PAYING FOR CURES

Use case: Value Based Contract Impact Assessment Model

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The Value Based Contract Impact Assessment Model (VBCAM) is an open-access tool developed to facilitate the planning of value-based contracts (VBCs) for cell and gene therapies and other novel therapies. VBCAM can be used during risk identification and financial planning before contracting parties enter into an agreement for a new clinical product. VBCAM incorporates the efficacy estimates from the product's clinical trials alongside performance values that reflect the payer's beliefs about the product's real-world performance.

This hypothetical case for use for VBCAM illustrates its role in the development of a value-based contract for a sickle cell gene therapy. The expected outcome is the absence of hospitalizations due to vascular-occlusion events (VOEs) for 24 months post-infusion. In the use-case clinical trial, 34 of 39 individuals (87.1%) had a complete absence of hospitalization due to VOEs 24 months post infusion. The use case assumes the same coverage criteria under all scenarios.

THE PAYER PERSPECTIVE

The payer is an insurance plan responsible for 750,000 lives with 24 enrolled individuals known to meet the criteria for sickle cell gene therapy. Based on clinical trial results indicating an 87.1% success rate, treating these 24 individuals may result in three or more outcome failures. The payer is also concerned that the gene therapy will not be as effective for real-world treatments outside of the controlled setting of the clinical trial. The payer and developer are negotiating a value-based contract to address the clinical uncertainty concerns of the payer. They are using VBCAM to evaluate the range of likely clinical outcomes associated with a value-based rebate agreement and its financial implications.

The payer inputs to VBCAM are as follows:

- Gene therapy cost: $3,200,000/patient
- Performance tracking costs for VBC: $3,000/patient
- Rebate for patients with a product performance failure: 75%
- Number of patients anticipated to be treated annually: 8
- Term of the VBC: 3 years
- Discount offered as alternative to VBC rebate:10%

The cost of treatment with the 10% discount and no VBC is presented in Table 1.

Table 1: Treatment costs with and without 10% discount for a total of 24 patient treatments

Flat rebate scenario	Market costs	Costs after discount 10%
Per patient costs	$3,200,000	$2,880,000
Per cohort cost (8/yr)	$25,600,000	$23,040,000
Total costs (3yr contract)	$76,800,000	$69,120,000

Value Based Contract Impact Assessment Model (VBCAM) use case

Using VBCAM, the payer can compare treatment costs for the product between a VBC and a non-value-based discount. VBCAM simulates the financial impact based upon clinical trial results, the payer's projections of successful outcomes (subjective belief), and a weighted combination of these two outcome rates that combines information from the clinical trial with the subjective beliefs (Combined: Posterior distribution).. The model compares the financial impact results of all three scenarios to the financial impact of a discount in lieu of a VBC. The underlying model in VBCAM does not consider the impact of payer re-insurance or stop-loss coverage.

In this example, the payer anticipates successful outcomes in 80% of treated patients in a real-world environment for the payer's covered population – a slight adjustment from the clinical trial success rate of 87.1%. VBCAM reports outcomes in all three scenarios as well as the lower and upper bounds of costs due to outcome variability.

VBCAM addresses performance uncertainty by considering the clinical trial failure rate of 12.9% (87.1% success rate), a real-world anticipated failure rate of 20% (80% payer anticipated success rate), and the combined failure rate of 16.5% (83.5% success rate). Simulating a 75% rebate in the VBC terms reveals average rebates equating to 11.0%, 16.4%, and 12.6% of costs respectively. The variability in the outcomes is significant however, as the combined success rate of 83.5% could translate to plausible discounts ranging from 0% to 31%.

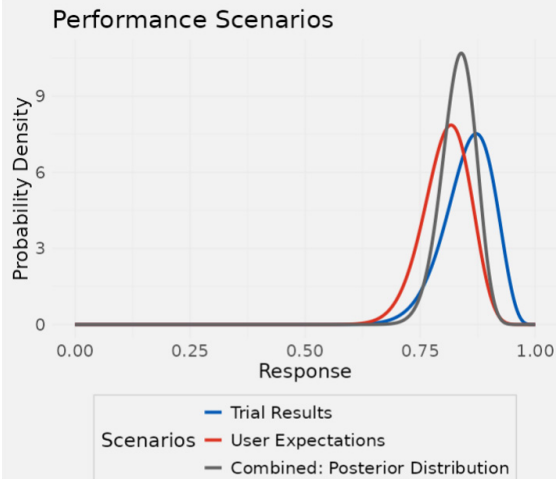

Figure 1: Outcome success probability based on clinical trial results, payer expectations and a composite of the two.

A single patient being treated one time has only two possible financial outcomes – the full treatment cost ($3,203,000) or the treatment cost less the rebate ($803,000) However, the model simulates a single patient treatment 10,000 times to determine the average outcome for the contract and the range in which the contract outcome will fall 95% of the time. This range of outcomes underscores the unpredictability of treatment success and provides insight into the outcome and financial risks associated with the different treatment success rates.

The payer insights gained from this modeling include:

- Sharing the outcome risk though a VBC for the 24 patients would potentially decrease the full product costs of $76.9 million (product cost + outcome tracking cost). Based on the blended success rate of 83.5%, product costs could plausibly decrease by as much as $25 million or not decrease at all if all patient treatments are successful.
- Achieving the average per patient cost based on outcomes is feasible but improbable, given the wide spread of possible outcomes, as indicated by the plausible equivalent discount range of 0% to 31%.
- On average, a 75% rebate within a VBC will offer a larger discount than a 10% flat discount rate.
- Acceptance of a 10% flat discount would deliver financial certainty but would not address the concern for performance risk.
- Contracting for the 10% discount may provide some cost savings without the administrative burden of a VBC, but it also means that the product performance risk is not shared.

Table 2 Treatment costs modeling outcomes and rebates under a VBC

VBC scenario	Unit	Clinical trial outcomes scenario	Payer anticipated outcomes scenario	Blended outcome rate scenario
Assumed performance	Rate	87.1% (34/39)	80.0%	83.5%
Per patient costs	Average	$2,852,090	$2,678,750	$2,799,740
	95% plausible range	$ 2.2 to $3.2 million	$1.9 to $3.2 million	$2.1 to $3.2 million
Per cohort cost (8/yr.)	Average	$22,816,720	$21,430,000	$22,397,920
	95% plausible range	$17.7 – $25.6 million	$15.5 - $25.6 million	$17.3 - $25.6 million
Total costs (3 yr. VBC)	Average	$68,450,160	$64,290,000	$67,193,760
	95% plausible range	$53.0 - $76.9 million	$46.4 - $76.9 million	$51.8– $76.9 million

Note that because VBCAM is a statistical simulation the resulting estimates may differ slightly if you run VBCAM yourself with these same inputs. The payer insights above remain the same.

THE DEVELOPER PERSPECTIVE

For the developer analysis, we can go beyond the VBCAM results of one payer's population. We can consider the total number of patients covered by multiple payers in VBC arrangements with the developer. In our example, the developer expects 84 patients to be treated under VBC terms over the next three years, or 28 patients per year. Keeping all but the number of patients (28/year versus 8/year) consistent, VBCAM provides the results found in Table 3 and 4. The market cost numbers represent anticipated developer reimbursement.

Table 3: Flat based rebate revenue for a total of 84 patient treatments

Flat rebate scenario	Market costs	Costs after discount 10%
Per patient revenue	$3,200,000	$2,880,000
Per cohort revenue (28/yr)	$89,600,000	$80,640,000
Total revenue (3yr contract)	$268,800,000	$241,920,000

Value Based Contract Impact Assessment Model (VBCAM) use case

Table 4: VBC scenario revenue

VBC scenario	Unit	Clinical trial outcomes scenario	Payer anticipated outcomes scenario	Blended outcome rate scenario
Assumed performance	Rate	87.1% (34/39)	80.0%	83.5%
Per patient revenue	Average	$2,845,863	$2,671,606	$2,798,094
	95% plausible range	$2.4 to $3.2 million	$2.2 to $3.1 million	$2.4 to $3.2 million
Per cohort revenue (28/yr.)	Average	$79,684,164	$74,804,968	$78,346,632
	95% plausible range	$68.3 - $89.6 million	$61.8 - $87.9 million	$67.6 - $89.0 million
Total revenue (3 yr. contract)	Average	$239,052,492	$224,414,904	$235,039,896
	95% plausible range	$204.9 - $268.8 million	$185.3 - $263.6 million	$202.9 - $267.1 million

Using the same rebate percentage of 75%, simulating the VBCs with a larger population shows on average a similar discount rate to the payer with the smaller population: 11.0%, 16.4%, and 12.6% from successful outcome rates of 87.1%, 80%, and 83.5%. However, the variability in the financial outcomes, as seen in the 95% plausible ranges, is reduced. For instance, under the blended success rate scenario of 83.5%, an overall discount rate achieved from the VBCs now ranges from 0.6% to 24.5% for the developer.

The developer insights gained from this modeling include:

- On average, a 75% rebate within a VBC will result in lower revenues than if a 10% discount is offered. But achieving the "average" per patient cost, while feasible, is improbable.
- The larger number of treated patients has decreased the variability of results compared to the single payer's experience, narrowing the discount range to 0.6% to 24.5% as opposed to 0% to 31%. Additional increases in the number of patient treatments through VBCs will further decrease this variability.
- Negotiation of a 10% discount, on average, may deliver higher revenues for the developer. However, a developer's decision to complete a VBC would need to consider the 10% discount against the potential for an additional gain of up to 9.4% in revenue or an additional loss of up to 14.5% of revenue based on the model's outcomes, as well as other non-financial considerations.

VBCAM does not determine if a discount or value-based contract should be pursued. It is designed to facilitate negotiations between a developer and a payer using variables of percentage discounts and rebate values. Users should assess simulated results based upon their particular financial goals and outcome expectations.

For further guidance about considering a value-based contract from the payers' and developers' perspectives, see the NEWDIGS Implementation Brief, Is innovative contracting right for you?

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https://newdigs.tuftsmedicalcenter.org/payingforcures/