

## 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	- Primary Pulmonary Hypertension	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	Bosentan	- Pulmonary Arterial Hypertension (PAH)	Cardiovascular System	Special Drugs	MIMS HK
12	Burosumab	- X-linked hypophosphataemia	NA	Safety Net (Community Care Fund)	HA Website
13	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
14	Cladribine	- Relapsing forms of multiple sclerosis (RRMS)	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
15	Deferiprone (Kelfer)	- Thalassemia Major	Nutrition and Blood	Special Drugs	MIMS HK
16	Dimethyl fumarate	- Relapsing forms of multiple sclerosis (RRMS)	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
17	Dinutuximab Beta	- Treatment of high-risk or relapse/refractory neuroblastoma	NA	Safety Net (Community Care Fund)	HA Website
18	Eculizumab	- Atypical Hemolytic Uremic Syndrome (aHUS)	NA	Safety Net (Community Care Fund)	HA Website
19	Eculizumab	- Paroxysmal nocturnal hemoglobinuria (PNH)	NA	Safety Net (Samaritan Fund)	HA Website
20	Elosulfase Alfa	- Mucopolysaccharidosis IVA	NA	Special Drugs	HA Website
21	Eltrombopag	- Chronic immune thrombocytopenia - Severe aplastic anaemia (SAA)	Nutrition and Blood	Safety Net (Samaritan Fund)	HA Website
22	Epoprostenol	- Primary Pulmonary Hypertension	Cardiovascular System	General Drugs	MIMS HK
23	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
24	Evolocumab	- Homozygous Familial Hypercholesterolemia (HoFH)	Cardiovascular System	Special Drugs	MIMS HK
25	Fingolimod	- Relapsing forms of multiple sclerosis (RRMS)	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
26	Galsulfase	- Mucopolysaccharidosis VI	NA	Special Drugs	HA Website
27	Growth Hormone	- Dwarfism	Endocrine System	Safety Net (Samaritan Fund)	HA Website
28	Human C1-esterase inhibitor	- Hereditary angioedema (HAE)	Respiratory System	Special Drugs	HA Website
29	Icatibant	- Hereditary angioedema (HAE)	Respiratory System	Safety Net (Samaritan Fund)	HA Website
30	Idursulfase	- Mucopolysaccharidosis II	NA	Special Drugs	HA Website
31	Iloprost	- Pulmonary Arterial Hypertension (PAH)	Cardiovascular System	Special Drugs	MIMS HK
32	Imiglucerase	- Gaucher's Disease	NA	Special Drugs	HA Website
33	Interferon-Beta	- Multiple Sclerosis (MS)	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
34	Interferon-Gamma 1b	- Chronic Granulomatous Disease	Malignant Disease and Immunosuppression	Special Drugs	HA Website
35	Laronidase	- Mucopolysaccharidosis I	NA	Special Drugs	HA Website
36	Luspatercept	- Transfusion-dependent anaemia associated with myelodysplastic syndromes with ring sideroblasts	Nutrition and Blood	Self-financed item	HA Website
37	Macitentan	- Pulmonary Arterial Hypertension (PAH)	Cardiovascular System	Special Drugs	MIMS HK

38	Mepolizumab	- Hypereosinophilic syndrome (HES) - Eosinophilic granulomatosis with polyangiitis (EGPA)	Malignant Disease and Immunosuppression	Self-financed item	HA Website
39	Migalastat	- Fabry Disease	NA	Special Drugs	HA Website
40	Natalizumab	- Relapsing forms of multiple sclerosis (RRMS)	Malignant Disease and Immunosuppression	Safety Net (Samaritan Fund)	HA Website
41	Nintedanib	- Idiopathic Pulmonary Fibrosis (IPF)	Respiratory System	Safety Net (Samaritan Fund)	HA Website
42	Nintedanib	- Progressive fibrosing interstitial lung disease (PF-ILD)	Respiratory System	Self-financed item	HA Website
43	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
44	Ofatumumab	- Relapsing forms of multiple sclerosis (RRMS)	Malignant Disease and Immunosuppression	Special Drugs	HA Website
45	Onasemnogene abeparovvec	- Infantile Onset Spinal Muscular Atrophy (SMA) - Pre-symptomatic Spinal Muscular Atrophy (SMA)	NA	Safety Net (Community Care Fund)	HA Website
46	Pasireotide	- Cushing's disease / Acromegaly	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
47	Penicillamine	- Wilson's Disease	Musculoskeletal and Joint Diseases	General Drugs	MIMS HK
48	Ravulizumab	- Atypical Hemolytic Uremic Syndrome (aHUS)	NA	Safety Net (Community Care Fund)	HA Website
49	Ravulizumab	- Paroxysmal nocturnal hemoglobinuria (PNH)	NA	Safety Net (Community Care Fund)	HA Website
50	Risdiplam	- Infantile Onset Spinal Muscular Atrophy (SMA) - Childhood Onset Spinal Muscular Atrophy (SMA)	NA	Safety Net (Community Care Fund)	HA Website
51	Risedronate	- Paget's disease of bone	Endocrine System	Special Drugs	MIMS HK
52	Rituximab	- Severe, active non-life-threatening granulomatosis with polyangiitis / microscopic polyangiitis	Malignant Disease and Immunosuppression	Safety Net (Samaritan Fund)	HA Website
53	Rufinamide	- Adjunct for seizures associated with the Lennox-Gastaut syndrome (LGS)	Central Nervous System	Special Drugs	MIMS HK
54	Ruxolitinib	- Graft versus host disease (GvHD)	Malignant Disease and Immunosuppression	Self-financed item	HA Website
55	Selexipag	- Pulmonary Arterial Hypertension (PAH)	Cardiovascular System	Special Drugs	MIMS HK
56	Sildenafil citrate	- Pulmonary Arterial Hypertension (PAH)	Cardiovascular System	Special Drugs	MIMS HK
57	Siltuximab	- Multicentric Castleman's disease	Malignant Disease and Immunosuppression	Safety Net (Samaritan Fund)	HA Website
58	Siponimod	- Secondary progressive multiple sclerosis (SPMS)	Malignant Disease and Immunosuppression	Safety Net (Samaritan Fund)	HA Website
59	Tafamidis	- Hereditary Transthyretin Amyloidosis with Cardiomyopathy	NA	Safety Net (Community Care Fund)	HA Website
60	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
61	Teriflunomide	- Relapsing remitting multiple sclerosis in adults (RRMS)	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
62	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
63	Tobramycin	- Cystic Fibrosis	Infection	General Drugs	MIMS HK
64	Tocilizumab	- Juvenile Idiopathic Arthritis - Systemic juvenile idiopathic arthritis - Refractory giant cell arteritis	Musculoskeletal and Joint Diseases	Safety Net (Samaritan Fund)	HA Website
65	Zinc Acetate	- Wilson's Disease	Nutrition and Blood	General Drugs	FDA

#### Hospital Authority "Drug Formulary" categories\*

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> )