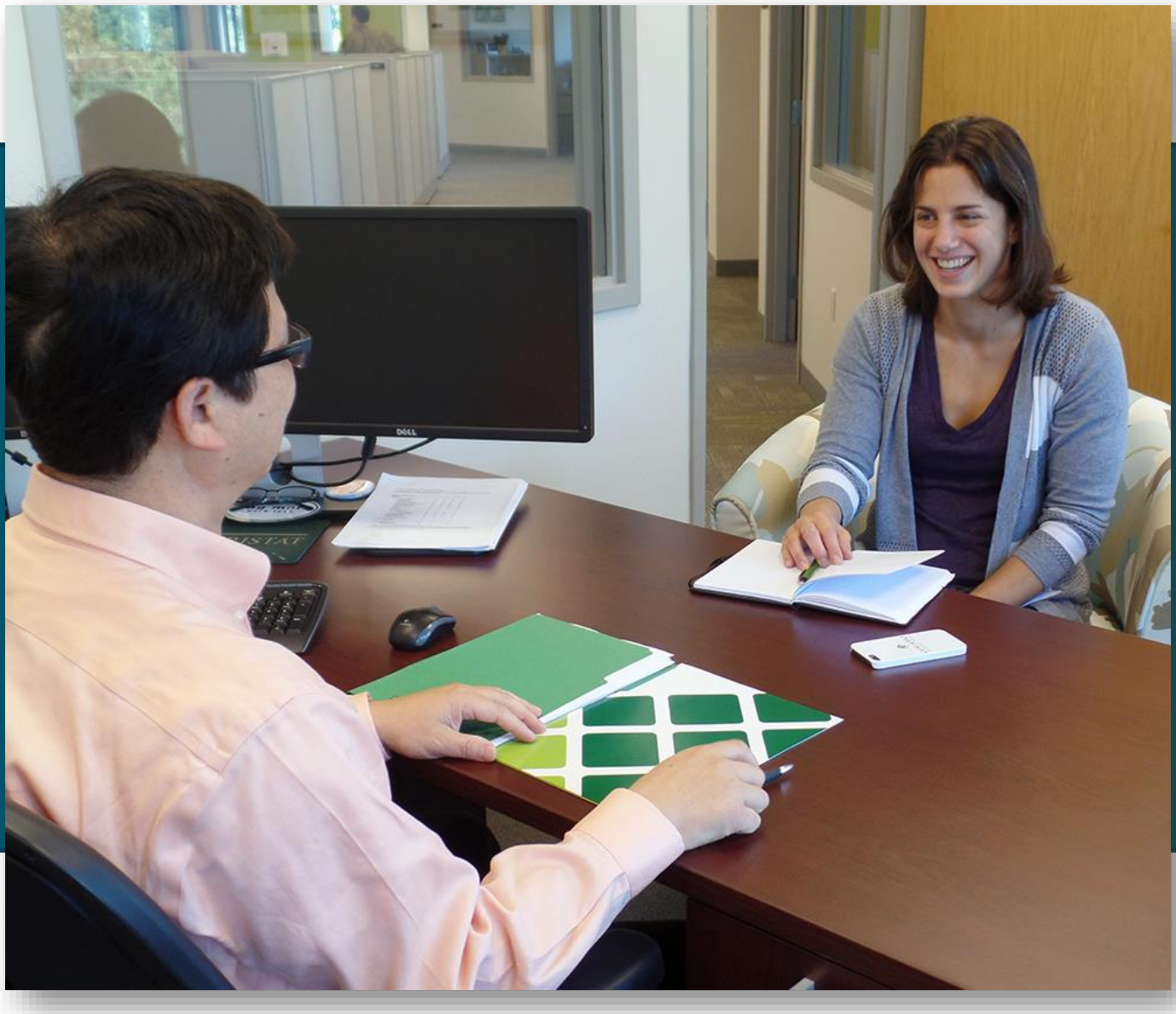
VERISTAT

UNDERSTANDING & OPERATIONALIZING A COMPLEX ADAPTIVE DESIGN

Accelerating a Go/No-Go Decision



A Veristat Adaptive Trial Case Study

UNDERSTANDING & OPERATIONALIZING A COMPLEX ADAPTIVE DESIGN

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Background

A clinical-stage biopharmaceutical company who develops drugs to treat cancers came to Veristat with a complex statistical methodology for running a phase II adaptive design trial with the goal of expediting a go/no-go decision for a specific patient population. The sponsor's challenge was that they didn't understand the complex adaptive design methodology enough to explain it to their senior management team and investors, nor could they determine how to operationalize the methodology into a trial design. They partnered with Veristat to review the methodology, explain it to their teams and investors, and to put it into an explicable adaptive trial design that the regulatory agencies, sites, project teams and clinical study vendors could all understand and implement. The adaptive enrichment design trial would need to run across sites throughout North America, Europe and Japan.

Study Demographics

INDICATION:
Mesothelioma

STUDY PHASE:
Phase II Pivotal Study

PRIMARY SERVICES PROVIDED:
Data Management (EDC), Biostatistics & Programming, DSMB, CDISC, Medical Writing and Project Management

65 Sites

> 300 patients

14 Countries
Throughout North America, Europe & Japan

GOAL

The sponsor had identified a biomarker in lung cancer that they hoped could predict an increased sensitivity to their product, so they wanted to quickly determine if there was a significant treatment effect in the overall patient population and in the biomarker positive patient population. They came to Veristat with a complex adaptive methodology provided to them by an adaptive design consultant. Veristat needed to explain the study design to their client, their client's investors, the US Food & Drug Administration (FDA), the European Medicines Agency (EMA), the Japan Pharmaceuticals and Medical Devices Agency (PMDA), to the project team and finally to the study sites in order for the study to begin.

SOLUTION

Reviewed & Revised 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 adaptive methodology.

Explained the Trial Design

Next, Veristat's biostatistics team described the methodology and complex protocol to the sponsor's senior management team and investors so that they could better understand the study and how it was being implemented as an adaptive population enrichment design.

Once the sponsor understood the trial design, Veristat went to the FDA and the EMA to explain and defend the design. Our lead biostatistician even flew to Japan to sit with the Japan PMDA to explain and defend the adaptive design. After extensive discussions with the regulatory agencies, the FDA, EMA and PMDA approved the study design to begin within their respective countries.

Lastly, Veristat needed to train the sites on how to implement the adaptive design. The sites needed to understand what could change after each interim analysis, since the following adaptations were possible: **1)** *the study could continue enrolling both biomarker (+) and biomarker (-) patients,* or **2)** *the study could continue enrolling biomarker (+) patients only,* or **3)** *the study could stop for futility*.

RESULT

Implemented The Study Design

Due to the efforts of the Veristat biostatistics team and their ability to explain the study to the FDA, EMA and Japan PMDA, the study received approval to start in the US, Europe, and Japan. Our project teams implemented the design and the study ran smoothly until the first interim analysis.

Reached a No-Go Decision

At the interim analysis, the data lead to the sponsor to stop the study for futility in the study sample overall and in the biomarker (+) patient sample. Despite the study drug failing to show treatment effect for the specific biomarker patient population, the study achieved its goal of getting to a decision quickly. The company is still developing and testing the product for other types of cancer and has re-allocated its resources to pursue those studies for the product. The project's key to success was the Veristat team successfully designed, communicated and implemented the adaptive methodology to all the clinical development stakeholders.

Contact Veristat Today

To learn more about adaptive designs or to explore whether an adaptive design would be beneficial for your trial or development program, reach out to us today.

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