

Achieving Market Access for a Novel Ultra-Orphan Treatment

In the pharmaceutical industry, pricing a new product is one of the most complex decisions to be made given the number of stakeholders involved. Our client was a multi-national pharmaceutical company that recently developed a revolutionized cancer treatment but was struggling with communicating its value proposition to the market.

The Pricing Solutions team developed recommendations for market access and value pricing strategy in the EU5 and USA for the client's novel, first-in-class, ultra-orphan oncology therapy.

Achieved Market Access Across the US & the EU5

The Challenge

Our client had significant experience in developing novel therapies but was struggling with pricing their products according to value and in bringing them to market. Given the initial data from phase I trials, our client had high expectations for their novel therapy. They tasked the Pricing Solutions team with helping them to:

- Review and refine the therapy's value story and understand how it resonates with stakeholders
- Determine the likely market access and finding pathways for the therapy given the [ultra-rare] orphan disease status
- Assess Payer willingness-to-pay for the product and determine a price corridor for the product in each scope market

Building a Solution That Fits

In developing a solution to the client's needs, Pricing Solutions highlighted one major obstacle – the rarity of the disease. This meant that even experienced stakeholders [Payers] had very low awareness and understanding of what it entailed, its epidemiology and the available treatments for it. To overcome this, we created a 3-step process:

1. Information Gathering Interviews

Pricing Solutions team proposed an interview

programme involving 24 Payers and Key Opinion Leaders (KOLs) from US and European markets. To ensure an informed and knowledgeable discussion, Pricing Solutions held innovative 'Payer-briefing workshops', with Payers that allowed them to get an in-depth, first-hand understanding of the rare condition and the treatment being proposed.

We then conducted interviews with 43 Payers and 12 KOLS across the 6 markets of interest, where we discussed unmet needs, advantages and disadvantages of the new product, strength of the value messages supporting it, its development plan, potential reimbursement & pricing, and funding & access arrangements.

2. Qualitative Research Study

Our client had no initial hypotheses on target prices for their product. In order to establish a price corridor, Pricing Solutions utilised a Van Westendorp analysis that allowed us to have an open conversation behind pricing reasoning. In addition, we carried out a short 'Discrete Choice' exercise with Payers which helped us to identify key value drivers and their willingness-to-pay. An example of two discrete choice slides, similar to that reviewed by Payers, is shown below.

3. Final Recommendations

Feedback from the primary research program was collated with our own expertise, and insights from



desk research. As part of a detailed final report, Pricing Solutions provided:

- Stakeholder perceptions of the therapy, strengths and weaknesses of its trial design, and the extent to which it fulfilled unmet needs
- An outline of how the product would be assessed and used in each scope market
- Key recommendations on shaping phase II trials, how to begin to shape pricing and access expectations, the optimal value story and next steps for market access.

The Result?

Our client is now very well positioned to optimise their product's route to market access across the US and the EU5, and has a clear understanding of how the product will be received by stakeholders. They also better understand the true value of the advantages conferred by their therapy, how these can be reflected in its price and how value can be communicated.

As a result, Pricing Solutions was able to propose price levels that allowed for the desired level of access, which exceeded our client's expectations but were based on robust rationale.

This in turn will allow the client to begin pricing negotiations with Payers at a higher price point enabling the full value of the drug to be realised.

What's the Pricing Solutions Difference?

Pricing Solutions combines pricing and pharmaceutical expertise that helps clients to assess their market positioning even at early stages of a product development cycle. We adopt a holistic, multi-dimensional approach to pricing and access and have great experience in recruiting, interviewing and surveying a host of stakeholder types.

In this case, our team and our client believed that the innovative approach to briefing Payers on the therapy area and the product was crucial to robust outcomes for this project as with a deeper understanding they could provide more profound feedback. This insight helped our team to accurately determine stakeholder's willingness-to-pay, evaluate treatment's market value and develop a value-based pricing strategy for a novel treatment. If you'd like any further detail about this project or to see how Pricing Solutions can assist you, please do get in touch.

Our Firm

Our mission is to dramatically improve clients' profitability and market share through improved pricing. Pricing Solutions' four core services include Pricing Strategy, Pricing Analytics, Pricing Research and Pricing Training. Known for our World Class Pricing™ methodology, we have grown to become one of the world's leading pricing consultancies with offices in North America, Europe, Asia Pacific and Latin America. Our global team of researchers, consultants and partners are recognized thought-leaders.

We deliver customized cross-functional solutions, helping our clients achieve a typical payback of 10:1 on their investment.

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Which of these options would you be more likely to recommend for reimbursement, and why?

	Option A	Option B	Option C
Patient population	Patients with rare form of cutaneous T-cell lymphoma who have received at least one prior systemic therapy		
Efficacy	Overall response lasting at least 4 months (ORR4) = 20% PFS = 12 months	Overall response lasting at least 4 months (ORR4) = 25% PFS = 15 months	Overall response lasting at least 4 months (ORR4) = 30% PFS = 20 months
Safety & tolerability	Less adverse events compared to alternative Tx	Less adverse events compared to alternative Tx	Comparable to existing treatment options
Quality of Life results	No formal results	Improvements, as shown on a validated scale	Improvements, as shown on a validated scale
Price for year*	150,000€	200,000€	300,000€

*Prices are hypothetical

Which of these options would you be more likely to recommend for reimbursement, and why?

	Option A	Option B	Option C
Patient population	Patients with rare form of cutaneous T-cell lymphoma who have received at least one prior systemic therapy		
Efficacy	Overall response lasting at least 4 months (ORR4) = 38% PFS = 14 months	Overall response lasting at least 4 months (ORR4) = 35% PFS = 13 months	Overall response lasting at least 4 months (ORR4) = 40% PFS = 15 months
Safety & tolerability	Comparable to existing treatment options	Less adverse events compared to alternative Tx	Comparable to existing treatment options
Quality of Life results	Improvements, as shown on a validated scale	Improvements, as shown on a validated scale	No formal results
Price for year*	180,000€	220,000€	220,000€

*Prices are hypothetical

Above: Example of the discrete choice exercises Payers reviewed, to understand their key drivers for pricing decisions