ELIQUENT LIFE SCIENCES

ELIQUENT Outcomes

Global Regulatory Strategy and Success in Obtaining a New Indication for a Biopharmaceutical Product

Objectives

To obtain approval of a new indication simultaneously across key global markets, including the United States, European Union, and Japan. Specifically, the client identified the following goals:

- PMDA endorsement for implementation of a global Phase 3 study in Japan and pre-endorsement of PMDA that the trial is categorized as the registration trial of the Japan CTD filing.
- PMDA endorsement for submission of the Japanese registration filing once the global Phase 3 study is completed to seek the chance of global simultaneous approval.
- Obtain approval of the new indication for Japanese commercialization without delay from the global approval.

Background

Since 2018, ELIQUENT Japan has provided regulatory services for a global immunology company engaged in the development of therapies for the treatment of autoimmune diseases in the United States, Japan, Europe, Middle East, Africa, and China.

After developing and receiving approval for an innovative biologic therapy targeting autoimmune diseases, the company sought to expand the therapy's indication to include another related condition with a high unmet medical need. To achieve this goal, the company partnered with ELIQUENT Japan for regulatory support and global pathway capabilities.

Challenges

Each global health authority has a distinct regulatory requirements and review processes. Coordinating submissions, responding to regulatory agencies, and ensuring that all data packages met the stringent requirements of each region posed significant challenges.

A significant barrier was the proposed PMDA Pre-NDA meeting to pursue J-NDA filing based on a single registration trial in Japan, contingent on the availability of topline results from the global Phase 3 study. This approach posed a challenge, as the US-FDA did not endorse submitting the US-NDA with just one registration trial. Despite this, the client agreed that Japan would be the first region to hold the pre-NDA regulatory meeting.

Solutions

ELIQUENT Japan developed a global regulatory strategy tailored to the specific requirements of each targeted region. This strategy included identifying key milestones, aligning clinical endpoints with regulatory expectations, and designing a unified dossier that could be adapted for regional differences.

The ELIQUENT team played a crucial role in preparing the client for interactions with regulatory agencies. Before the start of the Japanese regulatory communication, the client had the End of Ph2 US-FDA meeting (EOP2-meeting), and the US-FDA agreed to implement the global Phase 3 study. However, the US-FDA did not agree to submit a US-NDA with only one Phase 3 trial because the data from a single Phase 3 study may not be substantial enough to start the NDA review in the US-FDA.

Eliquent Japan implemented the PMDA EOP2 formal consultation meeting process after the US-EOP2 meeting, and PMDA agreed to implement the global Phase 3 study in Japan. At the PMDA EOP2-meeting, PMDA hesitated to give a formal commitment to categorize the global Phase 3 as the registration trial for J-NDA, but unlike the US-FDA, PMDA agreed to continue discussing the J-NDA plan with this study once the study is completed.

Eliquent Japan implemented the PMDA pre-NDA formal consultation meeting process when the global Phase 3 study was completed. We successfully obtained the endorsement of PMDA to file J-NDA with the orphan drug priority review, and it was followed by J-NDA submission.

Outcomes

The client successfully obtained the first global approval for the new indication – a significant milestone that will allow expedited entry to multiple markets and enable the company to meet the needs of global patients.

This case study underscores the importance of a well-coordinated global regulatory strategy. By leveraging ELIQUENT Japan's deep regulatory knowledge and strategic approach, the client maximized the commercial and therapeutic impact of their innovative biologic, and was able to bring an important new treatment option to patients around the world.