Cost-Effectiveness of Eculizumab and Efgartigimod for the Treatment of generalized Myasthenia Gravis

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Objective:
To evaluate the cost-effectiveness of eculizumab and efgartigimod, each added to conventional immunosuppressive therapy (CT) versus CT alone, among patients with refractory, anti-acetylcholine receptor antibody-positive (AChR-Ab+) generalized myasthenia gravis (gMG), and gMG patients, respectively.

Background:
Eculizumab was approved in the United States in 2017 to treat gMG in patients who are AChR-Ab+. Efgartigimod is currently under review by the Food and Drug Administration. The cost-effectiveness of these treatments is not known.

Design/Methods:
A semi-Markov model employing 4-week cycles over a two-year time horizon was developed from the healthcare system perspective. Model inputs, including mean change in quantitative myasthenia gravis score (QMG), were collected from pivotal trials of each intervention. Costs of drugs, drug administration, and myasthenia gravis-related hospitalizations and emergency room visits were included. Efgartigimod's price was estimated from company statements. Utilities were derived from unpublished data. Total costs, quality-adjusted life-years gained (QALY), and cost/QALY compared to CT were calculated using an annual discount rate of 3%. Scenario analyses were conducted to assess the impact of different dosing intervals and different populations on efgartigimod's cost-effectiveness. One-way and probabilistic sensitivity analyses were conducted to evaluate uncertainty.

Results:
In refractory, AChR-Ab+ gMG patients, eculizumab had $855,400 in total costs, 1.13 QALYs, and cost/QALY of $5,210,000. Among the same patient population, efgartigimod incurred total costs of $710,900, 1.30 QALYs, and cost/QALY of $1,976,000 compared to CT and dominated eculizumab. In gMG patients, weekly dosing efgartigimod had $692,700 total costs, 1.27 QALYs, and cost/QALY of $2,076,000. With redosing occurring every 8 weeks, efgartigimod had $697,000 total costs, 1.23 QALYs, and cost/QALY of $2,442,000. At willingness to pay thresholds of up to $200,000, CT remained the preferred therapy in all one-way sensitivity analyses and probabilistic sensitivity analysis runs.

Conclusions:

Eculizumab and efgartigimod, using the annual placeholder price, exceed typical willingness-to-pay thresholds which may result in limited patient access.