Worldwide Clinical Trials | Oncology

Case Study

Accelerating Approval Pathway For Phase III Ovarian Cancer Trial

A late-stage biotech was developing a combination therapy for a rare form of ovarian cancer. It is challenging to diagnose, difficult to treat due to its chemo-resistance, and at the time, there were no approved treatments specifically for this indication.

With a successful phase II trial nearing completion, the biotech was planning an accelerated approval application with the U.S. FDA. As part of the requirements for an Accelerated Approval, the biotech needed to have a confirmatory trial underway. The biotech was looking for a new global partner to initiate and conduct the confirmatory trial and was referred to Worldwide Oncology.

At a Glance

Goals

- Launch confirmatory trial
- Accelerate global startup
- Improve enrollment

Benefits

- Faster trial initiation
- Data-driven site selection
- Met NDA milestones

Challenges

Our Worldwide team was tasked with several challenging asks during this study, including:

The small biotech selected Worldwide to manage their global phase III confirmatory trial in support of an Accelerated Approval application.

Startup speed was critical, as the confirmatory study was part of the company's post-marketing requirements and was linked to a corporate milestone for NDA submission. Recruitment for the trial would be challenging due to numerous factors, most notably the rare nature of the indication (only 2-5% of all ovarian cancers). With an anticipated enrollment rate of <0.1 patients/site/year, a global footprint of 100+ sites would be required to complete enrollment within 2 years, along with support from regional cooperative groups like the Gynecologic Oncology Group (GOG) Foundation and the European Network of Gynaecological Oncological Trial Groups (ENGOT). Additionally, because the cancer can be difficult to diagnose and awareness of the disease is limited, patient education and community site engagement would be needed.

A final challenge was aligning the timing of rest of world startup and enrollment as it was anticipated that U.S. patient participation would drop off upon FDA conditional approval.

Solutions

To combat the challenges faced in this trial, Worldwide implemented several solutions:

To address the urgency around trial initiation, Worldwide rapidly assigned a seasoned study team with deep experience managing ovarian cancer studies. Services included global project management, regulatory, site start-up, and clinical monitoring, as well as clinical science support to review patient-level safety and efficacy data. The sponsor retained their global safety provider and separately outsourced data management and biostatistics, in line with their corporate outsourcing model that had been followed for other trials. Though not part of Worldwide's scope, we needed to communicate and work collaboratively with these partners to deliver a global study.

To proactively address anticipated recruitment challenges and ensure enrollment goals would be met within a defined timeline, Worldwide applied a data-driven approach to country selection and enrollment rate modeling. We aligned with the sponsor's recent experience and planned to activate nearly 100 seasoned gynecologic oncology sites across 17 countries, including the U.S., Western and Eastern Europe, and Asia Pacific. We collaborated with cooperative groups like the GOG and ENGOT to support site selection and trial enthusiasm and leaned into the sponsor's efforts to build patient awareness about this rare disease.

Additional Challenge

An unexpected shift in FDA expectations created an urgent new timeline challenge:

In this role, Worldwide demonstrated and leaned heavily upon its core values of listening, flexibility and commitment when the sponsor's interpretation of the FDA's "underway" requirement for a confirmatory trial shifted, necessitating an immediate acceleration of timelines. The regulatory guidance on this topic was so vague that the FDA eventually released a draft guidance titled: "Accelerated Approval and Considerations for Determining Whether a Confirmatory Trial is Underway." With only 10 months to meet this goal, the Worldwide team jumped on a plane for an in-person solution session, modeled how to meet the earlier enrollment milestone, and aligned on additional countries, sites and recruitment measures that could enable success.

Outcomes

Worldwide achieved several impactful outcomes throughout this trial, including:

Worldwide and the sponsor collectively met both the end-of-year goal for initiation of the confirmatory trial and the expedited startup and enrollment milestones needed as part of the NDA submission less than a year later. The NDA submission was approved by the FDA and the confirmatory trial that was initiated is part of the company's post-marketing requirements as detailed by the FDA. We remain hopeful that patients will continue to have access to the first ever FDA-approved treatment specifically for this indication, and that the confirmatory trial will validate a benefit to patient outcomes in support of full approval of the therapy.

Worldwide Clinical Trials

About Worldwide Clinical Trials

Worldwide Clinical Trials (Worldwide) is a global CRO serving development-driven biopharmaceutical companies, with more than 4,400 professionals operating across more than 70 countries. The company delivers therapeutically dedicated expertise in neuroscience, oncology, rare disease, and internal medicine, with comprehensive support across every development phase – from early-stage and first-in-human studies through Phase III registration trials.

The company's flexible service model – spanning full-service trial management to functional service partnerships through Worldwide Flex – is powered by a peoplefirst, partnershipdriven outsourcing approach that strengthens collaboration, enhances data transparency, and supports more informed decisionmaking. This provides sponsors with tailored, adaptable solutions that keep pace with the evolving demands of clinical research.

Learn more at www.worldwide.com