CASE STUDY

VERISTAT

# Targeted Indication Selection Using Simon 2-Stage Design

How Veristat Helped a Biotech Sponsor Prioritize Oncology Indications Through Efficient, Multi-Cohort Trial Design

## Background

A biotechnology company exploring the potential of a novel oncology therapy engaged Veristat to design and manage a Phase 2 study that would evaluate multiple tumor-specific cohorts. The objective: to identify the most promising indications for further development by using a statistically planned, resource-efficient approach. Veristat implemented a multi-cohort Simon 2-stage design to allow early termination for futility or continuation of cohort enrollment based on pre-specified response thresholds.

## Sponsor Challenge

### Selecting Indications with High Likelihood of Success and Managing Real-Time Cohort Decisions

With limited clinical data across tumor types, the sponsor aimed to identify which indications showed the most promising early signs of efficacy within a Phase 2 study. Rather than launching multiple independent studies—which would require more patients, extended timelines, and higher financial investment—they pursued a more efficient strategy. A single, multi-cohort study with a consistent schedule of events, a defined dose optimization plan, and aligned efficacy evaluation timing offered a streamlined approach to explore multiple indications in parallel.

To fully realize the potential of this design, the study required robust coordination of ongoing data collection and well-planned interim analyses across cohorts. Efficient, timely decision-making would be critical to maintaining trial momentum and making the most of available resources. Establishing clearly defined hypotheses and decision criteria from the outset was key to enabling meaningful interpretation of early signals and ensuring the study could adapt effectively as data emerged.

## STUDY DEMOGRAPHICS

**STUDY PHASE**
- Phase 2, single-arm, multi-cohort

**INDICATION**
- Advanced/metastatic tumors (multiple cohorts)

**THERAPY TYPE**
- Novel biologic

**PRIMARY ENDPOINT**
- Objective Response Rate (ORR) by cohort

**SECONDARY ENDPOINTS**
- Progression-Free Survival (PFS), Duration of Response (DoR)

**SERVICES PROVIDED**
- Protocol and Statistical Design
- Cohort Prioritization Strategy
- Real-Time Data Monitoring
- Regulatory Strategy and Communications

## Veristat Solution

Veristat designed a multi-cohort Simon 2-stage study, allowing each tumor cohort to be independently recruited pre-determined sample sizes to be assessed for early signs of efficacy. Using customized null and alternative hypotheses for each indication based on standard of care response rates and published findings from other studies, Veristat defined tailored decision thresholds. If a minimum number of responses (R1) were observed in Stage 1 from the pre-specified Stage 1 sample size (N1), the cohort advanced to Stage 2 for further enrolment; otherwise, it was closed for futility. To support real-time decision making and manage the complexity of interim analyses and dose exploration, Veristat implemented a centralized cohort monitoring plan working towards real-time data access for review. Predefined stopping rules enabled rapid cohort-level decisions to be made efficiently, while exploratory analyses of multiple dose levels provided early insight into therapeutic windows, further informing development strategy. This efficient and adaptive approach ensured minimal recruitment delays and enabled fast, data-driven decision-making.

## Impact

- Enabled early identification of promising indications within targeted dose levels, advancing only high-potential cohorts
- Avoided unnecessary patient exposure in tumor types showing low response and non-therapeutic dose levels
- Reduced trial complexity and cost by using a single protocol across tumor types
- Facilitated real-time decision-making for dose and cohort evaluation
- Positioned the sponsor for a focused, data-driven approach to clinical development expansion
- Veristat continues to support the sponsor with regulatory interactions and strategic planning for the next phase of development.

## THE ADAPTIVE ADVANTAGE

Adaptive designs can offer:

- Reduced time and cost
- Improved patient safety
- Minimized exposure to ineffective treatments
- Enable faster, data-driven decisions

## 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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