Board Clatterbridge Hospital Christie Hospital NHS Foundation Trust Birmingham Women's and Children's Hospital Queen Elizabeth Hospital Birmingham University Hospitals Bristol NHS Foundation Trust Newcastle Hospitals NHS Foundation Trust King's College, Imperial College University College London Hospitals Barts Health NHS Trust	NA
Renal	HST25	Lumasiran	Treatment of primary hyperoxaluria type 1	(0-99)	YES	YES	Cardiff and Vale University Health Board Birmingham Women's and Children's NHS Foundation Trust Queen Elizabeth Hospital Birmingham Great Ormond Street Hospital for Children NHS Trust Royal Free London NHS foundation trust	PP277
Oncology	TA539	Lutetium (177Lu) oxodotreotide	Treatment of Unresectable or metastatic neuroendocrine tumours in adults.	(16-99)	YES	N/A	Royal Free Hospital, London University Hospitals Birmingham NHS Foundation Trust	PP195
Metabolic/ Lysosomal Storage Disorder	HST4	Migalastat	Treatment of Fabry disease	(12-99)	YES	YES	Cardiff and Vale University Health Board Salford Royal NHS Foundation Trust, Manchester Queen Elizabeth Hospital Birmingham Manchester University Hospital	CP55

Neurology	TA588	Nusinersen	Treatment of Spinal muscular atrophy	(0-17)	YES	YES	Cardiff and Vale University Health Board University Hospitals Bristol Birmingham Women's & Children Hospital University Hospitals Birmingham	PP191
Metabolic Disorder	HST17	Odevixibat	Treatment of Progressive familial intrahepatic cholestasis (PFIC) in patients aged 6 months or older	(6 months - 17)	YES	YES	Cardiff and Vale University Health Board Anneurin Bevan University Health Board Swansea Bay University Health Board Birmingham Childrens Hospital Leeds Teaching Hospitals NHS Trust Kings College Hospital NHS Foundation Trust	PP249
Neurology	HST15	Onasemnogene abeparvovec	Treatment of Spinal muscular atrophy	(0-1)	YES	YES	Sheffield Royal Infirmary Manchester University NHS Foundation Trust Guy's and St. Thomas Hospital University Hospital Bristol NHS Foundation Trust	In developemnt
Neurology	HST24	Onasemnogene abeparvovec	Treatment of presymptomatic spinal muscular atrophy in babies 12 months and under	(0-12 months)	YES	NO	Sheffield Royal Infirmary Manchester University NHS Foundation Trust Guy's and St. Thomas Hospital University Hospital Bristol NHS Foundation Trust	In development
Neurology	HST10	Patisiran	Treatment of hereditary transthyretin amyloidosis	(16-99)	YES	YES	NHS National Amyloidosis Centre, Division of Medicine (Royal Free Campus) University College London Hospital NHS Foundation Trust	PP187
Haematology	TA778	Pegcetacoplan	Treatment of Paroxysmal nocturnal haemoglobinuria	(18-99)	YES	NO	Betsi Cadwaladr University Health Board Hywel Dda University Health Board Swansea Bay University Health Board Cardiff and Vale University Health Board Royal Liverpool & Broadgreen Hospital Manchester University Hospital Leeds Teaching Hospital Kings College Hospital NHS Foundation Trust	In developemnt
Metabolic/ Lysosomal Storage Disorder	TA915	Pegunigalsidase alfa	Treatment of adults with Fabry disease	(18-99)	YES	YES	Cardiff and Vale University Health Board Salford Royal NHS Foundation Trust, Manchester Queen Elizabeth Hospital Birmingham Manchester University Hospital	CP55
Haematology	TA698	Ravulizumab	Treatment of Paroxysmal nocturnal haemoglobinuria	(16-99)	YES	NO	Betsi Cadwaladr University Health Board Hywel Dda University Health Board Swansea Bay University Health Board Cardiff and Vale University Health Board Sciensus (Healthcare at Home) Royal Liverpool & Broadgreen Hospital Manchester University Hospital Leeds Teaching Hospital Kings College Hospital NHS Foundation Trust University Hospitals Birmingham NHS Foundation Trust	In development - CP152

Haematology	TA710	Ravulizumab	Treatment of Atypical haemolytic uraemic syndrome (aHUS)	(16-99)	YES	NO	Betsi Cadwaladr University Health Board Hywel Dda University Health Board Swansea Bay University Health Board Cardiff and Vale University Health Board Sciensus (Healthcare at Home) Royal Liverpool & Broadgreen Manchester University NHS Foundation Trust Leeds Teaching Hospital Kings College Hospital NHS Foundation Trust Alder Hey Childrens' Hospital, Liverpool	In developemnt
Haematology	AWMSG 322 / 4869	Ravulizumab	Treatment of Paediatric patients with a body weight of 10 kg or above with paroxysmal nocturnal haemoglobinuria (PNH)	(0-17)	YES	NO	Cardiff and Vale University Health Board Alder Hey Children's Hospital, Liverpool Birmingham Women's and Childrens Bristol Childrens Hospitals	In developemnt
Neurology	TA755	Risdiplam	Treatment of Spinal muscular atrophy in children and adults	(0-17)	YES	YES	Cardiff and Vale University Health Board University Hospital Bristol NHS Foundation Trust Alderhey Childrens' Hospital, Liverpool	PP240
Metabolic Disorder	TA729/ PP223	Sapropterin Dihydrochloride	Treatment of Hyperphenylalaninaemia (HPA) in phenylketonuria (PKU) and tetrahydrobiopterin (BH4) disorders	(0-99)	YES	YES	Betsi Cadwaladr University Health Board Cardiff and Vale University Health Board Alderhey Childens Hospital Manchester University Hospital Birmingham Childrens Hospital	PP223
Neurology	HST20	Selumetinib	Treatment of Symptomatic and inoperable plexiform neurofibromas associated with type 1 neurofibromatosis in children aged 3 years and over	(3-99)	YES	YES	Guy's and St Thomas' NHS Foundation Trust (Evelina London Children's Hospital) Manchester University NHS Foundation Trust	Y - PP267 due for publication Oct 2023
Endocrinology	TA863/ ID5086	Somatogon	Growth disturbance in children and young people aged 3 years and over	(3-17)	YES	NO	Cardiff and Vale University Health Board Alder Hey Childrens' Hospital Birmingham Children's Hospital	In development
Intestinal Disorder	TA804	Teduglutide	Treatment of Short bowel syndrome	(1-99)	YES	YES	Cardiff and Vale University Health Board Salford Royal NHS Foundation Trust, Manchester Alder Hey Childrens Hospital	Y - PP265
Neurology	TA303/ 4033	Teriflunomide	Treatment of relapsing-remitting multiple sclerosis in children aged 10-17 years.	(10-17)	YES	YES	Cardiff and Vale University Health Board Alder Hey Childrens' Hospital, Liverpool	NA
Respiratory	TA880	Tezepelumab	Treatment of severe asthma	(12-17)	YES	YES	Cardiff and Vale University Health Board Alder Hey Childrens' Hospital Manchester University Hospitals NHS Foundation Trust	NA
Oncology	TA554	Tisagenlecleucel-T	Treatment of Relapsed or refractory B-cell acute lymphoblastic leukaemia in people up to 25 years	(0-25)	YES	YES	Cardiff and Vale University Health Board Great Ormond Street Hospital for Children NHS Trust	PP185
Rheumatology	TA735	Tofacitinib	Treatment of Juvenile idiopathic arthritis	(2-17)	YES	YES	Cardiff and Vale University Health Board Alder Hey Childrens Hospital Bristol Childrens Hospital	PP229

Immunology	OW24	<u>Ustekinumab</u>	Treatment of inflammatory bowel disease (ulcerative colitis and Crohn's disease) in children	(0-17)	YES	YES	Cardiff and Vale University Health Board (treatment initiation) All Local Welsh Health Boards (treatment continuation) Alder Hey Childrens' Hospital Birmingham Childrens Hospital	N/A
Immunology	OW25	<u>Vedolizumab</u>	Treatment of inflammatory bowel disease (ulcerative colitis and Crohn's disease) in children	(0-17)	YES	YES	Cardiff and Vale University Health Board (treatment initiation) All Local Welsh Health Boards (treatment continuation) Alder Hey Children's Hospital Birmingham Children's Hospital	N/A
Metabolic/ Lysosomal Storage Disorder	AWMSG 1214	<u>Velaglucerase alfa</u>	Treatment of Type 1 Gaucher disease	(18-99)	YES	YES	Cardiff and Vale University Health Board Salford Royal NHS Foundation Trust, Manchester Queen Elizabeth Hospital Birmingham Manchester University Hospital	<u>CP55</u>
Metabolic Disorder	HST13	<u>Volanesorsen</u>	Treatment of Familial chylomicronaemia syndrome	(16-99)	YES	N/A	Cardiff and Vale University Health Board Wrexham Maelor Hospital	<u>PP217</u>
Ophthalmology	HST11	<u>Voretigene neparvovec</u>	Treatment of Inherited retinal dystrophies caused by RPE65 gene mutations	(16-99)	YES	NO	Great Ormond Street Hospital for Children NHS Trust Manchester Eye Hospital Moorefields Eye Hospital, London Oxford Eye Hospital	<u>PP196</u>
Neurology	TA868	<u>Vutrisiran</u>	Treatment of hereditary transthyretin-related amyloidosis	(18-99)	YES	YES	NHS National Amyloidosis Centre, Division of Medicine (Royal Free Campus) University College London Hospital NHS Foundation Trust	<u>PP187</u>