5.22 EFGARTIGIMOD ALFA, Solution for subcutaneous injection 1000 mg in 5.6 mL, Vyvgart®, Argenx Australia Pty. Ltd

1 Purpose of Submission

1.1 The Category 4 submission requested to list efgartigimod alfa (Vyvgart®) 1000 mg/5.6 mL solution for subcutaneous injection (EFG SC) as an add-on to standard therapy for the treatment of adult patients with generalised myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody positive, under the same circumstances as recommended for efgartigimod alfa (Vyvgart®) 400 mg/20 mL solution concentrate for intravenous infusion (EFG IV).

1.2 Listing was requested on a cost-minimisation basis versus EFG IV.

2 Background

2.1 At the time of the November 2025 PBAC meeting, EFG IV vial for infusion was not listed on the PBS. It was recommended at the March 2025 PBAC Meeting as a Section 100 (Highly Specialised Drug Program) Authority Required (Written/Electronic) listing for the treatment of adult patients with gMG who are anti-acetylcholine receptor (AChR) antibody positive.

Registration status

2.2 Efgartigimod alfa was TGA registered on 24 February 2025 as a 'add-on to standard therapy for the treatment of adult patients with gMG who are anti-acetylcholine receptor (AChR) antibody positive'.

2.3 Two forms of efgartigimod were registered concurrently by the TGA: 400 mg/20 mL concentrated solution for intravenous infusion vial and 1000 mg/5.6 mL solution for injection vial (subcutaneous injection).

2.4 The TGA Delegate agreed with the European Medicines Agency that the SC and IV forms are therapeutically equivalent.[1]

1 https://www.ema.europa.eu/en/documents/variation-report/vyvgart-h-c-005849-x-0003-epar-assessment-report_en.pdf

Previous PBAC consideration

2.5 EFG IV 400 mg/20 mL was recommended for the treatment of adult patients with gMG who are AChR antibody positive by the PBAC at its March 2025 meeting.

2.6 The PBAC considered that EFG IV "should substitute for IVIg and PLEX rather than be added on to or used in combination with these modalities." (Paragraph 8.14, efgartigimod alfa Public Summary Document [PSD], March 2025 PBAC Meeting).

2.7 EFG SC 1000 mg/5.6 mL has not been previously considered by the PBAC.

3 Requested listing

3.1 The submission requested a Section 100 Highly Specialised Drug (S100 HSD) listing for the initial treatment phase and a General Schedule, Authority Required listing for the Continuing phase. A S100 HSD listing means the prescriber must be affiliated with a private or public hospital entity in order to satisfy the authority requirements.

3.2 The Secretariat noted that at its March 2025 meeting, the PBAC recommended the subcutaneous injection zilucoplan for gMG be listed on the S100 HSD schedule for both initial and continuing phases of treatment.

3.3 The submission could not provide the full requested restriction as the listing was still under negotiation at the time. The Secretariat advised that the restriction for EFG SC should provide sufficient quantity for 4 doses per cycle.

3.4 The submission requested the following new listing: suggested deletions are in strikethrough.

Name, restriction, manner of administration and form	Maximum quantity (packs)	Maximum quantity (units)	No. of repeats	Dispensed price for maximum quantity		Proprietary name and manufacturer
				Published	Effective	
Efgartigimod alfa, 1000 mg of efgartigimod alfa in 5.6 mL (180 mg/mL)	4 ~~vials~~	4 ~~vials~~	2	$[redacted] (Public hospital)	$[redacted] (Public hospital)	VYVGART Argenx SC

3.5 The submission requested a Special Pricing Arrangement (SPA) with an effective approved ex-manufacturer price (AEMP) per vial of $[redacted], and an effective dispensed price for maximum quantity (DPMQ) of $[redacted] for Public Hospitals under S100 HSD. The requested published AEMP per vial was $[redacted], with a published DPMQ of $[redacted].

3.6 The submission based the maximum quantity and number of repeats on the recommended dosage and administration in the Product Information. Patients will have once-weekly injections of one vial, for four weeks, followed by a four-week period of no treatment. For a 6-monthly script, 12 vials should be sufficient, with the recommended dose being the full 1000 mg.

3.7 The submission did not request a grandfathering restriction. In its pre-PBAC response, the Sponsor advised that it was supportive of a grandfathering restriction, should it

allow patients who start on the EFG IV access program to be able to access EFG SC. The EFG IV restrictions allow for this transition.

4 Comparator

4.1 The submission nominated EFG IV (Vyvgart®) 400 mg/20 mL (20 mg/mL) as the comparator.

4.2 The submission did not consider other near-market comparators. The PBAC advised in March 2025 that EFG IV, zilucoplan, ravulizumab and rozanolixizumab "should be considered as non-inferior with each other and with IVIg." (Paragraph 8.1, efgartigimod alfa PSD, March 2025 PBAC Meeting).

4.3 The PBAC could only recommend listing EFG SC at a higher price than the alternative therapy or therapies if it was satisfied that it provides, for some patients, a significant improvement in efficacy or reduction of toxicity over the alternative therapy or therapies (*National Health Act 1953*, Section 101(3B)). The alternative therapies in this case should include EFG IV, zilucoplan, ravulizumab and rozanolixizumab. Since there was no data to establish superiority over the comparators, there was no justification for the price of EFG SC to be higher than EFG IV, zilucoplan, ravulizumab or rozanolixizumab.

4.4 The PBAC advised that EFG IV, zilucoplan, ravulizumab and rozanolixizumab were appropriate as comparators, while noting that these drugs have not yet been listed on the PBS. The PBAC also advised that IVIg is an appropriate comparator as it was for the four gMG medicines recommended in March 2025.

5 Consideration of the evidence

Sponsor hearing

5.1 There was no hearing for this item.

Consumer inputs

5.2 The PBAC noted and welcomed the input from individuals (10), health care professionals (1) and organisations (1) via the Consumer Comments facility on the PBS website. The comments described a range of benefits of treatment with EFG SC, including improved access to medicines for gMG, fewer side effects, and a marked increase in quality of life. Individual comments described the impact of gMG as "debilitating," with everyday tasks becoming increasingly challenging, and lifestyle being restricted. Individuals also described the ability to self-administer EFG SC weekly as providing "more freedom" from hospital-administered infusions or a strict daily pill regimen.

5.3 The PBAC noted that the Myasthenia Alliance Australia (MAA) supported the equitable and timely access to EFG SC as a safe, effective, and flexible treatment option that can improve quality of life, reduce hospital visits, and empower patients with greater

independence and workforce participation. MAA also highlighted that people living with gMG in Australia face the significant and ongoing burden of fluctuating symptoms, intrusive treatments, and treatment-related side effects.

Clinical trials

5.4 The submission was based on the ADAPT-SC study, a randomised, directly comparative open-label, parallel group study that included a 4-week (1 cycle) comparative EFG SC vs. IV treatment period (plus 7-weeks follow up). In total N=55 patients were analysed in each of the EFG treatment arms (N=110 in total).

Table 1: Studies and associated reports presented in the submission

Trial ID	Protocol title/ Publication title	Publication citation
Direct randomised trials		
ADAPT-SC/ ADAPT-SC+ NCT04735432	ADAPT-SC Howard Jr et al Subcutaneous efgartigimod PH20 in generalized myasthenia gravis: A phase 3 randomized noninferiority study (ADAPT-SC) and interim analyses of a long-term open-label extension study (ADAPT-SC+)	July 2021 *Lancet Neurol* 2021; 20(7): 526-536

Source: pp24-25 of the submission.

5.5 The submission's request was based on the comparison between the eligibility criteria and outcomes of the ADAPT-SC the ADAPT-IV trials.

Comparative effectiveness

5.6 The primary outcome least square (LS) mean change from baseline in total IgG level at day 29 was −66.4% (95% CI −68.91, −63.86) in the EFG SC arm and −62.2% (95% CI −64.67, −59.72) in the EFG IV arm. The corresponding LS mean difference in the percent change from baseline in total IgG levels at day 29 between the 2 arms in the mITT population (EFG SC vs EFG IV) was −4.2% (95% CI: −7.73 to −0.66; p<0.0001) in favour of EFG SC. The submission noted that the upper limit of the 95% CI (−0.66%) was markedly below the prespecified NI margin of 10%, being less than 0%, and it stated that treatment non-inferiority was clearly established in all populations.

5.7 The clinical efficacy of EFG SC was assessed using validated clinical outcome scales, including the participant-reported MG-ADL scale and the physician-assessed QMG scale. The submission considered that although the study was not powered for clinical efficacy, EFG SC showed similar clinical efficacy results to EFG IV.

Comparative harms

5.8 The submission stated that an overall tolerable safety profile was shown with EFG SC. Injection site reactions contributed to the higher frequency of treatment-related and procedure-related AEs in the EFG SC arm compared with the EFG IV arm. All the injection site reactions were grade 1 or 2 severities. None of the events were serious or led to EFG SC or IV discontinuation.

Clinical claim

5.9 The submission claimed non-inferior comparative effectiveness and non-inferior comparative safety of EFG SC compared with EFG IV.

5.10 The TGA and EMA have found EFG SC and EFG IV to be therapeutically equivalent.

Economic analysis

5.11 The submission presented a cost-minimisation approach of EFG SC compared with EFG IV. The cost minimisation was set over the course of one year of treatment and used the same average number of cycles as recommended in March 2025, 4.72 cycles per year. Both EFG SC and EFG IV are administered in a cycle of 4 weeks of treatment, followed by 4 weeks of no treatment. The claim of equi-effectiveness is based on the clinical conclusions of the trial presented in the submission (ADAPT-SC).

5.12 The proposed equi-effective dose is one vial of EFG SC 1000 mg/5.6 mL = 2.4 vials of EFG IV 400 mg/20 mL infusion. This equated to an annual dose of EFG SC of 18,880 mg per patient per year.

Table 2: Proposed Cost-Minimisation of EFG SC to EFG IV

	PBAC March 2025 (EFG IV)			Proposed (EFG SC)			
	Annually	**MBS cost**			**Annually**	**MBS cost**	
Drug Costs:							
AEMP per vial [a]			$[redacted]	AEMP per PFS			$[redacted]
Cycles	4.72			Cycles	4.72		
Infusions	18.88			Injections	18.88		
Vials per infusion	2.40			PFS per injection	1		
Vials per cycle (i.e. per Rx)	9.62			Injections per cycle (i.e. per Rx)	4		
Vials p.a.	45.4						
Annual Drug cost			$[redacted]				$[redacted]
Drug Administration cost:							
MBS 14245 (@85%)	18.88	$97.20	$1,835.14				
MBS 116 (@85%)	2	$76.00	$152.00	MBS 116 (@85%)	6 [b]	$76.00	$456.00
Annual drug administration cost			$1,987.14				$456.00
Total Annual Cost			$[redacted]				$[redacted]

Source: Table ES.3 Cost Minimisation of the submission Main Body. Abbreviations: AEMP = approved ex-manufacturer price; EFG IV = efgartigimod alfa vial for infusion; EFG SC = efgartigimod alfa pre-filled syringe for subcutaneous injection; MBS = Medicare benefits schedule; PFS = pre-filled syringe; Rx = prescription.

This cost-minimisation is based on effective prices before the application of an SPA.

a. This AEMP is a placeholder and not the final agreed AEMP for EFG IV.

b. The sponsor assumed there would be 6 visits to a specialist annually, 5 for the administration of the first 5 doses of EFG SC and training of the patient or carer in at-home administration and one follow up visit.

5.13 The EFG IV dose is the same as the average dose recommended by the PBAC in March 2025. This is because EFG IV is dose on a mg per kg basis, whereas the EFG SC is dosed as 1000 mg per patient.

5.14 The cost minimisation used a proxy effective ex-manufacturer price of $[redacted] per EFG IV vial. This is not an agreed price.

5.15 For administration costs for EFG SC, the sponsor assumed there would be 6 visits to a specialist annually, 5 for the administration of the first 5 doses and training of the patient or carer in at-home administration and one follow up visit. The sponsor assumed that all patients would move to at home administration by either themselves or a carer after the fifth dose with a physician. The submission provided data from the clinical study which showed that 76.4% (42/55) patients were considered adequately trained for self-administration after four or less training visits. However, the remaining 23.6% were not considered adequately trained for self-administration after 9 training visits.

5.16 The PBAC had previously recommended the listing of EFG IV, zilucoplan, ravulizumab and rozanolixizumab for the treatment of gMG on the basis of a cost-comparison versus IVIg, supported by a cost-per-responder analysis versus placebo. The cost-comparison was based on the drug cost per patient per year accounting for a total average annual IVIg dose of 541.1 grams with a premium to account for the administration benefits associated with the newer therapies compared with IVIg. (Paragraph 8.24, efgartigimod alfa PSD, March 2025 PBAC meeting).

Drug cost/patient/year: $[redacted]

5.17 The estimated drug cost of EFG SC per patient per year would be $[redacted], based on 18.88 required injections (reflecting the average of 18.88 infusions for the comparator EFG IV).

Estimated PBS usage and financial implications

5.18 The submission presented a market share approach to estimate the utilisation and financial impacts associated with the PBS listing of EFG SC. The submission assumed that a proportion of patients on EFG IV will transition to EFG SC, applying an uptake rate in the financial estimates of [redacted]% in Year 1, [redacted]% in Year 2 and [redacted]% in the remaining Years 3 through 6.

5.19 The requested price was based on the AEMP of EFG IV used in March 2025 PBAC submission. This price has not been agreed for the listing of EFG IV.

5.20 Table 3 presents the estimated extent of use, cost of EFG SC to the PBS/RPBS and the net financial implications to the PBS/RPBS and MBS. The financial impact to Services Australia will be determined by that agency as part of the post PBAC process.

5.21 The submission estimated that 10,000 to < 20,000 scripts of EFG SC would be supplied over the first 6 years of listing (500 to < 5,000 in Year 1 to 500 to < 5,000 in Year 6).

5.22 The submission stated that the estimated net financial impact to the PBS/RPBS for the listing of EFG SC is $10 million to < $20 million over 6 years (Year 1 $0 to < $10 million to Year 6 $0 to < $10 million).

Table 3: Estimated use and financial implications

	Year 1	Year 2	Year 3	Year 4	Year 5	Year 6
Estimated extent of use						
Number of scripts dispensed[a]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]	[redacted][1]
Estimated financial implications of EFG SC						
Cost to PBS/RPBS less co-payment	$[redacted][2]	$[redacted][3]	$[redacted][3]	$[redacted][3]	$[redacted][4]	$[redacted][4]
Estimated financial implications of EFG IV						
Cost to PBS/RPBS less co-payment	-$[redacted][2]	-$[redacted][3]	-$[redacted][3]	-$[redacted][3]	-$[redacted][4]	-$[redacted][4]
Net financial implications						
Net cost to PBS/RPBS	$[redacted][5]	$[redacted][5]	$[redacted][5]	$[redacted][5]	$[redacted][5]	$[redacted][5]
Net cost to MBS	-$[redacted][5]	-$[redacted][5]	-$[redacted][5]	-$[redacted][5]	-$[redacted][5]	-$[redacted][5]
Net cost to Government	$[redacted][5]	$[redacted][5]	$[redacted][5]	$[redacted][5]	$[redacted][5]	$[redacted][5]

[a] Assuming 4.72 scripts per patient per year as estimated by the submission.
Abbreviations: MBS = Medical Benefits Scheme; PBS = Pharmaceutical Benefits Scheme; RPBS = Repatriation Pharmaceutical Benefits Scheme.
Source: UCM workbook "EFG Section 4-Base Case-Nov2025-3Oct.xlsx", and Tables 4-12 p96 and 4-13 p97 of the submission.
The redacted values correspond to the following ranges:
[1] 500 to < 5,000
[2] $100 million to < $200 million
[3] $200 million to < $300 million
[4] $300 million to < $400 million
[5] $0 to < $10 million

5.23 When the cost savings from the reduction of MBS 14245 services was included, the cost to the MBS over 6 years was $0 to < $10 million ($0 to < $10 million in Year 1, increasing to $0 to < $10 million in Year 6).

5.24 The estimates had assumed a script equivalence for EFG SC to EFG IV of 1:1. At the time of the PBAC consideration, the restriction criteria of EFG IV were under negotiation.

5.25 The estimates used MBS 14245 for the infusion of EFG IV with an assumption of four administrations per dispensing script. However, the estimates submitted to PBAC for EFG IV at March 2025 used only MBS 116. MBS item 14245 is not appropriate for use with the infusion of EFG IV (being inconsistent with the duration of infusion stated in the PI).

5.26 The estimates assumed EFG IV is the only available drug for gMG, however zilucoplan, ravulizumab and rozanolixizumab were also recommended at the March 2025 PBAC meeting. Thus, the estimates did not account for patients who may be treated with EFG SC rather than the other gMG medicines, should they also be listed.

5.27 The Secretariat noted that the submission used 500 to < 5,000 gMG patients in year 6 and a [redacted]% growth rate to back-calculate the patients and consequently the script volumes for EFG IV. This method is inconsistent with the PBAC recommendation made at its March 2025 meeting for EFG IV. This inconsistency has led to an overestimation of the treatable cohort, and subsequently the overall script volume for the estimated EFG IV market.

Financial Management – Risk Sharing Arrangements

5.28 At its March 2025 meeting, the PBAC advised "that a single risk sharing arrangement (RSA) that includes all of the new therapies (in all settings) would be required to mitigate the risk of use outside the intended restriction," and that the "Department and each sponsor should work to ensure the cost per patient does not exceed the estimates in the cost-comparison and the financials" (paragraph 8.33, EFG IV PSD, March 2025 PBAC meeting).

5.29 The submission did not explicitly propose inclusion of EFG SC in this RSA.

5.30 The PBAC noted that EFG SC should be included in the same RSA as advised in March 2025, due to risk of use outside the eligible PBS population.

6 PBAC Outcome

6.1 The PBAC recommended the listing of EFG SC for the treatment of gMG under the same circumstances as EFG IV based on a cost comparison to IVIg, as per EFG IV which was recommended in March 2025. The PBAC advised that a general schedule (s85) listing would be inappropriate and recommended that EFG SC be listed as a Section 100 (Highly Specialised Drug) Authority Required (Written/Online) listing, consistent with its recommendation for EFG IV and other novel gMG medicines.

6.2 The PBAC considered that the clinical claim of non-inferior effectiveness and non-inferior safety of EFG SC versus EFG IV was reasonable, noting the clinical data provided, along with the opinion of the TGA and EMA that the SC and IV forms are therapeutically equivalent.

6.3 While the submission had presented a cost-minimisation approach (CMA) compared with EFG IV, the PBAC did not consider that this was a reasonable basis for listing EFG SC. In particular, the PBAC did not accept the reduced use of MBS items as an acceptable cost offset as part of the CMA, as it was unclear that the savings would be realised in practice. And although the PBAC agreed that EFG IV was a relevant comparator, it considered that EFG SC could also be used in place of IVIg, zilucoplan, ravulizumab or rozanolixizumab. The PBAC considered that there was insufficient evidence to support superior efficacy or safety of any of these newly recommended agents (including EFG SC) over IVIg, hence PBAC advised that the subcutaneous form be priced as per the other gMG medicines recommended at the March 2025 PBAC meeting. This comprised a cost-comparison to IVIg (average annual dose per year of 541.1g) with a price premium for the administration benefits of IVIg (refer to para 8.24, efgartigimod alfa PSD, March 2025 PBAC meeting). For EFG SC, noting the submission's proposed equivalence of 1 SC vial to 2.4 vials of EFG IV, the PBAC advised that the cost comparison should incorporate an annual dose EFG SC of 18,880 mg per year (or simply put, the 1 vial EFC SC should be priced equivalent to 2.4 vials of EFG IV).

6.4 The PBAC advised that the financial estimates were uncertain owing to the fact that no gMG medicine is currently PBS listed, and that the estimates omitted zilucoplan, ravulizumab and rozanolixizumab. Each of zilucoplan, ravulizumab and rozanolixizumab may be replaced in practice by EFG SC. The PBAC also advised that MBS item 14245 was not appropriate to be included in the estimates, and consistent with its advice regarding the economic analysis, it was unclear that savings to the MBS would be realised in practice.

6.5 The PBAC advised that the efgartigimod alfa subcutaneous form should also enter the Risk Sharing Arrangement for the gMG medicines as recommended in March 2025, to mitigate the same risks in terms of use outside the intended restriction and the increase in frequency of cycles over time.

6.6 The PBAC advised that EFG SC is suitable for prescribing by medical practitioners only, consistent with its advice for EFG IV.

6.7 The PBAC noted that the criteria prescribed by the *National Health (Pharmaceuticals and Vaccines – Cost Recovery) Regulations 2022* for Pricing Pathway A were not met in its recommendation of EFG SC, as EFG SC is not expected to provide a substantial and clinically relevant improvement in efficacy, or reduction of toxicity, over IVIg, and is not expected to address a high and urgent unmet clinical need because an alternative therapy (IVIg) is available.

6.8 The PBAC noted that this submission is not eligible for an Independent Review as it received a positive recommendation.

Outcome:
Recommended

7 Recommended listing

7.1 Add new SC form (shown in *italics*) as follows:

Acute severe patients

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
EFGARTIGIMOD ALFA					
efgartigimod alfa 1000 mg in 5.6 mL vial	*NEW (HSD Public)* *NEW (HSD Private)*	*4*	*4*	*1*	*Vyvgart SC*
efgartigimod alfa 400mg/20mL vial	NEW (HSD Public) NEW (HSD Private)	12	12	1	Vyvgart
Category / Program: ☒Section 100 – Highly Specialised Drugs Program – Public (Code HB) / Private (Code HS)					
Prescriber type: ☒Medical Practitioners					
Restriction type: ☒Authority Required (Written/Online - Immediate assessment)					
Authority type: ☒ Complex Authority Required (CAR)					
Administrative Advice: Definitions for the purposes of administering this restriction. Where the term **'gMG biological agent'** is referenced in this restriction, it refers to efgartigimod alfa, ravulizumab, rozanolixizumab, and zilucoplan. The following are settings and time limits where a gMG biological agent are PBS subsidised: (1) 3 months of acute treatment - 'acute severe gMG' (2) 6 months of bridging therapy - 'bridging therapy for gMG' (3) Continuous therapy - 'treatment refractory gMG' A patient may transition sequentially from one setting to another where all criteria are met e.g. (1) to (2) to (3), but cannot return to an earlier treatment setting. **Definitions:** A non-steroidal immunosuppressant (NS-IST) is one of the following: (i) azathioprine, (ii) ciclosporin, (iii) cyclophosphamide, (iv) methotrexate, (v) mycophenolate (vi) tacrolimus The Myasthenia Gravis Foundation of America (**MGFA**) Disease Classification can be accessed at https://myasthenia.org/ The Myasthenia Gravis Composite (**MGC**) scoring profile can be accessed at https://www.criteria.blood.gov.au/NeurologicalScales#MGC The Myasthenia Gravis-Activities of Daily Living (**MG-ADL**) scoring profile can be accessed at https://myasthenia.org/					

Administrative Advice: **Treatment switching:** Switching between gMG biological agents is permitted. Treatment switching should be limited to when a patient moves between different treatment settings, i.e. moving from 'acute severe gMG' to 'bridging therapy for gMG' [(1) to (2)]; or moving from 'bridging therapy for gMG' to 'treatment refractory gMG' [(2) to (3)]. In the acute severe (1) or bridging therapy (2) settings a patient may switch to an alternate gMG biological agent if they are experiencing an intolerance or toxicity necessitating treatment withdrawal. Patients can switch by requalifying through the relevant restrictions. Only a balance of the time limits specified within the relevant treatment phase will be approved i.e. the remaining of the 3 months (acute severe gMG); or 6 months (bridging therapy for gMG) after accounting for the treatment of the previous gMG biologic. In the treatment refractory setting (3), a patient may switch to an alternate gMG biological agent via either: a) the initial restriction if they have trialled a different gMG biologic, but did not respond to treatment; or b) the continuing restriction if they have trialled a different gMG biological agent and have responded to treatment. Mark any remaining unused repeat prescriptions as "cancelled".
Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday). Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au Applications for authorisation under this restriction should be made using the Online PBS Authorities system (see www.servicesaustralia.gov.au/hpos) Alternatively, applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos Or mailed to: Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.
Administrative Advice: No increase in the maximum number of repeats may be authorised.
Administrative Advice: Special Pricing Arrangements apply.
Restriction Summary [new1] / Treatment of Concept: [new1A]
Indication: Acute severe generalised myasthenia gravis (gMG)
Treatment Phase: [Blank]
Clinical criteria:
Patient must have a diagnosis of MGFA Disease Class II to IV
AND
Clinical criteria:
Patient must have a positive serology for acetylcholine receptor (AChR) binding autoantibodies
AND
Clinical criteria:
Patient must not be experiencing a myasthenic crisis
AND
Clinical criteria:
Patient must be considered by the treating clinician to have rapidly deteriorating gMG disease in the absence of immediate treatment with a gMG biological agent
AND
Clinical criteria:

Patient must not be receiving concomitant treatment with any of the following: (i) another gMG biological agent, (ii) intravenous immunoglobulin (IVIg), (iii) plasma exchange (PLEX), (iv) rituximab for this condition.
AND
Clinical criteria:
Patient must be receiving concomitant treatment with a non-steroidal immunosuppressant (NS-IST); or
Patient must be commencing treatment with an NS-IST within 2 weeks; or
Patient must have had a thymectomy
AND
Clinical criteria:
Patient must be receiving concomitant treatment with an oral corticosteroid
AND
Clinical criteria:
Patient must not receive more than 3 months total of treatment with gMG biological agents under this PBS indication (e.g. for 'acute severe gMG')
AND
Clinical criteria:
Patient must not have accessed a prior PBS-subsidised gMG biological agent; or
Patient must have developed an intolerance/toxicity to a PBS-subsidised gMG biological agent necessitating a change in treatment
Treatment criteria:
Must be treated by a prescriber who is either: (i) a neurologist; (ii) a clinical immunologist with expertise in the treatment of myasthenia gravis; or
Must be treated by a medical practitioner who has consulted at least one of the above mentioned specialist types, with an agreement reached that the patient should be treated with this pharmaceutical benefit on this occasion
Prescribing Instructions: The authority application must be via the Online PBS Authorities System or in writing via HPOS form upload or mail. If the application is submitted through HPOS form upload or mail, it must include the following: (1) details of the proposed prescription (2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice)

Bridging therapy

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
EFGARTIGIMOD ALFA					
efgartigimod alfa 1000 mg in 5.6 mL vial	*NEW (HSD Public)* *NEW (HSD Private)*	*4*	*4*	*3*	*Vyvgart SC*
efgartigimod alfa 400mg/20mL vial	NEW (HSD Public) NEW (HSD Private)	12	12	3	Vyvgart
Category / Program: ☒ Section 100 – Highly Specialised Drugs Program – Public (Code HB) / Private (Code HS)					
Prescriber type: ☒Medical Practitioners					
Restriction type: ☒Authority Required (Written/Online - Immediate assessment)					
Authority type: ☒ Complex Authority Required (CAR)					

Administrative Advice:
Definitions for the purposes of administering this restriction.

Where the term **'gMG biological agent'** is referenced in this restriction, it refers to efgartigimod alfa, ravulizumab, rozanolixizumab, and zilucoplan.

The following are settings where a gMG biological agent are PBS subsidised:
(1) 3 months of acute treatment - 'acute severe gMG'
(2) 6 months of bridging therapy - 'bridging therapy for gMG'
(3) Continuous therapy - 'treatment refractory gMG'
A patient may transition sequentially from one setting to another where all criteria are met e.g. (1) to (2) to (3), but cannot return to an earlier treatment setting.

Definitions:
A non-steroidal immunosuppressant (NS-IST) is one of the following:
(i) azathioprine,
(ii) ciclosporin,
(iii) cyclophosphamide,
(iv) methotrexate,
(v) mycophenolate
(vi) tacrolimus

The Myasthenia Gravis Foundation of America (**MGFA**) Disease Classification can be accessed at https://myasthenia.org/

The Myasthenia Gravis Composite (**MGC**) scoring profile can be accessed at https://www.criteria.blood.gov.au/NeurologicalScales#MGC

The Myasthenia Gravis-Activities of Daily Living (**MG-ADL**) scoring profile can be accessed at https://myasthenia.org/

Administrative Advice:
Treatment switching:
Switching between gMG biological agents is permitted. Treatment switching should be limited to when a patient moves between different treatment settings, i.e. moving from 'acute severe gMG' to 'bridging therapy for gMG' [(1) to (2)]; or moving from 'bridging therapy for gMG' to 'treatment refractory gMG' [(2) to (3)].

In the acute severe (1) or bridging therapy (2) settings a patient may switch to an alternate gMG biological agent if they are experiencing an intolerance or toxicity necessitating treatment withdrawal. Patients can switch by requalifying through the relevant restrictions. Only a balance of the time limits specified within the relevant treatment phase will be approved i.e. the remaining of the 3 months (acute severe gMG); or 6 months (bridging therapy for gMG) after accounting for the treatment of the previous gMG biologic.

In the treatment refractory setting (3), a patient may switch to an alternate gMG biological agent via either:
a) the initial restriction if they have trialled a different gMG biologic, but did not respond to treatment; or
b) the continuing restriction if they have trialled a different gMG biological agent and have responded to treatment.
Mark any remaining unused repeat prescriptions as "cancelled".

Administrative Advice:
Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday).
Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au
Applications for authorisation under this restriction should be made using the Online PBS Authorities system (see www.servicesaustralia.gov.au/hpos)
Alternatively, applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos

Or mailed to: Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.
Administrative Advice: No increase in the maximum number of repeats may be authorised.
Administrative Advice: Special Pricing Arrangements apply.
Restriction Summary [new2] / Treatment of Concept: [new2A]
Indication: Bridging therapy for generalised myasthenia gravis (gMG)
Treatment Phase: [Blank]
Clinical criteria:
Patient must have a diagnosis of MGFA Disease Class II to IV
AND
Clinical criteria:
Patient must have a positive serology for acetylcholine receptor (AChR) binding autoantibodies
AND
Clinical criteria:
Patient must not be experiencing a myasthenic crisis
AND
Clinical criteria:
Patient must be receiving concomitant treatment with a non-steroidal immunosuppressant (NS-IST); or
Patient must have had a thymectomy
AND
Clinical criteria:
Patient must be receiving concomitant treatment with an oral corticosteroid
AND
Clinical criteria:
Patient must have a MG-ADL score of ≥ 6 and a MGC score of ≥ 10, despite having undergone 2 of the following 3 remission inducing treatments: (i) NS-IST for 3 months; (ii) oral corticosteroids for 3 months; (iii) a thymectomy
AND
Clinical criteria:
Patient must not be receiving concomitant treatment with any of the following: (i) another gMG biological agent, (ii) intravenous immunoglobulin (IVIg), (iii) plasma exchange (PLEX), (iv) rituximab for this condition
AND
Clinical criteria:
Patient must not receive more than 6 months of bridging treatment with any gMG biological agent under this PBS indication
Treatment criteria:
Must be treated by a prescriber who is either: (i) a neurologist; (ii) a clinical immunologist with expertise in the treatment of myasthenia gravis; or
Must be treated by a medical practitioner who has consulted at least one of the above mentioned specialist types, with an agreement reached that the patient should be treated with this pharmaceutical benefit on this occasion

Prescribing Instructions: The authority application must be via the Online PBS Authorities System or in writing and must include: (a) the MG-ADL and MGC score after 3-months of remission-inducing treatments (include the date the assessments were conducted) (b) details of remission-inducing treatments [date commencement and duration of drug therapy including drug names and dosages, and/or date of the thymectomy] If the application is submitted through HPOS form upload or mail, it must include the following: (1) details of the proposed prescription (2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice)
Prescribing Instruction: A retrospective assessment for one of the MGC score or MG-ADL score can be accepted in cases where it was not conducted after completing 3 months of remission inducing treatments.

Treatment Refractory patients

MEDICINAL PRODUCT medicinal product pack	PBS item code	Max. qty packs	Max. qty units	№.of Rpts	Available brands
EFGARTIGIMOD ALFA					
efgartigimod alfa 1000 mg in 5.6 mL vial	*NEW (HSD Public)* *NEW (HSD Private)*	*4*	*4*	*3*	*Vyvgart SC*
efgartigimod alfa 400mg/20mL vial	NEW (HSD Public) NEW (HSD Private)	12	12	3	Vyvgart
Category / Program: ☒ Section 100 – Highly Specialised Drugs Program – Public (Code HB) / Private (Code HS)					
Prescriber type: ☒Medical Practitioners					
Restriction type: ☒Authority Required (Written/Online - Immediate assessment)					
Authority type: ☒ Complex Authority Required (CAR)					
Administrative Advice: Definitions for the purposes of administering this restriction. Where the term **'gMG biological agent'** is referenced in this restriction, it refers to efgartigimod alfa, ravulizumab, rozanolixizumab, and zilucoplan. The following are settings where a gMG biological agent are PBS subsidised: (1) 3 months of acute treatment - 'acute severe gMG' (2) 6 months of bridging therapy - 'bridging therapy for gMG' (3) Continuous therapy - 'treatment refractory gMG' A patient may transition sequentially from one setting to another where all criteria are met e.g. (1) to (2) to (3), but cannot return to an earlier treatment setting. **Definitions:** A non-steroidal immunosuppressant (NS-IST) is one of the following: (i) azathioprine, (ii) ciclosporin, (iii) cyclophosphamide, (iv) methotrexate, (v) mycophenolate (vi) tacrolimus					

The Myasthenia Gravis Foundation of America (**MGFA**) Disease Classification can be accessed at https://myasthenia.org/ The Myasthenia Gravis Composite (**MGC**) scoring profile can be accessed at https://www.criteria.blood.gov.au/NeurologicalScales#MGC The Myasthenia Gravis-Activities of Daily Living (**MG-ADL**) scoring profile can be accessed at https://myasthenia.org/
Administrative Advice: **Treatment switching:** Switching between gMG biological agents is permitted. Treatment switching should be limited to when a patient moves between different treatment settings, i.e. moving from 'acute severe gMG' to 'bridging therapy for gMG' [(1) to (2)]; or moving from 'bridging therapy for gMG' to 'treatment refractory gMG' [(2) to (3)]. In the acute severe (1) or bridging therapy (2) settings a patient may switch to an alternate gMG biological agent if they are experiencing an intolerance or toxicity necessitating treatment withdrawal. Patients can switch by requalifying through the relevant restrictions. Only a balance of the time limits specified within the relevant treatment phase will be approved i.e. the remaining of the 3 months (acute severe gMG); or 6 months (bridging therapy for gMG) after accounting for the treatment of the previous gMG biologic. In the treatment refractory setting (3), a patient may switch to an alternate gMG biological agent via either: a) the initial restriction if they have trialled a different gMG biologic, but did not respond to treatment; or b) the continuing restriction if they have trialled a different gMG biological agent and have responded to treatment. Mark any remaining unused repeat prescriptions as "cancelled".
Administrative Advice: Any queries concerning the arrangements to prescribe may be directed to Services Australia on 1800 700 270 (hours of operation 8 a.m. to 5 p.m. Monday to Friday). Prescribing information (including Authority Application forms and other relevant documentation as applicable) is available on the Services Australia website at www.servicesaustralia.gov.au Applications for authorisation under this restriction should be made using the Online PBS Authorities system (see www.servicesaustralia.gov.au/hpos) Alternatively, applications for authority to prescribe should be submitted online using the form upload facility in Health Professional Online Services (HPOS) at www.servicesaustralia.gov.au/hpos Or mailed to: Services Australia Complex Drugs Reply Paid 9826 HOBART TAS 7001
Administrative Advice: No increase in the maximum quantity or number of units may be authorised.
Administrative Advice: No increase in the maximum number of repeats may be authorised.
Administrative Advice: Special Pricing Arrangements apply.
Restriction Summary [new3] / Treatment of Concept: [new3A]
Indication: Treatment refractory generalised myasthenia gravis (gMG)
Treatment Phase: Initial treatment – Treatment refractory gMG patients
Clinical criteria:
Patient must have a diagnosis of MGFA Disease Class II to IV
AND
Clinical criteria:
Patient must have a positive serology for acetylcholine receptor (AChR) binding autoantibodies
AND
Clinical criteria:
Patient must not be experiencing a myasthenic crisis

AND
Clinical criteria:
Patient must not have received treatment with a gMG biologic within 3 months prior to the first authority application for this indication (i.e. in treatment refractory setting); or
Patient must be considered by the treating clinician to have deteriorating gMG disease during a treatment break with a gMG biological agent;
AND
Clinical criteria:
Patient must not be receiving concomitant treatment with any of the following: (i) another gMG biological agent, (ii) intravenous immunoglobulin (IVIg), (iii) plasma exchange (PLEX), (iv) rituximab for this condition.
AND
Clinical criteria:
Patient must be receiving concomitant treatment with a non-steroidal immunosuppressant (NS-IST); or
Patient must have had a thymectomy
AND
Clinical criteria:
Patient must have a MG-ADL score of ≥ 6 and a MGC score of ≥ 10, despite having undergone 2 of the following 3 remission inducing treatments: (i) NS-IST for a minimum of 12 months, (ii) oral corticosteroids for a minimum of 12 months; (iii) a thymectomy.
AND
Clinical criteria:
The treatment must provide no more than 6 months of therapy per initial authority application
Treatment criteria:
Must be treated by a prescriber who is either: (i) a neurologist; (ii) a clinical immunologist with expertise in the treatment of myasthenia gravis, or
Must be treated by a medical practitioner who has consulted at least one of the above mentioned specialist types, with an agreement reached that the patient should be treated with this pharmaceutical benefit on this occasion
Prescribing Instruction: Patients who are considered to have deteriorating gMG disease while on a treatment break with a gMG biologic may qualify with 9 months of remission inducing treatments (rather than 12 months)
Prescribing Instructions: The authority application must be via the Online PBS Authorities System, or in writing and must include: (a) details of remission-inducing treatments [date commencement and duration of drug therapy including drug names and dosages, and/or date of the thymectomy] (b) the baseline MG-ADL and MGC scores assessed after completing the 12 months of remission-inducing treatments (include the date the assessments were conducted) If the application is submitted through HPOS form upload or mail, it must include: (1) details of the proposed prescription; (2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice);
Restriction Summary [new4] / Treatment of Concept: [new4A]
Indication: Treatment refractory generalised myasthenia gravis (gMG)
Treatment Phase: Continuing treatment – Treatment refractory gMG patients
Clinical criteria:
Patient must have previously received PBS subsidised treatment with a gMG biological agent for this PBS indication.

AND
Clinical criteria:
Patient must have demonstrated a clinical improvement based on a decrease in MG-ADL score of at least 2 points from baseline
AND
Clinical criteria:
Patient must have demonstrated a clinical improvement based on a decrease in MGC score of at least 3 points from baseline
AND
Clinical criteria:
Patient must not be receiving concomitant treatment with any of the following: (i) another gMG biological agent, (ii) intravenous immunoglobulin (IVIg), (iii) plasma exchange (PLEX), (iv) rituximab for this condition.
AND
Clinical criteria:
Patient must be receiving concomitant treatment with a non-steroidal immunosuppressant (NS-IST); or
Patient must have had a thymectomy
AND
Clinical criteria:
The treatment must provide no more than 6 months of therapy per continuing authority application
AND
Treatment criteria:
Must be treated by a prescriber who is either: (i) a neurologist; (ii) a clinical immunologist with expertise in the treatment of myasthenia gravis; or
Must be treated by a medical practitioner who has consulted at least one of the above-mentioned specialist types, with an agreement reached that the patient should be treated with this pharmaceutical benefit on this occasion.
Prescribing Instructions: The authority application must be via the Online PBS Authorities System, or in writing and must include the MG-ADL and MGC scores assessed from the most recent course of treatment. If the application is submitted through HPOS form upload or mail, it must include: (1) details of the proposed prescription; (2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice);
Restriction Summary [new5] / Treatment of Concept: [new5A]
Indication: Treatment refractory generalised myasthenia gravis (gMG)
Treatment Phase: Transitioning from non-PBS to PBS-subsidised treatment - Grandfathering treatment
Clinical criteria:
Patient must have received non-PBS subsidised treatment with this drug for this PBS indication prior to [PBS listing date].
AND
Clinical criteria:
Patient must have a diagnosis of MGFA Disease Class II to IV
AND
Clinical criteria:
Patient must have a positive serology for acetylcholine receptor (AChR) binding autoantibodies

AND
Clinical criteria:
Patient must not be experiencing a myasthenic crisis
AND
Clinical criteria:
Patient must not be receiving concomitant treatment with any of the following: (i) another gMG biological agent, (ii) intravenous immunoglobulin (IVIg), (iii) plasma exchange (PLEX), (iv) rituximab for this condition.
AND
Clinical criteria:
Patient must be receiving concomitant treatment with a non-steroidal immunosuppressant (NS-IST); or
Patient must have had a thymectomy
AND
Clinical criteria:
Patient must have had a MG-ADL score of ≥ 6 and a MGC score of ≥ 10, despite having undergone 2 of the following 3 remission inducing treatments: (i) NS-IST for a minimum of 12 months, (ii) oral corticosteroids for a minimum of 12 months; (iii) a thymectomy.
AND
Clinical criteria:
Patient must have demonstrated a clinical improvement based on a decrease in MG-ADL score of at least 2 points from baseline
AND
Clinical criteria:
Patient must have demonstrated a clinical improvement based on a decrease in MGC score of at least 3 points from baseline
AND
Clinical criteria:
The treatment must provide no more than 6 months of therapy under this restriction
Treatment criteria:
Must be treated by a prescriber who is either: (i) a neurologist; (ii) a clinical immunologist with expertise in the treatment of myasthenia gravis, or
Must be treated by a medical practitioner who has consulted at least one of the above mentioned specialist types, with an agreement reached that the patient should be treated with this pharmaceutical benefit on this occasion
Prescribing Instructions: The authority application must be via the Online PBS Authorities System, or in writing and must include: (a) details of remission-inducing treatments [date commencement and duration of drug therapy including drug names and dosages, and/or date of the thymectomy] (b) the baseline MG-ADL and MGC scores assessed after completing the 12 months of remission-inducing treatments (include the date the assessments was conducted) (c) the MG-ADL and MGC scores assessed from the most recent course of treatment demonstrating clinical improvement to treatment (include the date the assessments were conducted) If the application is submitted through HPOS form upload or mail, it must include: (1) details of the proposed prescription; (2) a completed authority application form relevant to the indication and treatment phase (the latest version is located on the website specified in the Administrative Advice);

Prescribing Instruction: A retrospective assessment for one of the MGC score or MG-ADL score after completing the 12 months remission inducing treatments can be accepted for grandfathered patients in cases where it was not conducted prior to commencing non-PBS subsidised treatment for this indication.
Administrative Advice: Patients may qualify for PBS-subsidised treatment under this restriction once only. For continuing PBS-subsidised treatment, a 'Grandfathered' patient must qualify under the 'Continuing treatment' criteria.
Administrative Advice: This grandfather restriction will cease to operate from 12 months after the date specified in the clinical criteria.

8 Context for Decision

The PBAC helps decide whether and, if so, how medicines should be subsidised through the Pharmaceutical Benefits Scheme (PBS) in Australia. It considers applications regarding the listing of medicines on the PBS and provides advice about other matters relating to the operation of the PBS in this context. A PBAC decision in relation to PBS listings does not necessarily represent a final PBAC view about the merits of the medicine or the circumstances in which it should be made available through the PBS. The PBAC welcomes applications containing new information at any time.

9 Sponsor's Comment

The sponsor had no comment.