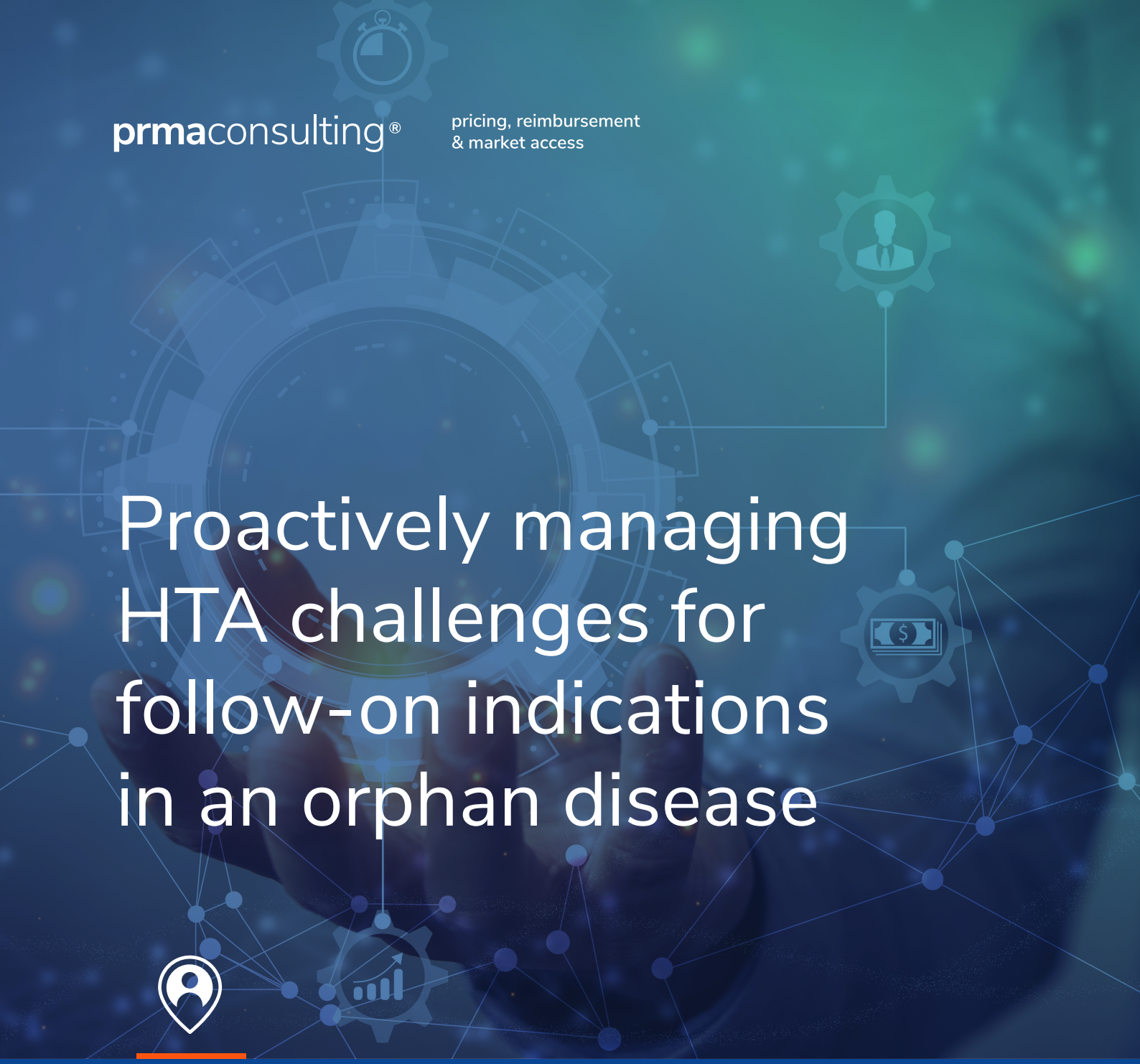
prmaconsulting®

pricing, reimbursement
& market access

Proactively managing HTA challenges for follow-on indications in an orphan disease



SUMMARY

Deep-dive primary research with payers in the US and EU5 enabled development of risk mitigation plans for a selection of market access pathways applicable to follow-on indications.

CLIENT SITUATION

- A multinational pharmaceutical company was developing a novel therapeutic approach for a rare hematological disease.
- The client needed to actively plan for the launch of their asset in the lead indication and several follow-on orphan and pediatric indications.
- Concerns had been raised about the implications of the follow-on indications for HTA and reimbursement evaluations: what would be the impact on price renegotiations?

PRMA CONSULTING SOLUTION

- Based on a review of analogous products, we provided a critical analysis of the impact of follow-on indications on the expected HTA, pricing, and reimbursement outcomes for the lead indication.
- Information gaps were closed via in-depth primary research with payers in the US and EU5.
- A risk mitigation plan was developed for a selection of market access pathways applicable to the follow-on indications.

CLIENT VALUE

- Key areas of focus in the submission strategy were highlighted: the accuracy of the estimated patient numbers was particularly important given the small population. The expected patient numbers in the follow-on indications had to be presented as part of the initial submission for the lead indication, to ensure:
  - the total eligible patient population did not exceed the orphan threshold for the product
  - flexibility to negotiate price up front and minimize subsequent price renegotiations.

I can offer only compliments; you were responsive, on task, on time. Your support and partnership was great and we would like to work with you again."

Chief Scientific Officer, multinational pharmaceutical company

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