

# Utilising payer research to inform early development of an orphan disease treatment

Gathering stakeholder insights across five target markets in the EU and US to explore the pricing and reimbursement landscape

## The challenge

Our client had a product in early development which utilised a novel mechanism of action in the treatment of a rare disease. This treatment targeted the underlying cause of disease, providing a significant advantage over current symptomatic therapies. A competitor product was being launched at the time of the research.

The client wished to explore the pricing and reimbursement landscape in this therapeutic area to align the development of their product with payers' expectations.

## The solution

We recommended national and regional payer research across the EU and the US. In total, thirteen interviews were conducted across five markets to gather a range of stakeholder insights from experts across the target markets.

Following a thorough review of the client's internal literature, we developed a range of interview materials including a comprehensive discussion guide and stimulus material. These ensured interviews were succinct and the resulting payer responses were comparable, enabling us to provide reliable recommendations to the client.

Insights were analysed for each market and presented in a strategic slide deck, with EU vs. US differences highlighted where appropriate.

## Key results

The responses from payers during interview provided insights on the available therapeutic options, the existing unmet need and the perceived value of our client's product.

The qualitative data gained from interviews also provided information into the evidence (e.g. efficacy / safety profiles, real world data) that would be required to secure reimbursement and achieve optimal pricing in the target markets.

Finally, interviewees shared their opinions on the potential influence of an orphan drug designation on the funding of this drug.

## Value to the client

Our research provided the client with substantial, qualitative information on the therapeutic landscape and payers' expectations to achieve optimal pricing and reimbursement for their novel product.

Conducted at the early stage of the development of this product, our research gave our client the opportunity to align trial design with payers' requirements and expectations.

Our results also outlined potential future funding opportunities and challenges that our client would need to consider when launching this product.

Figure 1. Payer insights presented in a various formats, illustrating qualitative/quantitative data

