



Early scientific advice support for an orphan drug for a rare cardiovascular disease



SUMMARY

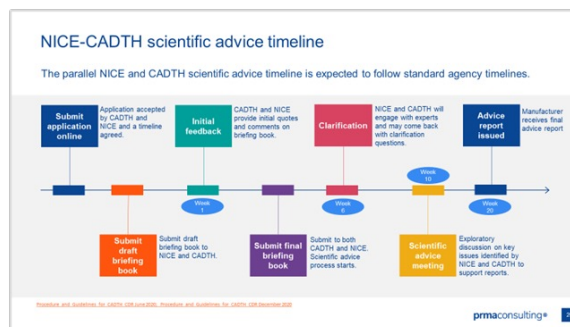
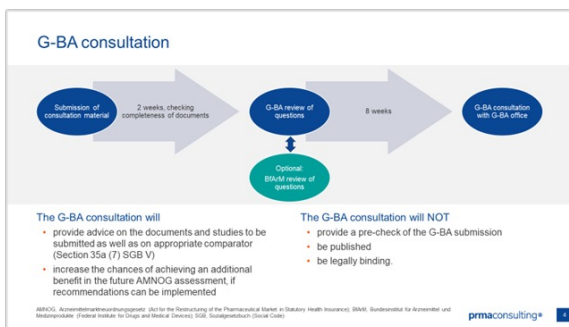
The client gained strategic recommendations, which they incorporated into their evidence generation planning and market access strategy. This was supported by feedback from HTA agencies, and specific insight about the target population and subpopulations, appropriate comparators, endpoints, and proposed approach to economic modeling.

CLIENT SITUATION

A pharmaceutical manufacturer developing products for rare diseases was seeking early scientific advice (ESA) from key 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

- Mock ESA briefing books, mock ESA meetings, and expert recommendations were provided by PRMA Consulting, a member of Fishawack Health.



MOCK ESA briefing book

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

- developing a list of themes and questions to be addressed with the HTA agencies, which were reviewed and discussed at an internal alignment workshop
- drafting a 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.

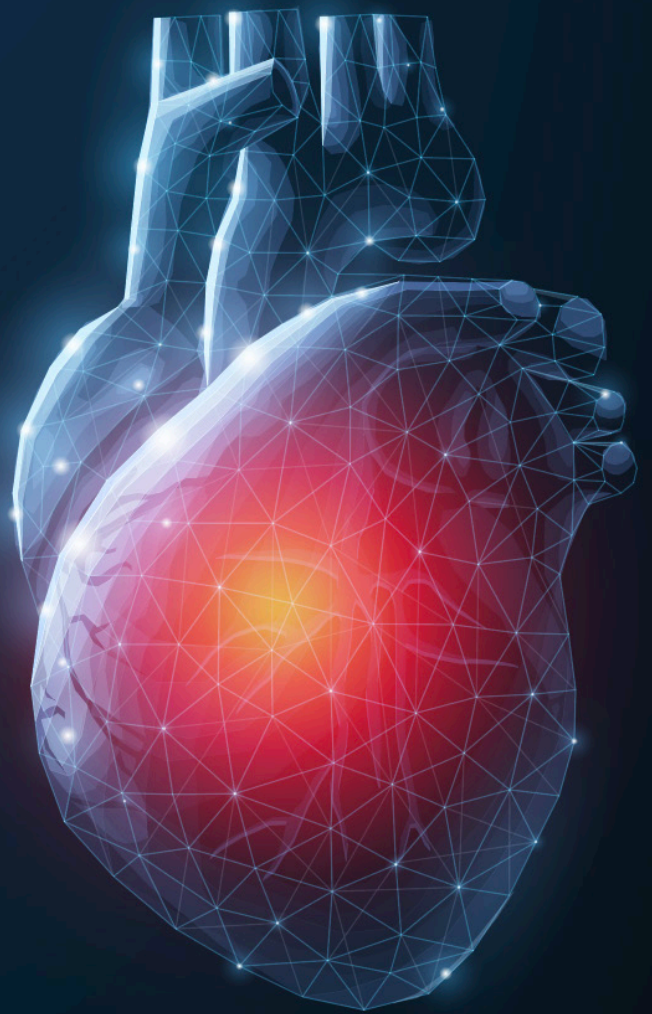
Mock ESA meetings

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

A report with recommendations was developed based on the feedback obtained in the ESA meetings.

- 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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