Cost-Effectiveness Model for On-Demand Treatment of HAE Attacks

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BACKGROUND

• Hereditary angioedema (HAE) is a rare C1-inhibitor (C1-INH) deficiency characterized by recurrent episodes of painful and often debilitating swelling in subcutaneous and/or submucosal tissues.

• Therapeutic agents targeting the specific physiological pathway of HAE attacks can offer improved outcomes with limited side effects compared to heparin.

• HAE attacks can be unpredictable and the need to treat sudden attacks promptly and effectively is critical to minimize risk of hospitalization or death.

• Depending on frequency and severity of attacks, on-demand treatment may remain a suitable option for many patients; even patients receiving prophylaxis therapy can experience "break-through" attacks for which on-demand rescue medication is required.

• Expensive HAE therapies have been even more costly due to frequent re-dosing and downstream costs associated with administration and hospitalization.

OBJECTIVE

• Cost and utility estimates for on-demand treatment of HAE attacks that take into account re-dosing rates, administration costs, and associated effects will help to better clarify and control disease management expenses.

METHODS

• Treadle Pro software was used to develop a decision tree model to evaluate costs and utilities associated with on-demand treatment of HAE attacks (FIGURE 1).

• Four comparators were included: Berinert® (pdC1INH), Firazy® (octasent®), Kalbitor® (ecallantide), and Recosent® (rhC1INH).

• Variables specific to each therapy included drug cost as Wholesale Acquisition Cost (WAC), proportion of self-administration, and the ability to self-administer (pdC1INH, icatibant, and rhC1INH are all labeled for self-administration).

• Resulting cost-effectiveness scatter plots were derived from PSA model (FIGURE 2).

• Weight-based dosing of pdC1INH and rhC1INH with different vial utilization appears in PSA scatter-plot as point diamonds.

RESULTS

• Base-case scenario results are shown in TABLE 3. Cost per attack is a key re-dosing, healthcare-provider administration, drug price mark-up, and potential hospitalization.

• Quality-adjusted life-years (QALY) are out of 72 hour attack period.

• Derived effectiveness compares to attack-free HAE utility of 0.83.

• Budget impact model results (TABLE 3) show annual treatment costs ranging from $6.64M (rhC1INH) up to $10.93M (ecallantide).

• The model indicates that rhC1INH is the dominant therapy in the base case analysis; rhC1INH is both less expensive and more effective than other therapeutic options.

• Ecallantide is highly effective but also the most expensive, resulting in the least cost-effective therapy in the model.

• Driven by higher re-dosing rates, icatibant suffers from comparatively poor cost-effectiveness measures.

• Tornado diagrams (FIGURE 2) indicate that costs are widely influenced by re-dosing rates (1 x to 2 x most influential in all utilized) and the ability to self-administer (pdC1INH, icatibant, and rhC1INH are all labeled for self-administration).

• Population-weighted averages in tornado diagram for pdC1INH, but rhC1INH were through separate weight-based dosing; because rhC1INH is dosed as 250u, subjects over 42kg sell 2 vials, while pdC1INH is dosed as 500u in number of doses used per year.

• PSA scatter-plot (FIGURE 3) shows each of 5000 trials as pale dots, while mean cost-effectiveness is shown by large diamonds.

• Mean cost and effectiveness from PSA: $12390 and 0.786 for rhC1INH, $14132 and 0.738 for icatibant, $13050 and 0.804 for pdC1INH.

• Large variance in cost for pdC1INH and rhC1INH results from weight-based dosing, while self-administration and re-dosing rates influence both cost and effectiveness.

• Sensitivity analyses to assess the robustness of the model were performed by assigning value ranges to each variable in the model and analyzing the impact of both a maximum and minimum form the upper and lower bounds of the distribution while the peak of the distribution is the baseline value.

• Upper and lower range limits were drawn from published literature such as peer-reviewed journals, conference proceedings and abstracts when possible.

• One-way tornado diagrams were used to assess the influence of each variable on the overall cost estimates of the model.

• A 5000-trial probabilistic sensitivity analysis (PSA) was performed to evaluate ranges of cost-effectiveness for each therapy for simultaneous variability across all model variables, with a cost-effectiveness scatter plot used to establish variance for the therapies.

• Accounting for associated downstream costs of on-demand treatment for HAE attacks presents a more complete picture of disease management expenses than drug costs alone.

• Cost-effectiveness is significantly influenced by re-dosing rates and the ability to self-administer.

• This model indicates that rhC1INH is most cost-effective in many scenarios while ecallantide is the least cost-effective.

• Although a rare disease, appropriate selection of on-demand therapy could represent substantial savings to the health system.

CONCLUSIONS

• The study was conducted by AHRM Inc and supported by Pharming Healthcare Inc, USA.

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• Although a rare disease, appropriate selection of on-demand therapy could represent substantial savings to the health system.

References


Disclosures

1 CT employees of Pharming Inc; 2 employees of Pharming Healthcare Inc; 3 employees of Pharming, Inc; 4 employee of Pharming Inc.

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