

# Market access and pricing opportunity for an asset in development for a rare and progressive form of liver disease



## SUMMARY

The client received clear payer feedback on the clinical value, trial design, and pricing opportunities. Strategic recommendations were provided for optimizing the value of the asset.

## CLIENT SITUATION

To inform due diligence, our client wanted to understand the market access, pricing, and reimbursement opportunities and challenges for an asset in development for an inherited, rare, and progressive form of liver disease, that usually starts in pediatric patients.

## PRMA CONSULTING SOLUTION

### Secondary research

A review of the literature was conducted to understand the:

- burden of disease
- clinical outcomes
- cost of relevant procedures.

Case studies of potential price analogs were also developed.

### Primary research

A discussion guide and pre-read was used to facilitate interviews in order to:

- elicit feedback on the target product profile
- understand perspectives on trial design, including surrogate endpoints
- assess willingness to pay
- identify activities that could be conducted to strengthen the value proposition.

One-to-one telephone interviews were conducted with 10 payers (4 in the US and 2 per EU3 country).

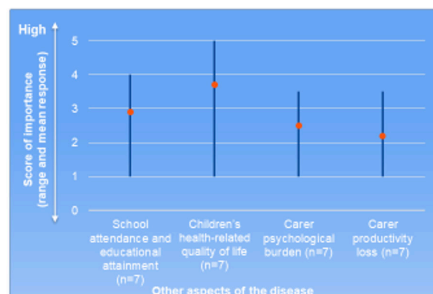
## CLIENT VALUE

- Based on the research the client received clear payer feedback on the unmet need, clinical value, trial design, and pricing opportunities with clear recommendations on how the value of the asset could be optimized and potential challenges mitigated.
- This informed the due diligence process and the decision whether the asset should be in-licensed.

### Delaying disease progression and preventing early death were considered the most important unmet needs

The most important unmet needs were considered to be delaying disease progression, preventing early deaths, and improving QoL.

- While QoL was an important consideration for decision-making in the EU3, it was less important in the US.
- Carers' psychological burden and productivity loss played a limited role in payers' perception of disease burden. In Germany, the burden of carers was not considered at all.



“G-BA looks at patient only not really care giver.”  
Germany #1

“The most important unmet needs are having a normal childhood life and improving children's QoL.”  
UK #1

“Progression of disease and the medical care offset are very important. I would rate them as a 5.”  
US #2