Researching market access pathways, reimbursement requirements and implications of orphan drug designation for a rare disease product

What our client needed

With a rare disease therapy in early development, our client wanted to gain a deeper understanding of the market access routes available and the evidence requirements for key reimbursement pathways. In particular, they were looking to understand:

- What clinical evidence was used by the manufacturers of analogous rare disease therapies in their health technology assessment (HTA) submissions
- The impact orphan drug designation (ODD) had on HTA decision-making for these comparable products
- The ODD application process in Europe and the US
- The benefits of applying for European Medicines Agency (EMA) and US Food & Drug Administration (FDA) ODD

How we supported them

The project required a broad range of desk research approaches, including an HTA landscape assessment, research into the ODD application process, and a targeted literature review (TLR).

We conducted searches in the EU4+UK and the US to identify clinical trial phase evidence submitted to HTA agencies. We noted whether ODD was awarded prior to review, and analysed the critique and outcomes reported by HTA agencies in key markets. The HTA landscape assessment data were summarised into a data extraction table. Our wider findings, trends, conclusions, and recommendations were presented to the client in a PowerPoint deck.

We also conducted supplementary desk research into the EMA and FDA ODD submission process, which we presented in separate PowerPoint reports. These reports included the benefits and implications of each designation, along with our recommendations for the client's product.

A TLR was performed to provide further information on the evidence requirements in this space. This was achieved by identifying data relating to the therapy area, which included current treatments, epidemiology, cost and resource use, health-related quality of life, and economic evaluations. Whilst no geographical restrictions were applied to the TLR, the primary focus was the US, Canada, EU4+UK, Japan and China.

The outcome

We presented the client with detailed information and conclusions to support their future market access strategy. Our conclusions highlighted some of the expected clinical and economic evidence challenges typically associated with rare diseases, including appropriate clinical trial design with appropriate comparators, and high economic uncertainty, which may require a Patient Access Scheme (PAS) or discount.

How we added value

We delivered a detailed report for each research area, ensuring that the level of detail was sufficient for the client's needs. We applied client feedback to each of the individual reports, before creating a succinct executive summary that combined all workstreams and recommendations for their future market access strategy.