

## 香港罕見疾病藥物清單

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編號	成分名	參考適應症	藥物分類	藥物名冊分類*	適應症資料來源
1	Rufinamide	- 輔助雷葛氏症候群引起的癲癇；LGS	中樞神經系統藥物	專用藥物	MIMS HK
2	Eculizumab	- 非典型溶血性尿毒症候群：aHUS	NA	安全網藥物 (關愛基金)	醫管局網頁
3	Interferon-Gamma 1b	- 慢性肉芽腫疾病	癌瘤及免疫系統藥物	專用藥物	醫管局網頁
4	Eltrombopag	- 慢性免疫性血小板減少性紫癜 - 重度再生障礙性貧血·SAA	營養劑及血液科藥物	安全網藥物 (撒瑪利亞基金)	醫管局網頁
5	Canakinumab	- Cryopyrin相關週期性綜合症·CAPS - 幼年特發性關節炎	癌瘤及免疫系統藥物	安全網藥物 (撒瑪利亞基金) 自費藥物	醫管局網頁
6	Pasireotide	- 庫欣病 / 肢端肥大症	癌瘤及免疫系統藥物	專用藥物	MIMS HK
7	Tobramycin	- 囊腫性纖維化	眼科藥物	通用藥物	MIMS HK
8	Growth Hormone	- 侏儒症	內分泌系統藥物	安全網藥物 (撒瑪利亞基金)	醫管局網頁
9	Aqalsidase Alfa	- 法布瑞氏症	NA	專用藥物	醫管局網頁
10	Aqalsidase Beta	- 法布瑞氏症	NA	專用藥物	醫管局網頁
11	Imiglucerase	- 高雪氏症	NA	專用藥物	醫管局網頁
12	Icatibant	- 遺傳性血管性水腫；HAE	呼吸系統藥物	自費藥物	醫管局網頁
13	Tafamidis	- 遺傳性轉甲狀腺素蛋白類澱粉沉着症造成之心肌病變；ATTR-CM	NA	安全網藥物 (關愛基金)	醫管局網頁
14	Evolocumab	- 同合子家族性高膽固醇血症；HoFH	心臟科藥物	專用藥物	MIMS HK
15	Nintedanib	- 特發性肺纖維化；IPF	呼吸系統藥物	安全網藥物 (撒瑪利亞基金)	醫管局網頁
16	Tocilizumab	- 幼年特發性關節炎 - 全身性幼年特發性關節炎	肌肉骨骼和關節疾病藥物	安全網藥物 (撒瑪利亞基金)	醫管局網頁
17	Laronidase	- 黏多醣症第1型	NA	專用藥物	醫管局網頁
18	Idursulfase	- 黏多醣症第2型	NA	專用藥物	醫管局網頁
19	Elosulfase Alfa	- 黏多醣症第4A型	NA	專用藥物	醫管局網頁
20	Galsulfase	- 黏多醣症第6型	NA	專用藥物	醫管局網頁
21	Adalimumab	- 活性、非感染性、中段 / 後段 / 全葡萄膜炎 - 小兒慢性非感染性前葡萄膜炎 - 幼年特發性關節炎	眼科藥物	安全網藥物 (撒瑪利亞基金)	醫管局網頁
22	Tisagenlecleucel	- 用於治療年齡為 25 歲或以下的復發或難治性 B 細胞急性淋巴瘤母細胞性白血病患者 / 兩種或以上全身系統性療程後復發或難治性的瀰漫性大型 B 細胞淋巴瘤的成年患者	NA	安全網藥物 (關愛基金)	醫管局網頁
23	Risedronate	- 原發性變形性骨炎	內分泌系統藥物	專用藥物	MIMS HK
24	Eculizumab	- 陣發性夜間血紅素尿症：PNH	NA	安全網藥物 (撒瑪利亞基金)	醫管局網頁
25	Alglucosidase Alfa	- 龐貝氏症	NA	專用藥物	醫管局網頁
26	Epoprostenol	- 原發性肺動脈高血壓	心臟科藥物	通用藥物	MIMS HK
27	Ambrisentan	- 原發性肺動脈高血壓	心臟科藥物	專用藥物	MIMS HK
28	Bosentan	- 肺動脈高壓；PAH	心臟科藥物	專用藥物	MIMS HK
29	Iloprost	- 肺動脈高壓；PAH	心臟科藥物	專用藥物	MIMS HK
30	Macitentan	- 肺動脈高壓；PAH	心臟科藥物	專用藥物	MIMS HK
31	Selexipaq	- 肺動脈高壓；PAH	心臟科藥物	專用藥物	MIMS HK
32	Sildenafil citrate	- 肺動脈高壓；PAH	心臟科藥物	專用藥物	MIMS HK
33	Baclofen	- 某些情況引起的肌肉痙攣 (如多發性硬化症、脊髓損傷/疾病)	肌肉骨骼和關節疾病藥物	專用藥物	醫管局網頁
34	Interferon-Beta	- 多發性硬化症；MS	癌瘤及免疫系統藥物	專用藥物	MIMS HK
35	Alemtuzumab	- 復發緩解型多發性硬化症；RRMS	癌瘤及免疫系統藥物	專用藥物	MIMS HK

36	Cladribine	- 復發緩解型多發性硬化症；RRMS	癌瘤及免疫系統藥物	專用藥物	MIMS HK
37	Dimethyl fumarate	- 復發緩解型多發性硬化症；RRMS	癌瘤及免疫系統藥物	專用藥物	MIMS HK
38	Fingolimod	- 復發緩解型多發性硬化症；RRMS	癌瘤及免疫系統藥物	專用藥物	MIMS HK
39	Natalizumab	- 復發緩解型多發性硬化症；RRMS	癌瘤及免疫系統藥物	安全網藥物（撒瑪利亞基金）	醫管局網頁
40	Ofatumumab	- 復發緩解型多發性硬化症；RRMS	癌瘤及免疫系統藥物	自費藥物	醫管局網頁
41	Teriflunomide	- 成人復發緩解型多發性硬化症；RRMS	癌瘤及免疫系統藥物	專用藥物	MIMS HK
42	Siponimod	- 次發進展型多發性硬化症；SPMS	癌瘤及免疫系統藥物	安全網藥物（撒瑪利亞基金）	醫管局網頁
43	Everolimus	- 結節性硬化症相關腎血管平滑肌脂肪瘤；TSC - 與結節性硬化症相關的室管膜下鉅細胞星形細胞瘤；TSC - 輔助治療與結節性硬化症相關的難治性部分性癲癇；TSC	癌瘤及免疫系統藥物	根據不同適應症，此藥品被歸類為專用藥物、安全網藥物（撒瑪利亞基金）及自費藥物。	MIMS HK
44	Rituximab	- 非致命活性及嚴重肉芽腫性血管炎或顯微多發性血管炎	風濕病學藥物	安全網藥物（撒瑪利亞基金）	醫管局網頁
45	Nusinersen	- 嬰兒期發病型脊髓肌肉萎縮症 - 兒童期發病型脊髓肌肉萎縮症 - 尚未出現症狀的脊髓肌肉萎縮症	NA	安全網藥物（關愛基金）	醫管局網頁
46	Risdiplam	- 嬰兒期發病型脊髓肌肉萎縮症 - 兒童期發病型脊髓肌肉萎縮症	NA	安全網藥物（關愛基金）	醫管局網頁
47	Deferiprone (Kelfer)	- 重型地中海貧血症	營養劑及血液科藥物	專用藥物	MIMS HK
48	Dinutuximab Beta	- 治療高風險或復發 / 難治的神經母細胞瘤	NA	安全網藥物（關愛基金）	醫管局網頁
49	Tafamidis Meqlumine	- TTR-家族性澱粉樣多發性神經病變；TTR-FAP	NA	安全網藥物（關愛基金）	醫管局網頁
50	Penicillamine	- 威爾遜氏症	肌肉骨骼和關節疾病藥物	通用藥物	MIMS HK
51	Zinc Acetate	- 威爾遜氏症	營養劑及血液科藥物	通用藥物	FDA

#### 醫院管理局《藥物名冊》分類\*

通用藥物	經證實對病人有關臨床情況適用和有效，並可供一般使用的藥物。公立醫院和診所提供這類藥物時，會收取標準費用。
專用藥物	在特定臨床應用下經專科醫生特別授權使用的藥物。如這類藥物是在特定的臨床應用下處方，公立醫院和診所會收取標準費用。如個別病人在特定臨床應用以外選擇使用專用藥物，便需自行支付藥物的費用。
獲安全網資助的自費購買藥物（自費藥物）	經證實有顯著療效，但超出醫管局一般資助服務範圍所能提供的非常昂貴藥物。這些藥物不屬公立醫院和診所標準收費提供的項目。需要使用這些藥物而有能力負擔費用的病人須自費購買。然而，相關基金會為需要這些藥物而經濟上有困難的病人提供安全網。
不獲安全網資助的自費藥物	僅經初步醫療驗證的藥物、與其他替代藥物相比僅具邊緣效益，但成本明顯昂貴的藥物，以及生活方式藥物(例如減肥藥)。這些藥物並不會包括在標準收費範圍內，病人需自費購買。

\*資料來源：醫管局藥物名冊網頁

## 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	Rufinamide	- Adjunct for seizures associated with the Lennox-Gastaut syndrome · LGS	Central Nervous System	Special Drugs	MIMS HK
2	Eculizumab	- Atypical Hemolytic Uremic Syndrome · aHUS	NA	Safety Net (Community Care Fund)	HA Website
3	Interferon-Gamma 1b	- Chronic Granulomatous Disease	Malignant Disease and Immunosuppression	Special Drugs	HA Website
4	Eltrombopag	- Chronic immune thrombocytopenia - Severe aplastic anaemia · SAA	Nutrition and Blood	Safety Net (Samaritan Fund)	HA Website
5	Canakinumab	- Cryopyrin-associated periodic syndromes · CAPS - Juvenile Idiopathic Arthritis	Malignant disease and immunosuppression	Safety Net (Samaritan Fund) Self-financed item	HA Website
6	Pasireotide	- Cushing's disease / Acromegaly	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
7	Tobramycin	- Cystic Fibrosis	Eye	General Drugs	MIMS HK
8	Growth Hormone	- Dwarfism	Endocrine System	Safety Net (Samaritan Fund)	HA Website
9	Agalsidase Alfa	- Fabry Disease	NA	Special Drugs	HA Website
10	Agalsidase Beta	- Fabry Disease	NA	Special Drugs	HA Website
11	Imiglucerase	- Gaucher's Disease	NA	Special Drugs	HA Website
12	Icatibant	- Hereditary angioedema · HAE	Respiratory System	Safety Net (Samaritan Fund)	HA Website
13	Tafamidis	- Hereditary Transthyretin Amyloidosis with Cardiomyopathy	NA	Safety Net (Community Care Fund)	HA Website
14	Evolocumab	- Homozygous Familial Hypercholesterolemia · HoFH	Cardiovascular System	Special Drugs	MIMS HK
15	Nintedanib	- Idiopathic Pulmonary Fibrosis · IPF	Respiratory System	Safety Net (Samaritan Fund)	HA Website
16	Tocilizumab	- Juvenile Idiopathic Arthritis - Systemic juvenile idiopathic arthritis	Musculoskeletal and Joint Diseases	Safety Net (Samaritan Fund)	HA Website
17	Laronidase	- Mucopolysaccharidosis I	NA	Special Drugs	HA Website
18	Idursulfase	- Mucopolysaccharidosis II	NA	Special Drugs	HA Website
19	Elosulfase Alfa	- Mucopolysaccharidosis IVA	NA	Special Drugs	HA Website
20	Galsulfase	- Mucopolysaccharidosis VI	NA	Special Drugs	HA Website
21	Adalimumab	- Non-infectious intermediate, posterior and panuveitis - Paediatric chronic non-infectious anterior uveitis - Juvenile idiopathic arthritis	Eye	Safety Net (Samaritan Fund)	HA Website
22	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 more lines of systemic therapy	NA	Safety Net (Community Care Fund)	HA Website
23	Risedronate	- Paget's disease of bone	Endocrine System	Special Drugs	MIMS HK
24	Eculizumab	- Paroxysmal nocturnal hemoglobinuria · PNH		Safety Net (Community Care Fund)	HA Website
25	Alglucosidase Alfa	- Pompe Disease	NA	Special Drugs	HA Website
26	Epoprostenol	- Primary Pulmonary Hypertension	Cardiovascular System	General Drugs	MIMS HK
27	Ambrisentan	- Primary Pulmonary Hypertension	Cardiovascular System	Special Drugs	MIMS HK

28	Bosentan	- Pulmonary Arterial Hypertension · PAH	Cardiovascular System	Special Drugs	MIMS HK
29	Iloprost	- Pulmonary Arterial Hypertension · PAH	Cardiovascular System	Special Drugs	MIMS HK
30	Macitentan	- Pulmonary Arterial Hypertension · PAH	Cardiovascular System	Special Drugs	MIMS HK
31	Selexipag	- Pulmonary Arterial Hypertension · PAH	Cardiovascular System	Special Drugs	MIMS HK
32	Sildenafil citrate	- Pulmonary Arterial Hypertension · PAH	Cardiovascular System	Special Drugs	MIMS HK
33	Baclofen	- Muscle spasms caused by certain conditions (such as multiple sclerosis, spinal cord injury/disease)	Musculoskeletal and joint disease	Special Drugs	HA Website
34	Interferon-Beta	- Multiple Sclerosis · MS	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
35	Alemtuzumab	- Relapsing forms of multiple sclerosis · RRMS	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
36	Cladribine	- Relapsing forms of multiple sclerosis · RRMS	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
37	Dimethyl fumarate	- Relapsing forms of multiple sclerosis · RRMS	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
38	Fingolimod	- Relapsing forms of multiple sclerosis · RRMS	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
39	Natalizumab	- Relapsing forms of multiple sclerosis · RRMS	Malignant Disease and Immunosuppression	Safety Net (Samaritan Fund)	HA Website
40	Ofatumumab	- Relapsing forms of multiple sclerosis · RRMS	Malignant Disease and Immunosuppression	Self-financed item	HA Website
41	Teriflunomide	- Relapsing remitting multiple sclerosis in adults · RRMS	Malignant Disease and Immunosuppression	Special Drugs	MIMS HK
42	Siponimod	- Secondary progressive multiple sclerosis · SPMS	Malignant Disease and Immunosuppression	Safety Net (Samaritan Fund)	HA Website
43	Everolimus	- Renal angiomyolipoma associated with tuberous sclerosis complex · TSC - Subependymal giant cell astrocytoma associated with tuberous sclerosis complex · TSC	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
44	Rituximab	- Severe, active non-life-threatening granulomatosis with polyangiitis / microscopic polyangiitis	Malignant Disease and Immunosuppression	Safety Net (Samaritan Fund)	HA Website
45	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
46	Risdiplam	- Infantile Onset Spinal Muscular Atrophy (SMA) - Childhood Onset Spinal Muscular Atrophy (SMA)	NA	Safety Net (Community Care Fund)	HA Website
47	Deferiprone (Kelfer)	- Thalassemia Major	Nutrition and Blood	Special Drugs	MIMS HK
48	Dinutuximab Beta	- Treatment of high-risk or relapse/refractory neuroblastoma	NA	Safety Net (Community Care Fund)	HA Website
49	Tafamidis Meglumine	- TTR-Familial Amyloidotic Polyneuropathy · TTR-FAP	NA	Safety Net (Community Care Fund)	HA Website
50	Penicillamine	- Wilson's Disease	Musculoskeletal and Joint Diseases	General Drugs	MIMS HK
51	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.

\*Reference: HA Drug Formulary Website