

Medication	Target patient condition	Evidence reviewed	Draft Guidance reviewed by NPMF	Sub-specialty clinician review completed	Final Guidance approved by NPMF
Vedolizumab	Inflammatory Bowel Disorders– unable to access due to adult PBS restrictions	✓	✓	✓	✓
Melatonin	Insomnia treated by General Paediatricians & Sleep Medicine with low levels of evidence for use	✓	✓	✓	✓
Fenfluramine	Genetic Epilepsy disorders that are resistant to other treatments	✓	✓	✓	✓
GLP-1 Analogues	Diabetic & obese patients that aren't being controlled with current therapies	✓	✓	✓	✓
Palivizumab	Prevention of RSV in neonates and infants	✓	✓	Pending	
Miglustat	Niemann Pick-C Genetic disease with limited treatment options	✓	✓	✓	✓
Romiplostim & Eltrombopag	Chronic thrombocytopenias – previously unable to access due to adult PBS restrictions	✓	✓	✓	Pending
Trametinib	Genetic tumour diseases refractory to usual treatment	✓	✓	Pending	
Ranibizumab	Retinopathy of Prematurity in neonates	✓	✓	Pending	
Burosumab	Genetic hypophosphatemia	✓	✓	Pending	
Canakinumab	Severe autoimmune disorder (CAPS) refractory to other immunosuppressant's	✓	✓	Pending	
Dupilumab (off-label)	Eosinophilic Oesophagitis	✓	✓	Pending	
Dinutuximab	Neuroblastoma patients	✓	✓		Pending
Gene Therapy	Spinal Muscular Atrophy	✓	✓		Pending