Actuarial Value Chain Modelling of Valoctocogene Roxaparvovec (Valrox) Therapy for Hemophilia A (An Adaptation from ICER Final Evidence Report*).

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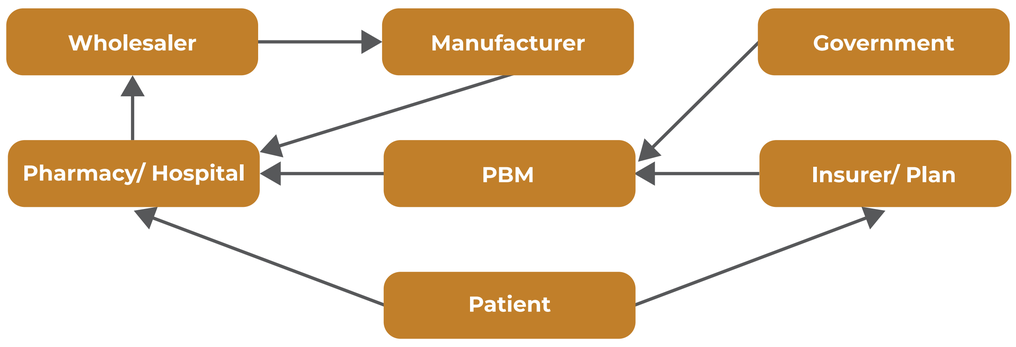
BACKGROUND

Gene therapy is a promising new treatment for Hemophilia A. This study used an actuarial value chain model to project the cashflows of Valrox, a new gene therapy for Hemophilia A, over a 15-year horizon. The study model was based on the efficacy and value report published by ICER to project net income for the manufacturer and the insurer over a 15-year horizon. Our study demonstrates the value of combining actuarial. health economics and outcomes research (HEOR) modelling to inform patient access and drug coverage decisions.

METHODOLOGY

An actuarial value chain model was built in Microsoft Excel (2019) to represent the cashflows between 7 health entities (insurer, government, manufacturer, wholesaler, Pharmacy Benefit Management (PBMs), pharmacy, patient) over a 15-year horizon. The model considers the following inputs and assumptions:

- Age and gender demographics: USA Population (USAfacts)
- Prevalence of Hemophilia A (Separately for children and adults, as a % of population)
- Treatment Market Share growth
- Success rate of Valrox treatment
- Treatment wear-off Hemophilia A monthly productivity costs 2022
- Treatment cost of Valrox
- Alternative treatment cost
- Adverse events costs
- Quality of life



STEPS

1. **Population Count**
   The model first yields outputs as – population counts on a monthly basis for those – healthy, unhealthy and untreated, successfully treated and unsuccessfully treated patients.
2. **Costs Associated with Treatment**
   The costs associated with each population: direct costs, indirect costs due to productivity loss, alternative treatment costs, adverse event costs, and inflation is weighed against QoL.
3. **Additional costs**
   These treatment costs are represented as cashflows from patients to other entities including overhead costs, profit margins, discount rate, service fees and rebates, risk sharing as per the insurance plan design.
4. **Final Output**
   The final output is the net income calculated based on the model and use the same to forecast over a 15-year time horizon in present value terms.

Deterministic scenario-based sensitivity analyses were performed to test different treatment uptake scenarios (0%, 50%, 100%, and 200% of baseline), lifetime model horizon, net income for each stakeholder, and QALY impact of drug pricing strategies (e.g. discounts, rebates, risk sharing). Note that the benefits of Valrox are overestimated with a shorter time horizon, as projected Factor VIII levels decrease over time.

CONCLUSION

The findings suggest that Valrox will generate a significant net income for the manufacturer and the insurer over a 15-year horizon. When combined, actuarial and HEOR (health economics and outcomes research) modeling can help gain new insights into the potential impact of Valrox on net gains by all entities of the drug supply value chain that critically impact drug coverage decisions. Also, manufacturers can foresee the financial terms of drug benefits resulting from different utilization scenarios.

FUTURE WORK

The actuarial model developed in this study can be extended in a number of ways. For example, the model could be extended to include a lifetime time horizon, and to incorporate additional scenarios, such as different treatment costs and different patient outcomes. More scenarios can be tested based on stakeholder feedback and data availability. The model can also be used to test alternative risk sharing and rebate scenarios.

RESULTS

The Population count is generated in the model (Table 1). Net gain is calculated and the value is cross verified as per ICER report placeholder cost of Valoctocogene Roxaparvovec. After a reasonable check of calculations is done, the cost of treatment was calculated as $344 Million for 15 years. This figure is used to model further cashflows.

Table - 1

Quality of Life Years	Healthy	29,835,604
	Unhealthy	155
	Gains from Treatment	276
	Total	**29,836,035**
Successful Treatment	Alternative Costs Avoided	340,824,370
	Productivity Costs Avoided	6,719,109
	Cost of Treatment	344,216,930
	Adverse Events	838,375
	Net Gain	**2,488,174**

Table - 2

	Patient	Manufacturer	Wholesaler	Pharmacy/ Hospital	Pharmacy Benefit Management	Insurer
Inflows		$ 212.39	$ 259.58	$ 306.13	$ 251.63	$ 307.55
Outflows	$ (407.80)	$ -	$ (212.39)	$ (259.58)	$ (205.88)	$ (251.63)
Overhead		$ (191.15)	$ (21.24)	$ (25.96)	$ (20.59)	$ (25.16)
Net Cashflow	$ (407.80)	$ 21.24	$ 25.96	$ 20.59	$ 25.16	$ 30.75
Margin		10%	10%	7%	10%	10%

Cashflows for 6 entities in terms of Net Present Value (in Millions)

A total outflow of $407 Million from patients treated with Valrox is shown to generate a Net Present Value gain of $21.24 Million for the Manufacturer, $25.96 Million to the Wholesaler, $20.59 to Pharmacies, $25.16 Million to PBMs, and $30.37 Million to Insurers. The model also shows that the benefits of gene therapy are overestimated over a shorter term (15 years) as projected Factor VIII levels decrease over time. More scenarios can be tested based on stakeholder feedback and data availability.

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The Point is Data