

List of Drugs for Rare Diseases in Hong Kong

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No.	Ingredients	Reference Indication	By BNF Therapeutic Class	Drug Formulary Category*	Indication Source
1	Adalimumab	- Hidradenitis suppurativa	Skin	Safety Net (Samaritan Fund)	HA Website
2	Adalimumab	- Non-infectious intermediate, posterior and panuveitis - Paediatric chronic non-infectious anterior uveitis	Eye	Safety Net (Samaritan Fund)	HA Website
3	Adalimumab	- Juvenile idiopathic arthritis	Musculoskeletal and joint disease	Safety Net (Samaritan Fund)	HA Website
4	Agalsidase Alfa	- Fabry Disease	NA	Special Drugs	HA Website
5	Agalsidase Beta	- Fabry Disease	NA	Special Drugs	HA Website
6	Alemtuzumab	- Relapsing forms of multiple sclerosis (RRMS)	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
7	Alglucosidase Alfa	- Pompe Disease	NA	Special Drugs	HA Website
8	Ambrisentan	- Pulmonary Arterial Hypertension (PAH)	Cardiovascular System	Special Drugs	MIMS HK
9	Avalglucosidase alfa	- Pompe Disease	NA	Special Drugs	HA Website
10	Baclofen	- Muscle spasms caused by certain conditions (such as multiple sclerosis, spinal cord injury/disease)	Musculoskeletal and joint disease	Special Drugs	HA Website
11	Belumosudil	- Graft versus host disease (GvHD)	Malignant Disease and Immunosuppression	Self-financed item	HA Website
12	Bosentan	- Pulmonary Arterial Hypertension (PAH)	Cardiovascular System	Special Drugs	MIMS HK
13	Burosumab	- X-linked hypophosphataemia	NA	Safety Net (Community Care Fund)	HA Website
14	Canakinumab	- Cryopyrin-associated periodic syndromes (CAPS) - Familial Mediterranean fever - Hyperimmunoglobulin D syndrome / mevalonate kinase deficiency - Systemic juvenile idiopathic arthritis - Tumour necrosis factor receptor associated periodic syndrome	Malignant disease and immunosuppression	Safety Net (Samaritan Fund)	HA Website
15	Carglumic acid	- Acute hyperammonaemia in methylmalonic and propionic acidemia	Nutrition and Blood	Special Drugs	HA Website
16	Carglumic acid	- For treatment of chronic hyperammonaemia in N-acetylglutamate synthase (NAGS) deficiency, methylmalonic acidemia (MMA) or propionic acidemia (PA)	Nutrition and Blood	Safety Net (Community Care Fund)	HA Website
17	Cladribine	- Hairy cell leukemia	Malignant Disease and Immunosuppression	Special Drugs	HA Website
18	Cladribine (Mavenclad)	- Relapsing forms of multiple sclerosis (RRMS)	Malignant Disease and Immunosuppression	Safety Net (Samaritan Fund)	HA Website
19	Deferiprone (Kelfer)	- Thalassemia Major	Nutrition and Blood	Special Drugs	MIMS HK
20	Dimethyl fumarate	- Relapsing forms of multiple sclerosis (RRMS)	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
21	Eculizumab	- Atypical Hemolytic Uremic Syndrome (aHUS)	NA	Safety Net (Community Care Fund)	HA Website
22	Eculizumab	- Paroxysmal nocturnal hemoglobinuria (PNH)	NA	Safety Net (Samaritan Fund)	HA Website
23	Elosulfase Alfa	- Mucopolysaccharidosis IVA	NA	Special Drugs	HA Website
24	Eltrombopag	- Chronic immune thrombocytopenia - Severe aplastic anaemia (SAA)	Nutrition and Blood	Safety Net (Samaritan Fund)	HA Website
25	Epoprostenol	- Primary Pulmonary Hypertension	Cardiovascular System	General Drugs	MIMS HK
26	Everolimus	- Renal angiomyolipoma associated with tuberous sclerosis complex (TSC) - Subependymal giant cell astrocytoma associated with tuberous sclerosis complex (TSC) - Adjunct in refractory partial seizures associated with tuberous sclerosis complex	Malignant Disease and Immunosuppression	Based on different indication, this drug had been listed on both special drugs, safety net (Samaritan Fund) and Self-financed item.	MIMS HK
27	Evolocumab	- Homozygous Familial Hypercholesterolemia (HoFH)	Cardiovascular System	Special Drugs	MIMS HK
28	Fingolimod	- Relapsing forms of multiple sclerosis (RRMS)	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
29	Galsulfase	- Mucopolysaccharidosis VI	NA	Special Drugs	HA Website
30	Growth Hormone	- Dwarfism	Endocrine System	Safety Net (Samaritan Fund)	HA Website
31	Human C1-esterase inhibitor	- Hereditary angioedema (HAE)	Respiratory System	Special Drugs	HA Website
32	Icatibant	- Hereditary angioedema (HAE)	Respiratory System	Special Drugs	HA Website
33	Idursulfase	- Mucopolysaccharidosis II	NA	Special Drugs	HA Website
34	Iloprost	- Pulmonary Arterial Hypertension (PAH)	Cardiovascular System	Special Drugs	MIMS HK
35	Imiglucerase	- Gaucher's Disease	NA	Special Drugs	HA Website
36	Interferon-Beta	- Multiple Sclerosis (MS)	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK

37	Interferon-Gamma 1b	- Chronic Granulomatous Disease	Malignant Disease and Immunosuppression	Special Drugs	HA Website
38	Iptacopan	- Paroxysmal nocturnal hemoglobinuria (PNH)	NA	Safety Net (Community Care Fund)	HA Website
39	Laronidase	- Mucopolysaccharidosis I	NA	Special Drugs	HA Website
40	Luspatercept	- Transfusion-dependent anaemia due to very low, low and intermediate-risk myelodysplastic syndrome (MDS) with ring sideroblasts	Nutrition and Blood	Safety Net (Samaritan Fund)	HA Website
41	Luspatercept	- Transfusion-dependent anaemia associated with beta-thalassaemia	Nutrition and Blood	Safety Net (Samaritan Fund)	HA Website
42	Macitentan	- Pulmonary Arterial Hypertension (PAH)	Cardiovascular System	Special Drugs	MIMS HK
43	Mavacamten	- Obstructive Hypertrophic Cardiomyopathy	Cardiovascular System	Special Drugs	MIMS HK
44	Mepolizumab	- Hypereosinophilic syndrome (HES)	Malignant Disease and Immunosuppression	Self-financed item	HA Website
45	Mepolizumab	- Relapsing-remitting or refractory eosinophilic granulomatosis with polyangiitis (EGPA) (add-on treatment)	Malignant Disease and Immunosuppression	Safety Net (Samaritan Fund)	HA Website
46	Migalastat	- Fabry Disease	NA	Special Drugs	HA Website
47	Natalizumab	- Relapsing forms of multiple sclerosis (RRMS)	Malignant Disease and Immunosuppression	Safety Net (Samaritan Fund)	HA Website
48	Nintedanib	- Idiopathic Pulmonary Fibrosis (IPF)	Respiratory System	Safety Net (Samaritan Fund)	HA Website
49	Nintedanib	- Rheumatic disease associated progressive fibrosing interstitial lung disease (PF-	Respiratory System	Safety Net (Samaritan Fund)	HA Website
50	Nusinersen	- Infantile Onset Spinal Muscular Atrophy (SMA) - Childhood Onset Spinal Muscular Atrophy (SMA) - Pre-symptomatic Spinal Muscular Atrophy (SMA)	NA	Safety Net (Community Care Fund)	HA Website
51	Ofatumumab	- Relapsing forms of multiple sclerosis (RRMS)	Malignant Disease and Immunosuppression	Special Drugs	HA Website
52	Onasemnogene abeparvovec	- Infantile Onset Spinal Muscular Atrophy (SMA) - Pre-symptomatic Spinal Muscular Atrophy (SMA)	NA	Safety Net (Community Care Fund)	HA Website
53	Pasireotide	- Cushing's disease / Acromegaly	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
54	Penicillamine	- Wilson's Disease	Musculoskeletal and Joint Diseases	General Drugs	MIMS HK
55	Ravulizumab	- Atypical Hemolytic Uremic Syndrome (aHUS)	NA	Safety Net (Community Care Fund)	HA Website
56	Ravulizumab	- Paroxysmal nocturnal hemoglobinuria (PNH)	NA	Safety Net (Community Care Fund)	HA Website
57	Riluzole	- Amyotrophic Lateral Sclerosis	Central Nervous System	Special Drugs	MIMS HK
58	Riociguat	- Chronic Thromboembolic Pulmonary Hypertension (CTEPH)	Cardiovascular System	Special Drugs	MIMS HK
59	Risdiplam	- Infantile Onset Spinal Muscular Atrophy (SMA) - Childhood Onset Spinal Muscular Atrophy (SMA)	NA	Safety Net (Community Care Fund)	HA Website
60	Risedronate	- Paget's disease of bone	Endocrine System	Special Drugs	MIMS HK
61	Rituximab	- Severe, active non-life-threatening granulomatosis with polyangiitis / microscopic polyangiitis	Malignant Disease and Immunosuppression	Safety Net (Samaritan Fund)	HA Website
62	Rituximab	- Pemphigus vulgaris	Malignant Disease and Immunosuppression	Safety Net (Samaritan Fund)	HA Website
63	Rufinamide	- Adjunct for seizures associated with the Lennox-Gastaut syndrome (LGS)	Central Nervous System	Special Drugs	MIMS HK
64	Ruxolitinib	- Graft versus host disease (GvHD)	Malignant Disease and Immunosuppression	Safety Net (Samaritan Fund)	HA Website
65	Selexipag	- Pulmonary Arterial Hypertension (PAH)	Cardiovascular System	Special Drugs	MIMS HK
66	Sildenafil	- Pulmonary Arterial Hypertension (PAH)	Cardiovascular System	Special Drugs	MIMS HK
67	Siltuximab	- Multicentric Castlemann's disease	Malignant Disease and Immunosuppression	Safety Net (Samaritan Fund)	HA Website
68	Siponimod	- Secondary progressive multiple sclerosis (SPMS)	Malignant Disease and Immunosuppression	Safety Net (Samaritan Fund)	HA Website
69	Tafamidis	- For the treatment of hereditary transthyretin amyloidosis in adult patients with cardiomyopathy (ATTR-CM)	NA	Safety Net (Community Care Fund)	HA Website
70	Tafamidis	- wild-type transthyretin-mediated amyloidosis cardiomyopathy	NA	Self-financed item	MIMS HK
71	Tafamidis Meglumine	- For the treatment of transthyretin amyloidosis in adult patients with stage 1 symptomatic polyneuropathy to delay peripheral neurologic impairment	NA	Safety Net (Community Care Fund)	HA Website
72	Teriflunomide	- Relapsing remitting multiple sclerosis in adults (RRMS)	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
73	Tisagenlecleucel	- Paediatric and young adult patients up to 25 years of age with B-cell acute lymphoblastic leukaemia that is refractory, in relapse posttransplant or in second or later relapse - Adult patients with relapsed or refractory diffuse large B-cell lymphoma after two or	NA	Safety Net (Community Care Fund)	HA Website
74	Tobramycin	- Cystic Fibrosis	Infection	General Drugs	MIMS HK
75	Tocilizumab	- Juvenile Idiopathic Arthritis - Systemic juvenile idiopathic arthritis - Refractory giant cell arteritis	Musculoskeletal and Joint Diseases	Safety Net (Samaritan Fund)	HA Website
76	Zinc Acetate	- Wilson's Disease	Nutrition and Blood	General Drugs	FDA

General Drugs	These are drugs with well-established indications and cost- effectiveness which are available for general use as indicated by patients with relevant clinical conditions and provided at standard fees and charges in public hospitals and clinics.
Special Drugs	These are drugs used under specific clinical conditions with specific specialist authorisation. Special drugs are provided at standard fees and charges in public hospitals and clinics when prescribed under specific clinical conditions. Patients who do not meet the specified clinical conditions but choose to use Special drugs are
Self-financed Items (SFIs) with Safety Net	These are drugs which are proven to be of significant clinical benefits but are very expensive for HA to provide as part of its standard services. These drugs are not covered by the standard fees and charges in public hospitals and clinics. Patients who require these drugs and can afford the costs have to purchase the drugs at their own expense. A safety net is provided through relevant funds to subsidize the drug expenses of patients who have financial difficulties.
SFIs without Safety Net	These include drugs with preliminary medical evidence only, drugs with marginal benefits over available alternatives but at significant higher costs, and lifestyle drugs (e.g. anti-obesity drugs). These drugs are not provided as part of HA's standard services nor covered by the standard fees and charges in public hospitals and clinics. Patients who choose to use these drugs must purchase them at their own expense.

(Source of information: <https://www.ha.org.hk/hadf/en-us/Drug-Formulary-Management.html>)