



Case Study

Successful Partnership Advances AML Program to Phase II/III

A late-stage oncology biotech selected Worldwide Clinical Trials to manage its Phase I/II European trial evaluating a promising acute myeloid leukemia (AML) therapy. The award was driven by the sponsor's positive experience partnering with Worldwide on a previous solid tumor study. Following the success of the Phase I/II program, the sponsor expanded the relationship and non-competitively awarded Worldwide the registrational global Phase II/III trial.

At a Glance

Indication	Acute Myeloid Leukemia (AML)
Phase I/II Study	22 patients across 9 sites in Italy and Poland
Global Expansion	300+ patients across 50+ sites in 18+ countries for the registrational Phase II/III trial
Result	Successful delivery of the Phase I/II study led to a non-competitive award of the global Phase II/III program



Challenge

A well-established class of AML therapies demonstrated strong efficacy in inducing remission, but its broader utility had been limited by concerns around cardiotoxicity. Our client was developing a next-generation therapy designed to address these safety limitations while maintaining the drug class's antitumor activity.

Given the historical safety concerns associated with this drug class, the FDA did not support conducting the Phase I/II study in the U.S., requiring proof-of-concept evaluation to be performed elsewhere. To advance development, the sponsor needed an experienced oncology partner capable of designing and executing a complex Phase I/II leukemia trial that would meet European regulatory expectations and generate the safety and efficacy data necessary to support future registrational development.



Solution

Worldwide was initially engaged to provide limited back-end Phase I/II study services, including biometrics, medical monitoring, safety management, and medical writing. However, as the study progressed, front-end operational challenges began to threaten key timelines and overall trial execution. Site engagement declined, critical study documentation was delayed, and enrollment momentum slowed, placing the program at risk.

In response to the challenges, the sponsor asked Worldwide to expand its role and assume full scope responsibility, including site startup, site management, clinical monitoring, patient enrollment support, and project management activities.

Leveraging deep hematology/oncology expertise, established relationships with European investigative sites, and a proactive site management approach, Worldwide strengthened study oversight, re-engaged key stakeholders, and worked closely with the sponsor and sites to address operational barriers. These efforts helped to restore study momentum, improve execution, and generate the data needed to advance the program.



Outcome

Worldwide managed 9 sites across Italy and Poland, enrolling 22 patients in the Phase I/II study. The trial successfully demonstrated no observed cardiotoxicity, providing critical proof of concept for the sponsor's next-generation approach and validating its ability to harness the therapeutic benefits of this drug class while addressing historical safety concerns. The resulting safety and efficacy data supported regulatory alignment with both the FDA and EMA, enabling advancement into a global registrational Phase II/III trial.

In recognition of Worldwide's successful delivery, operational leadership, and collaborative partnership, the sponsor non-competitively awarded the Phase II/III study to Worldwide. The global trial is expected to enroll more than 300 patients across 50+ sites in 18+ countries, including the U.S.



About Worldwide Clinical Trials

Worldwide Clinical Trials (Worldwide) is a global CRO serving development-driven biopharmaceutical companies, with more than 4,000 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.

Worldwide's flexible service model, Worldwide Flex, is supported by purpose-built technology that enhances data transparency, collaboration, and informed decision-making – offering sponsors tailored and adaptable solutions to meet the changing needs of clinical research.

Learn more at www.worldwide.com.