Exploring the potential value of improved care for secondary hyperparathyroidism (SHPT) with a novel investigational calcimimetic therapy

Stollnwerk B1, Iannazzo S2, Cooper K3, Belozeroff V3

1Amgen (Europe) GmbH, 2SHS Health Economics Consulting, 3Amgen Inc.

OBJECTIVES

Healthcare decision makers are increasingly interested in the economic value of emerging therapies. We assessed the potential value of a new investigational intravenous calcimimetic, etelcalcetide, i.e. Parsabiv™ [1,2] administered during dialysis sessions for the treatment of secondary hyperparathyroidism (SHPT) in adult patients on hemodialysis in the United States (US). In a comparative, double-blind trial, etelcalcetide showed superior reduction of parathyroid hormone levels compared to cinacalcet [1]. Cinacalcet (Sensipar™) is another currently available calcimimetic that is taken as a daily pill. However, since the real-world doses of cinacalcet are different from the trial doses, we estimated a range of plausible doses for etelcalcetide to define a range of prices where economic value could be demonstrated.

METHODS

We used a Markov model to estimate the cost-utility of etelcalcetide compared to cinacalcet [3]. This model was based on data from etelcalcetide trials [1,2] and previously published cost-effectiveness models in SHPT. It allowed extrapolation of treatment effects on mortality, cardiovascular events, fracture and parathyroidectomy (Figure 1). The efficacy of calcimetics on clinical outcomes is based on previous publications [4,5] and illustrated in Figure 2. Event costs and phosphate binders/vitamin D costs were taken from a US cinacalcet cost-effectiveness model [4]. The persistence of calcimetics was taken from US real-world data [6].

RESULTS

The estimated potential doses ranged from 30.0 mg to 7.8 mg per day for cinacalcet and from 2.5 mg to 7.0 mg per administration for etelcalcetide. Based on a WTP threshold of $150,000/QALY, cost-effectiveness analysis supports value-based prices for etelcalcetide from $17.82/mg - $35.30/mg.

For all considered scenarios (doses and WTP thresholds), the potential value-based prices ranged from $16.32 to $47.00 per QALY.

Figure 1. Events, resources and outcomes captured by the cost-effectiveness model.

Four calcimetics dosing scenarios were explored: trial mean dose; trial efficacy assessment period mean dose; estimated real-world mean dose (for etelcalcetide estimated based on open label extension study data [7]); estimated real-world most common dose (same sources as previous).

To determine the value-based price range, we used the cinacalcet wholesale acquisition cost (WAC) price of $0.83/mg and applied different potential US willingness-to-pay (WTP) thresholds (i.e. $100,000, $150,000 and $300,000 per quality-adjusted life-year (QALY)) [8-11].

Figure 2. Risk reduction in persistent subjects.

REFERENCES


DISCUSSION

Based on dosing scenarios which may be expected in real world practice, we determined a range of value-based prices for etelcalcetide in the US. This approach, which relies on the cost-effectiveness methodology and the best available evidence, represents a novel framework for exploring the potential value of a new medicine. In the analysis we varied the calcimimetic drug usage, but assumed the change of efficacy to be negligible. On the one hand, lower doses may yield lower efficacy. On the other hand, the impact of dosing on efficacy is limited: the ability to lower PTH or to reduce clinical events cannot infinitely be increased by increasing the calcimimetic dose.

In clinical trials doses of calcimetics may be titrated to levels that are higher than observed in real world practice. This may in part be due to lower PTH target levels than are used in clinical practice (e.g., 300 vs. 600 pg/mL) and/or lack of dose reduction unless necessary for safety reasons. As a consequence, in trials the calcimetics dose may exceed the dose that is needed to reduce clinical events.

Currently there is no empirical evidence that would demonstrate a calcimetics dose-dependency. The usage of trial doses, however, is expected to over-estimate calcimimetic costs, and therefore under-estimate cost-effectiveness. Therefore it might be valuable to consider alternative dosing scenarios.

CONCLUSIONS

Cost-effectiveness analysis is one method to estimate the range of prices for new therapies that may represent good value for money. It also enables the exploration of different doses that may be used in actual clinical practice.

CONFLICTS OF INTEREST

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