ELIQUENT LIFE SCIENCES

ELIQUENT Outcomes

Regulatory Strategy to Enable Japan's Participation in Global Phase 3 Studies Without Local Phase 1 Data

Objectives

To enable participation in global Phase 3 studies and prepare for simultaneous submissions in Japan and the U.S. without requiring separate Japanese Phase 1 data. Specifically, the client aimed to:

- Obtain PMDA endorsement for participation in global Phase 3 studies for Indication A and Indication B without conducting Phase 1 studies in Japan
- Align the Japanese submission timeline with the U.S. BLA filing based on interim analysis data.
- Avoid delays in clinical development and potential commercialization in Japan.

Background

Since 2023, ELIQUENT Japan has supported a global pharmaceutical company developing a novel therapy. The client launched two Phase 3 studies for Indication A and Indication B outside Japan in early 2024 and planned to seek accelerated approval in the U.S. based on interim data.

To align Japanese regulatory timelines with global development, the client engaged ELIQUENT Japan to pursue a strategy that would allow Japan to join the global trials and proceed with J-NDA submission - all without conducting a local Phase 1 study.

Challenges

Japanese regulatory precedent typically expects local Phase 1 data before enrolling patients in global studies. The client's plan to omit this step introduced several challenges:

- Justifying that existing global non-clinical and clinical data sufficiently support safety and efficacy for Japanese participants.
- Demonstrating no significant ethnic differences that would necessitate a local Phase 1 study.
- Ensuring that the clinical data package — based solely on interim Phase 3 results — would be acceptable for both the CTN (Clinical Trial Notification) and J-NDA filing.

Solutions

ELIQUENT Japan developed and executed a regulatory communication strategy aligned with ICH and Japanese guidelines. The approach focused on removing the need for a Japanese Phase 1 study without compromising regulatory expectations.

- ELIQUENT presented comprehensive non-clinical and early-phase clinical data to PMDA, clearly explaining why no safety or efficacy concerns were anticipated in the Japanese population.
- Scientific rationale was reinforced by referencing established regulatory guidance on ethnic factors, showing that the existing data met requirements for initiating trials in Japan.
- During formal consultation, ELIQUENT led the discussion with PMDA to clarify the sufficiency of the proposed clinical data package.

Outcomes

Through PMDA consultation, the agency confirmed that Japanese Phase 1 data would not be required for participation in the global Phase 3 studies or for the subsequent J-NDA submission.

This regulatory alignment enabled the client to proceed without development delays in Japan and to pursue a simultaneous NDA submission strategy with the U.S. — maximizing efficiency and accelerating access for patients.