国立保健医療科学院　保健医療経済評価研究センター
Center for Outcomes Research and Economic Evaluation for Health (C2H), National Institute of Public Health (NIPH) | URL:https://c2h.niph.go.jp

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[C2H2204] Summary of cost-effectiveness evaluation of Efgartigimod Alfa (Vyvgart)

1. Purpose of use

Generalized myasthenia gravis (gMG) patients who are not sufficiently responsive to steroids or an immunosuppressive drug)

2. Price of the device

Efgartigimod alfa has been reimbursed since April 2023, and the drug price is JPY 421,455 for Vyvgart® for intravenous infusion 400 mg as of September 2023. The price is determined based on the Cost Calculation Method with a 5% usefulness premium and a 10% market premium (no premium coefficient). The product is designated as an item for the Cost-effectiveness Evaluation with H1 classification.

3. Scope of Cost-effectiveness Evaluation

Efgartigimod alfa is used to improve symptoms of gMG. The scope of Cost-effectiveness Evaluation determined at the first session of the Expert Committee of Cost-Effectiveness Evaluation (ECCEE) is described below.

As it is expected that the efficacy of efgartigimod alfa could differ between acetylcholine receptor antibody–positive and antibody–negative patients, those populations were determined as the target populations. The comparators were prednisolone ± immunosuppressive drug ± acetylcholinesterase inhibitor.

Target population	Generalized myasthenia gravis (not sufficiently responsive to steroids or an immunosuppressive drug) (a) Acetylcholine receptor antibody–positive patients (b) Acetylcholine receptor antibody–negative patients
Comparator	Prednisolone ± immunosuppressive drug ± acetylcholinesterase inhibitor

4. Evaluation of additional benefits

The manufacturer did not conduct a systematic review in the base case analysis and evaluated the additional benefits of efgartigimod alfa for patients with gMG as a whole without complying with the scope determined at ECCEE. Based on the results of the Quantitative Myasthenia Gravis (QMG) responder rate (defined as a score improvement rate of ≥ 3) and the Myasthenia Gravis-Activities of Daily Living (MG-ADL) responder rates (defined as a score improvement rate of ≥ 2) in the ADAPT trial, a randomized controlled trial for efgartigimod alfa, the manufacturer concluded that efgartigimod alfa had additional benefits in the gMG population as a whole.

The academic group considered that the evaluation should be conducted according to the scope, and therefore evaluated the additional benefits for each population based on the ADAPT trial.

For population (a), acetylcholine receptor antibody–positive patients, the QMG responder rates were significantly higher for efgartigimod alfa (efgartigimod alfa 41/65 [63%], placebo 9/64 [14%], odds ratio 10.84 [95% confidence interval {CI} 4.18–31.20]), and MG-ADL responder rates were also significantly higher for efgartigimod alfa (efgartigimod alfa 44/65 [68%], placebo 19/64 [30%], odds ratio 4.95 [95% CI: 2.21–11.53]).

Based on the results, the academic group concluded that efgartigimod alfa had additional benefits for population (a).

For population (b), acetylcholine receptor antibody–negative patients, the QMG responder rates were 10/19 (53%) for efgartigimod alfa and 7/19 (36.8%) for placebo, and the MG-ADL responder rates were 13/19 (68.4%) for efgartigimod alfa and 12/19 (63.2%) for placebo.

As the sample of antibody-negative patients was not sufficiently large and the results had large uncertainty, the academic group concluded that it could not be judged from the currently available evidence that efgartigimod alfa had additional benefits for population (b).

5. Results of the cost-effectiveness analysis

The manufacturer conducted cost-utility analysis using a Markov model, which had states of efgartigimod alfa or the comparator as first-line treatments, intravenous immunoglobulin or plasma exchange as second-line treatments, eculizumab as third-line treatment (only for population [a]), and the best supportive care.

In the academic analysis, cost-minimization analysis was performed for population (b). For population (a), the model structure and transition probabilities were revised as it was considered that the rates of patients receiving eculizumab were overestimated; the drug cost of and costs related to efgartigimod alfa were revised and productivity loss was excluded.

The ECCEE accepted the following:

Population	Comparator	ICER (JPY/QALY)
(a) Acetylcholine receptor antibody–positive patients	Prednisolone ± immunosuppressive drug ± acetylcholinesterase inhibitor	111,660,260
(b) Acetylcholine receptor antibody–negative patients	Prednisolone ± immunosuppressive drug ± acetylcholinesterase inhibitor	Cost increase