

Market access roadmap for an ultra-orphan drug for a genetic cardiovascular disorder



SUMMARY

The client gained clear direction on key activities to maximize market access and P&R opportunities, including opportunities for early access and potential revenue generation.

CLIENT SITUATION

A specialty biopharmaceutical company wanted to develop a market access roadmap covering 19 countries for its lead asset in development for an ultra-rare genetic cardiovascular disorder.

PRMA CONSULTING SOLUTION

Landscape assessment

We provided an overview of the market access landscape for ultra-orphan drugs in the scope countries, including describing the health technology assessment (HTA) processes, key stakeholders, and evidence requirements. This was supplemented by critically reviewing HTAs of seven analogous drugs that had recently been assessed and developing two case studies per country.

Market access roadmap

To develop the market access roadmap, we critically reviewed the evidence package for the product and identified evidence gaps and key vulnerabilities from a national market access perspective. We then suggested strategies and evidence generation activities that should be conducted to minimize the risk in each scope country.

CLIENT VALUE

- The landscape assessment provided a comprehensive overview of the market access environment and key differences for orphan and ultra-orphan drugs compared with non-orphan drugs in each market, with real-life examples to support key findings.
- The roadmap provided clear direction on the key activities that need to take place to maximize the market access and P&R opportunities and minimize risks, including key priorities, timelines, suggested methodologies, and indicative budgets for those activities.
- An overview of early access schemes in key markets was provided in order to highlight opportunities for early access and potential revenue generation.

Analog	NICE	SMC											
Praluent			No additional benefit	Class C-m		Cost, insufficient							Annex 1A
Kanuma	2nd draft guidance		Non-quantifiable			ASMR IV							
Carbaglu				Class H		ASMR II							Annex 1B
Translarna			Minor (subject to reassessment)	648/96 Law	(subject to reassessment)	ASMR IV (subject to reassessment)							
Replagal				Class H		ASMR II							Special – 100% "Expensive drugs" list
Fabrazyme				Class H		ASMR II							Special – 100% "Expensive drugs" list
Glybera			Non-quantifiable										

Recommended/fully Reimbursed
 Recommended/reimbursed with restrictions
 Not recommended/Reimbursed
 Not assessed