

Specialist Advisory Committees

Objective advice to Pharmac

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Rare Disorders Advisory Committee Provisional recommendations 10 June 2025

Pharmac is piloting a new approach which will allow people to understand the advice we get within 30 business days of advisory committee meetings. More information about provisional recommendations is on the Pharmac website here: <https://www.pharmac.govt.nz/about/expert-advice/pharmacology-and-therapeutics-advisory-committee-ptac>

We are releasing provisional recommendations only at this time, which share the high-level outcome of the advice recommendation. Further information, which will explain the recommendation, will be shared when the full meeting record is completed. Applicants / suppliers are given the opportunity to withhold the provisional recommendation until more information is available.

Summary of Provisional Recommendations

Pharmaceutical and Indication	Provisional Recommendation
Pegunigalsidase alfa-iwxy , (branded as Elfabrio) as enzyme replacement treatment of Fabry Disease, within the context of treatments for rare disorders, subject to Special Authority criteria	Withheld*
Onasemnogene abeparvovec for the treatment of spinal muscular atrophy (SMA), pre-symptomatic or type 1, within the context for rare disorders, subject to Special Authority criteria	High priority
Garadacimab for the treatment of hereditary angioedema, within the context of treatments for rare disorders, subject to Special Authority criteria	High priority
Iptacopan for the treatment of paroxysmal nocturnal haemoglobinuria (PNH), within the context of treatments for rare disorders, subject to Special Authority criteria	High priority
Eculizumab for the treatment of paroxysmal nocturnal haemoglobinuria (PNH), within the context of treatments for rare disorders, subject to Special Authority criteria	High priority
Crovalimab for the treatment of paroxysmal nocturnal haemoglobinuria (PNH), within the context of treatments for rare disorders, subject to Special Authority criteria	High priority

*Withheld provisional recommendation – the applicant / supplier has requested the recommendation not be released until full record is available.

More information about the agenda items considered at the recent Rare Disorders Advisory Committee meeting can be found on the Pharmac website here: <https://www.pharmac.govt.nz/news-and-resources/news/agenda-for-june-2025-rare-disorders-advisory-committee-meeting>