VERISTAT

Understanding and Operationalizing a Complex Adaptive Design

Accelerating a Go/No-Go Decision for a Promising Oncology Vaccine in a Specific Patient Population

Background

A clinical-stage biopharmaceutical company developing cancer vaccines approached Veristat with a complex statistical methodology for a pivotal Phase II adaptive design trial, which had been originally provided by another consultant. Their objective was to expedite a go/no-go decision for a biomarker they had identified in a rare lung cancer, which they believed could predict increased sensitivity to their product. The sponsor aimed to determine quickly whether the treatment showed a significant effect in both the overall patient population and the biomarker-positive subgroup. The trial was designed to run across 65 sites in North America, Europe, and Japan.

Situation

The sponsor faced two main challenges:

- They did not understand the complex adaptive design methodology well enough to explain it to their senior management team and investors.
- They could not determine how to operationalize the statistical method into a workable trial design.

Veristat needed to review the design, explain it to the sponsor's leadership and investors, and then communicate it in practical terms to regulatory agencies, project teams, vendors, and study sites so that the trial could begin.

Solutions

Review & Revise the Adaptive Methodology

Veristat reviewed and revised the statistical methodology to align with the sponsor's study goals. We developed the study protocol and statistical analysis plan based on the complex adaptive methodology.

STUDY DEMOGRAPHICS

INDICATION
- Mesothelioma

STUDY PHASE
- Phase II Pivotal Study

SITES
- 65

PATIENTS
- >300

REGIONS
- 14 countries across North America, Europe & Japan

SERVICES PROVIDED
- Strategic Consulting
- Data Management (EDC)
- Biostatistics & Programming
- DSMB
- CDISC
- Medical Writing
- Project Management

Explain the Trial Design
Our biostatistics team then explained the methodology and protocol to the sponsor's senior management team and investors, enabling them to fully understand the study and its adaptive population enrichment design.

Once the sponsor was aligned, Veristat engaged with the FDA and EMA to explain and justify the design. Our lead biostatistician also traveled to Japan for a face-to-face meeting with the PMDA. After extensive discussions, all three agencies approved the design.

Finally, Veristat trained study sites on how to implement the adaptive design and prepared them for the possible outcomes of each interim analysis:

1. Continue enrolling both biomarker (+) and biomarker (–) patients
2. Continue enrolling biomarker (+) patients only
3. Stop for futility

Implement the Population Enrichment Study Design
With regulatory approvals in place, our project teams launched the study. The design was implemented successfully and the trial ran smoothly through the first interim analysis.

Impact

The adaptive enrichment design enabled a rapid decision, saving time and resources while guiding the sponsor's next steps.

- At the first interim analysis, the DSMB recommended stopping the study for futility in both the overall and biomarker (+) populations.
- Although the drug did not show a treatment effect, the trial met its core objective: quickly reaching a go/no-go decision.
- The sponsor was able to re-allocate resources to pursue development in other cancers and combination therapies.
- Veristat's ability to design, communicate, and operationalize the adaptive methodology ensured smooth execution across regulators, sites, and study teams.

Meet Veristat

Full-Service CRO and Consultancy That Accelerates Success

If you've struggled with missed deadlines and slow progress, talk to Veristat. We have a proven track record of starting—and accelerating—clinical development programs with the expertise to navigate the challenging path to regulatory submission and approval efficiently. Collaborate with experts who understand the value of speed without compromising quality.
It's not just business for Veristat, it's personal.

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