VERISTAT

Gaining Regulatory Approval Following a Single-Arm Phase I/II Study

Regulatory Submission Strategy and Novel Efficacy Endpoint for Treatment of an Ultra-Rare and Aggressive Hematologic Malignancy

A small biotech company engaged Veristat early in clinical development for help with a novel biologic being tested for treatment of an ultra-rare and aggressive hematologic malignancy with no available effective therapies. Using the results of a single-arm Phase I/II study, Veristat and the sponsor company collaborated to present a thorough, well-planned strategy for demonstrating the benefits and risks of the targeted therapy in this patient population to regulatory authorities. Based on Veristat's significant experience, the sponsor engaged our team for strategic consulting, statistical and programmatic support, and medical writing to plan and execute the marketing application process, eventually leading to both FDA and EMA approval.

Study Demographics

Indication:
Hematologic malignancy

Single-arm study

Primary Services Provided:

- Strategic Consulting
- Biostatistics
- Programming
- Project Management
- Medical Writing
- Data Management

SOLUTION

Leveraging our specialized oncology experience

The novel biologic product was tested in a single-arm study due to the ultra-rarity of the condition and that there were no other approved treatment options. Treatments for other hematologic malignancies were used but did not provide durable responses and were often challenging to administer due to toxicity in elderly patients which make up the majority of cases of this rare cancer. After initial dose escalation and an expansion cohort, it became clear that the novel product

led to high response rates, including complete responses, that were durable beyond what was expected based on the literature. In this rare disease, the impact of an improved response rate and a more durable response could make significant differences in the patient's quality of life. Veristat was first engaged to provide strategic support for the clinical program, including support of a breakthrough therapy designation and other interactions with the FDA.

Upon completion of the expansion cohort within the targeted hematologic disease, Veristat and the sponsor met with the FDA to discuss favorable study results and the plan for the regulatory filing. The FDA recommended including a prospectively designed confirmatory cohort to the study to support the regulatory submission. Veristat provided statistical support on the design and analysis of the confirmatory cohort, which was accepted by the agency. Following successful completion of the confirmatory cohort, Veristat provided statistical, programmatic, and medical writing support for the clinical study, integrated analyses, and the marketing application.

Nuanced response criteria and a novel efficacy endpoint

In this hematologic indication, the response criteria require evaluation of multiple disease compartments, including blood and bone marrow measurements, skin assessments and radiological and physical examination of other organs. While resolution of one or more of the diseased compartments may be meaningful, to achieve a complete response, all compartments were required to be disease-free.

Through a prospectively planned assessment of the disease responses observed during the dose escalation and expansion phases of the Phase I/II study, Veristat evaluated the similarity in duration of response among a set of near-complete response categorizations. Through this effort, Veristat and the sponsor were able to effectively demonstrate that the impact of the novel therapy on duration of response was similar for patients who met all criteria for a complete response, with the exception of some residual skin disease and those that achieved complete response with complete clearance of skin disease. This novel endpoint was included as the primary efficacy endpoint for the marketing application and eventual approval of the biologic product within the US and EU.

IMPACT

The biologic treatment has been on the market in the United States since FDA approval 2018 and in Europe since 2021. Veristat is continuing to work with the study sponsor on the use of this treatment for other indications.

ABOUT VERISTAT

CRO of choice for cell and gene therapies

Veristat has assembled a scientific team of experts who are adept at strategy and execution across the clinical development journey in this specialized area. Whatever the study's unique considerations – patients, products, process, follow-up, regulatory – Veristat can help you successfully get through it.

Contact Veristat Today

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