

Mapping the Rare Disease Landscape for Strategic Action for a Top BioPharmaceutical Company

The Challenge

A top biopharmaceutical company, has a strong global reputation in rare disease, with a clear commitment to scientific innovation, patient impact and health system progress.

1 However, the rare disease landscape is complex, fragmented and increasingly competitive, with significant variation across countries.

This created a need for a clearer, evidence-based view of how rare disease leadership is showing up across priority markets.

The organisation needed a structured benchmarking approach to assess external environments consistently, identify strengths and gaps and support more informed strategic planning across countries.

The Solution

We are developing a comprehensive rare disease benchmarking framework designed to assess country-level performance across a set of strategic indicators.

The ongoing project brings together desk research, structured analysis and a clear rating methodology to help compare rare disease environments across priority markets in a consistent and practical way.

Our approach includes developing a clear benchmarking framework across key rare disease indicators, assessing country-level policy, access, advocacy, diagnostic and stakeholder environments.

The work is designed to be both rigorous and usable. Each country profile captures the key findings in a clear, comparable format, while allowing researchers to summarise complex information in a way that is concise, evidence-led and strategically meaningful.

As the project continues, the benchmarking will help better understand where rare disease is well recognised, where barriers remain and where communications, policy engagement and stakeholder activity could have the greatest impact.

This case study is anonymised to protect current client confidentiality.

