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ELIQUENT Outcomes

Regulatory Strategy and Success in Enabling Japanese Enrollment in a Global Phase 3 Biopharmaceutical Trial

Objectives

To obtain PMDA endorsement for the implementation of a global Phase 3 Study in Japan and enable Japanese patient enrollment without delay. Specifically, the client aimed to:

- Secure PMDA agreement to submit the Clinical Trial Notification (CTN) for the global registration trial without undergoing a formal consultation on the study design.
- Initiate Japanese subject recruitment immediately to align with the ongoing global Phase 3 study timelines.
- Enable submission of the Japanese New Drug Application (J-NDA) based on interim analysis data from the global study, without conducting a separate Japanese Phase 1 trial.

Background

In early 2024, the client launched two global Phase 3 studies targeting Indication A and Indication B outside Japan, with plans to pursue an accelerated Biologics License Application (BLA) filing in the United States based on interim analysis data. For Japan, the client intended to enroll Japanese patients in these same Phase 3 studies and submit a J-NDA based on the same interim data, bypassing the need for local Phase 1 data.

Typically, PMDA requires a formal consultation to agree on the clinical study design before CTN submission. However, due to the limited time remaining in the global enrollment period, the client needed to begin enrolling Japanese patients immediately to ensure sufficient representation in the global dataset.

Challenges

- **Regulatory Timing Constraints:** The remaining enrollment window for the global Phase 3 studies was too short to accommodate the standard PMDA consultation process before CTN submission.
- **Lack of Local Phase 1 Data:** The client planned to proceed without Japanese Phase 1 data, which is uncommon and required strong justification to PMDA.
- **Urgency of Enrollment:** Delays in Japanese patient enrollment could jeopardize the inclusion of Japan in the global registration strategy.
- **Regulatory Strategy Innovation:** ELIQUENT Japan had to develop a novel regulatory communication approach to justify immediate CTN submission, including:
  - Demonstrating no safety concerns for Japanese patients based on overseas Phase 1 and 2 data.
  - Emphasizing the urgency of the enrollment timeline.
  - Proposing a follow-up PMDA consultation after CTN submission.

Solutions

ELIQUENT Japan developed a regulatory strategy tailored to the urgent need for Japanese patient enrollment in the global Phase 3 program. Recognizing the limited enrollment window and the absence of Japanese Phase 1 data, ELIQUENT Japan proposed a novel approach to PMDA.

A **PMDA Pre-meeting** was conducted to seek immediate submission of the Clinical Trial Notification (CTN) without undergoing the standard formal consultation process. ELIQUENT Japan led this effort by crafting a unique regulatory communication strategy, which included:

- Emphasizing the **tight enrollment timeline,** particularly the risk of missing Japanese patient inclusion due to the short remaining period.
- Demonstrating **no safety concerns** for Japanese patients based on the design and results of overseas Phase 1 and 2 studies.
- Highlighting the plan to **submit the J-CTN immediately,** with a formal PMDA consultation to follow post-submission.

This proactive and strategic engagement with PMDA enabled the client to move forward without delay, aligning Japanese participation with the global trial timeline.

Outcomes

PMDA agreed to the submission of the CTN for the global registration trial **without requiring a formal consultation** on the study design—an exceptional outcome. This decision enabled the **immediate initiation of Japanese subject recruitment,** ensuring Japan's inclusion in the global Phase 3 study.

Notably, this CTN was the **first ever submitted in Japanese for this product,** marking a significant regulatory milestone. The client was able to maintain alignment with global development timelines and is now positioned to pursue a **J-NDA filing based on interim global data,** without the need for local Phase 1 studies.

This case highlights the value of ELIQUENT Japan's regulatory expertise and strategic foresight in navigating complex timelines and requirements to achieve accelerated development goals.