

List of Approved Orphan Medicine (updated March 2024)

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	Olipudase Alfa	2 September 2022	MAL24026006AZ	Novartis Corporation (Malaysia) Sdn. Bhd.	Spinal muscular atrophy ICD 10 Code: G12.0
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		
Rafinlar 50mg Hard Capsule	Dabrafenib mesylate	26 May 2022	MAL24016002ACRZ	Novartis Corporation (Malaysia) Sdn. Bhd.	Advanced melanoma ICD 10 Code: C43.9

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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		