prmaconsulting®

pricing, reimbursement
& market access

Early scientific advice for a product in development for a rare cardiovascular disease

SUMMARY

Evidence generation plans and market access strategy were informed by feedback from HTA agencies and analysis of the target population, appropriate comparators, endpoints, and proposed approach to economic modeling.

CLIENT SITUATION

A medium-size company developing products for rare diseases wanted to obtain early scientific advice (ESA) from key health technology assessment (HTA agencies (CADTH, G-BA, and NICE) for an orphan drug that was being submitted for fast-track approval for a rare cardiovascular disease.

PRMA CONSULTING SOLUTION

ESA briefing book

ESA briefing books were prepared for a G-BA consultation meeting and a NICE-CADTH joint scientific advice meeting; this involved:

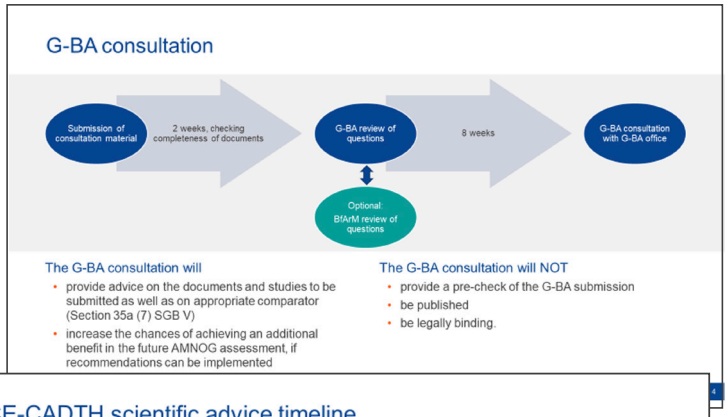
- developing a list of themes and questions to be addressed with the HTA agencies, which were reviewed and discussed at an internal alignment workshop
- developing a draft briefing book, including rationales supporting the client's view on the questions and relevant supporting evidence
- finalizing the briefing book based on client review and feedback.

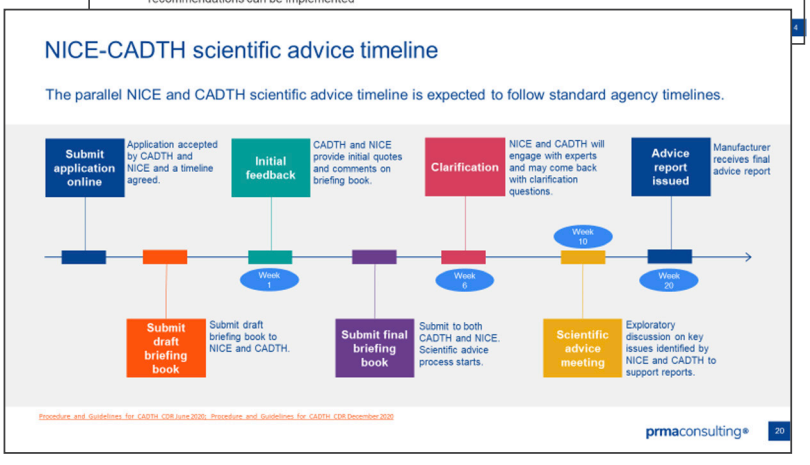
Mock ESA meetings

Materials were shared with expert advisors from Canada, Germany, and the UK for input. Mock ESA meetings involving the client and the advisors were then held to prepare for the final meetings.

CLIENT VALUE

- Based on the evidence package being developed for the product, the client received specific feedback on the target population and subpopulations, appropriate comparators, endpoints, and proposed approach to economic modeling.
- Feedback obtained from the HTA agencies was incorporated into evidence generation plans and the market access strategy.





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