About CTCL

While there is no cure for haemophilia, there are effective treatments available. However, these treatments often require lifelong infusion of drugs manufactured from human plasma or through recombinant biotechnology. Patients with severe haemophilia often require infusions several times a week. Older patients are often able to manage their condition with less frequent infusions. Advances to create longer lasting clotting factors have reduced infusion frequency for some patients.

The CDC advises that good quality medical care from doctors and nurses who know a lot about the disorder can help prevent some serious problems. A haematologist specialises in blood disorders, including haemophilia. In the US, often the best choice for care is to visit a comprehensive haemophilia treatment centre (HTC). An HTC not only provides care to address all issues related to the disorder, but also provides health education to help people with haemophilia stay healthy.

Along with haematologists, patients may also be treated by orthopaedic specialists in disorders of the bones and joints, which are commonly affected by haemophilia, as well as physical therapists.

Recent advances in gene technology offer the promise of correcting the basic genetic defect with DNA sequences coding for normal proteins and having the patient produce their own Factor VIII or Factor IX proteins.

Business Objective

Working for a full-service market research company working with a clinical-stage biotechnology company focused on development of therapeutic antibodies for improvement in cancer treatment, M3 Global Research was seeking to secure “payers” to participate in the research.

In preparation for clinical trial designs, it was imperative...
to understand, from a payer perspective, the data that clinical trials need to demonstrate to satisfy pricing/reimbursement and market access requirements, as this information can subsequently inform the pharmaceutical company’s trial designs, with a need to understand the payer assessments of treatment across relevant markets with appropriate stakeholders, specifically. The study was designed to determine the:

• value proposition which most resonated with payers
• specific aspects for improved outcomes by the therapy which are most valued by payers
• monetary value associated with specific improved outcomes

An additional objective was to gain insight on the value of innovation derived from the product in each market. Specifically, to:

• Derive the revenue-optimising price within each market by defining the price point relative to the access granted (and thus number of patients treated)
• Determine the likely market access and funding pathways for the drug within each market given the ultra-rare orphan disease status
• Assess the suitability of innovative access schemes or managed market entry agreements for the drug, given the potential for a ‘high’ price

**Challenges**

Timing was critical for success of this project, with just under three weeks in field.

**THE SAMPLE FRAME M3 GLOBAL RESEARCH RECRUITED FOR THE STUDY**

### FRANCE

3 **NATIONAL**
Commission de Transparence (TCI) / Commission Évaluation Économique et de Santé Publique (CEESP) / DGOS

2 **LOCAL**
member of formulary committee, e.g. from Centres de Lutte Contre le Cancer (CLCC) and academic hospitals

### GERMANY

1 **NATIONAL**
member of the Federal Joint Committee (G-BA)

2 **REGIONAL**
sick fund (Krankenkasse) executives

1 **LOCAL**
university hospital pharmacist (payer)

### ITALY

3 **NATIONAL**
mix of AIFA representatives from CTS (Comitato Scientifico e Tecnico) and CPR (Comitato Prezzi e Rimborsi)

### SPAIN

2 **NATIONAL**
representative at Direccion General de Farmacia y Productos Sanitarios (DGDFPS)

4 **REGIONAL**
representative of health ministries of large autonomous regions (e.g. Madrid, Andalucia, Catalonian) / representatives of regional HTA centres

### UNITED KINGDOM

2 **NATIONAL**
NICE - affiliated advisor

4 **REGIONAL**
regional hospital payer, in charge of major oncology hospitals

### UNITED STATES

12 **NATIONAL / REGIONAL / LOCAL PLANS**
medical directors

4 **NATIONAL / REGIONAL / LOCAL PLANS**
hospital pharmacy directors
Another potential challenge was tied to pre-interview coordination. In advance of the interview, all payers were required to participate in an online briefing with a physician KOL to familiarise themselves with the product and treatment path.

The biggest challenge for this research, however, was focused around the ability to secure the required stakeholders within commercial and governmental agencies to participate in this research.

The M3 Global Research Solution

M3 compiled an internal team of experts with intimate local knowledge of market access for each required market. The internal M3 team collaborated with the client with a detailed in-person kick-off session to outline all the details of the project from sampling to technology to ultimate objectives to the niche recruitment criteria. This face-to-face approach and coordination between M3 and the client aided in establishing best practices for the project, creating a risk mitigation strategy for any potential pitfalls.

The London-based M3 call centre, with local language recruiters, provided crucial assistance in working with the unique methodology. With each respondent completing an online briefing prior to their tele-depth interview, M3 Global Research carefully explained and detailed this methodology to the payers, which contributed to the success of the project.

In addition to working with our proprietary sample for payers, M3 Global Research utilised external sources to introduce additional respondents to the research beyond existing resources.

The internal M3 project lead provided daily feedback in addition to weekly calls with the client to track progress and pivot directions as requested by the client team.

Results for Success

While initially there was concern about the online briefings and how receptive the payers would be to this additional step, it was well-received by the payers and was a cornerstone to the success of the research. This step spared the client from wasting valuable interview time briefing the payer about the concept/offering that the treatment provides. From the payer’s perspective, they felt well-informed and knowledgeable from the start of the interview, allowing them to be more confident in their responses and spend more time focusing on providing the insight tied to the research objectives.

Initial contact, followed by regular contact combined with a risk mitigation strategy, helped ensure a complete recruit. With expectations and requirements well-documented and a strategy in place, M3 was able to deliver a full recruitment within the constrained timeline.

The use of an M3 team knowledgeable in market access with local language capabilities and contacts also contributed to the completion of the recruit. Utilising M3’s team to recruit the project and network beyond panel capabilities contributed to the successful outcome.