ELIQUENT LIFE SCIENCES

ELIQUENT Outcomes | Accelerating Global Trials in Japan

Objectives

A clinical-stage pharmaceutical company sought agreement from the PMDA to include Japanese patients in a global Phase 3 study of Product for Indication A and Indication B. The objective was to proceed without conducting a Japanese Phase 1 study, thereby supporting a potential simultaneous NDA submission in both Japan and the U.S.

Background

Client is developing Product, a novel biologic for the treatment of Indication A and Indication B. In early 2024, the company initiated two global Phase 3 studies outside Japan and planned to enroll Japanese patients within the same trials. The company aimed to file a Japan NDA based on interim global data, without generating Japanese Phase 1 data—typically required for drug development in Japan. ELIQUENT Japan was engaged to assess feasibility, guide regulatory strategy, and lead engagement with the PMDA.

Challenges

The primary challenge was obtaining PMDA's agreement to enroll Japanese patients in Phase 3 trials and pursue J-NDA submission without Japanese Phase 1 data.

Key challenges included:

- **Regulatory norms:** PMDA commonly expects Phase 1 data in Japanese subjects before participation in later-phase studies.
- **Ethnic sensitivity:** It was critical to demonstrate that safety and efficacy data were applicable to the Japanese population.
- **Timeline risk:** Conducting separate Phase 1 studies could cause delays, jeopardizing alignment with the global development program.

Solutions

Data-Based Regulatory Justification
ELIQUENT Japan worked with Client to develop a justification in accordance with ICH E5 and Japanese guidelines, showing that available clinical and non-clinical data posed no safety concerns and suggested no ethnic differences in efficacy or pharmacokinetics.

Strategic PMDA Consultation Support
In preparation for the PMDA consultation, ELIQUENT clarified the key review points during a pre-meeting and ensured these were explicitly addressed in the formal consultation briefing document. The team presented a clear rationale demonstrating that the planned data package was sufficient to support inclusion of Japanese participants in the Phase 3 program.

Outcomes

Client Outcome: PMDA confirmed that Japanese Phase 1 data were not required for Clinical Trial Notification (CTN) or for the J-NDA application. The development program could proceed as planned, allowing Japan to join the global Phase 3 studies without delay and supporting a potential simultaneous filing in the U.S. and Japan.

Patient Benefit: If development progresses successfully, patients in Japan could gain earlier access to a novel treatment for Indication A and Indication B.

ELIQUENT Benefit: This engagement enhanced ELIQUENT Japan's expertise in navigating regulatory pathways without local Phase 1 data and deepened its understanding of the key considerations for multi-regional studies involving Japan.

ELIQUENT Japan
Guided by decades of regulatory and clinical experience, ELIQUENT Japan partners with innovators to develop a synchronized, global go-to-market approach that unlocks the full potential of the Japanese market.

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