

List of Approved Orphan Medicine (updated April 2026)

Product Name	Active Ingredient	Date of <i>Designated Orphan Medicine</i>	Registration Number	Product Holder	Category Listed in the <i>Malaysian Rare Disease List</i>
Biologics					
Enspryng 120 mg solution for injection in pre-filled syringe	Satralizumab	31 May 2021	MAL22046009ACRZ	Roche (Malaysia) Sdn. Bhd.	Neuromyelitis Optica Spectrum Disorder (NMOSD) ICD 10 Code: G36.0
Nexviazyme 100mg Powder for Concentrate for Solution for Infusion	Avalglucosidase Alfa	16 June 2022	MAL23086006AZ	Sanofi-Aventis (Malaysia) Sdn. Bhd.	Pompe disease, GSD type II ICD 10 Code: E74.0
Xenpozyme 20mg Powder For Concentrate For Solution For Infusion	Olipudase Alfa	2 September 2022	MAL23106006AZ	Sanofi-Aventis (Malaysia) Sdn. Bhd.	Niemann Pick Disease A & B ICD 10 Code: E75.2
Zolgensma 2 x 10 ¹³ vg/mL Suspension for Intravenous Infusion	Onasemnogene abeparvovec	2 September 2022	MAL24026006AZ	Novartis Corporation (Malaysia) Sdn. Bhd.	Spinal muscular atrophy ICD 10 Code: G12.0
Spevigo 450 mg Concentrate For Solution For Infusion	Spesolimab	18 January 2023	MAL24106002AZ	Boehringer Ingelheim (Malaysia) Sdn. Bhd.	Generalized pustular psoriasis L40.1
Ultomiris 100mg/mL Concentrate For Solution For Infusion	Ravulizumab	5 July 2023	MAL24096028ACRZ	AstraZeneca Sdn Bhd	Paroxysmal nocturnal haemoglobinuria (PNH) D59.5 Atypical haemolytic uremic syndrome (aHUS) D58.8 Myasthenia gravis G70.0

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Soliris 10mg/ml Concentrate for Solution For Infusion	Eculizumab	6 September 2023	MAL24096027ACZ	AstraZeneca Sdn Bhd	Paroxysmal nocturnal haemoglobinuria (PNH): D59.5 Atypical haemolytic uremic syndrome (aHUS) D58.8 Myasthenia gravis G70.0 Neuromyelitis optica G36.0
Hunterase 2mg/mL Solution for Injection	Idursulfase beta	21 March 2022	MAL25026009AZ	DKSH Malaysia Sdn. Bhd.	Hunter disease E76.1
Strensiq 40 mg/ml Solution for Injection	Asfotase alfa	18 April 2024	MAL25036137ACRZ	AstraZeneca Sdn Bhd	Hypophosphatasia (no. 5.1 with ICD 10 code of E83.39)
Strensiq 100 mg/ml Solution for Injection			MAL25036138ACRZ		
New Chemical Entity (NCE)					
Koselugo Hard Capsules 10 mg	Selumetinib	9 December 2021	MAL23046003ACRZ	AstraZeneca Sdn Bhd	Neurofibromatosis type I ICD 10 Code : Q85.5
Koselugo Hard Capsules 25 mg			MAL23046004ACRZ		

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Rafinlar 50mg Hard Capsule	Dabrafenib mesylate	26 May 2022	MAL24016002ACRZ	Novartis Corporation (Malaysia) Sdn. Bhd.	Advanced melanoma ICD 10 Code: C43.9
Rafinlar 75mg Hard Capsule			MAL24016001ACRZ		
Meqsel 0.5mg Film-Coated Tablet	Trametinib Dimethyl Sulfoxide	26 May 2022	MAL24016005ACRZ	Novartis Corporation (Malaysia) Sdn. Bhd.	Advanced melanoma ICD 10 Code: C43.9
Meqsel 2mg Film-Coated Tablet			MAL24016004ACRZ		
Jinarc Tablets 15 mg	Tolvaptan	2 September 2021	MAL24036007ARZ	Otsuka Pharmaceuticals (Malaysia) Sdn. Bhd.	Autosomal dominant polycystic kidney disease (ADPKD) ICD 10 code: Q61.2
Jinarc Tablets 30 mg			MAL24036006ARZ		
Jinarc 45 mg and Jinarc 15 mg Tablets			MAL24036005ARZ		
Jinarc 60 mg and Jinarc 30 mg Tablets			MAL24036004ARZ		
Jinarc 90 mg and Jinarc 30 mg Tablets			MAL24036003ARZ		
Fabhalta 200mg Hard Capsule	Iptacopan	30 January 2024	MAL25036129ARZ	Novartis Corporation (Malaysia) Sdn. Bhd.	Paroxysmal nocturnal haemoglobinuria (PHN) ICD 10 code: D59.5

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Bylvay 200 micrograms hard capsules	Odevixibat sesquihydrate	17 January 2025	MAL26036005ACRZ	Zuellig Pharma Sdn. Bhd.	Progressive familial intrahepatic cholestasis K76.8 Alagille syndrome Q44.7
Bylvay 400 micrograms hard capsules			MAL26036006ACRZ		
Bylvay 600 micrograms hard capsules			MAL26036007ACRZ		
Bylvay 1200 micrograms hard capsules			MAL26036008ACRZ		