› CASE STUDY

VERISTAT

Preparing Data for the Marketing Authorization Application (MAA) of a Recently Acquired Product

Utilizing a Single Database for Multiple Trials & Standardizing Data by Study

Background

A mid-sized biopharmaceutical company dedicated to developing innovative therapies for patients with debilitating rare diseases acquired the rights to a new investigational drug from a large pharmaceutical company with the intention of preparing the data for a Marketing Authorization Application (MAA).

THERAPEUTIC AREA

- A rare genetic metabolic disorder

PRIMARY SERVICES PROVIDED

- Clinical Monitoring
- Data Management (EDC)
- Biostatistics & Programming
- CDISC Data Conversion
- Medical Writing
- Related Project Management

SITUATION

Veristat had been working with this sponsor on another clinical program and they asked us for help in reviewing and analyzing the study data, as they really didn't know what they had been given or even what the status of the clinical program was. Our client had a very aggressive deadline of wanting to begin the study in January of that year, and having the regulatory submission modules ready for publishing by the end of October that same year.

Goal

Our client's goal was to get the study data cleaned, analyzed, and converted into submission-ready format within ten months, from start to finish.

CHALLENGES & SOLUTIONS

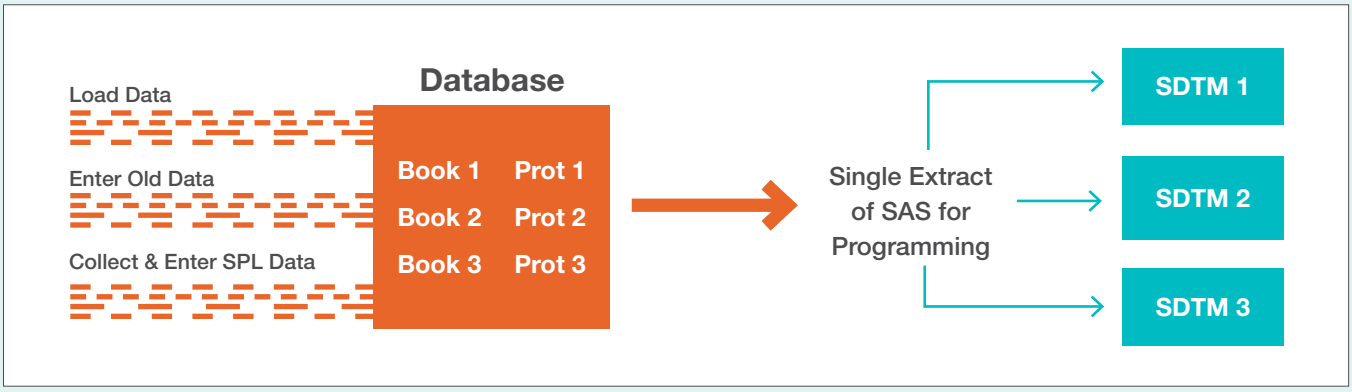
Veristat's integrated team quickly began reviewing and analyzing the SAS data sets and determined that the program had three "ongoing studies," all of which had stopped. Therefore, our client had to complete those studies. We needed to offer a solution that enabled our client to restart the three studies quickly and cost-effectively so they could clean and analyze the existing data, and enter the final data from the ongoing studies as well. By putting all the data into a database, we could make the Study Data Tabulation Model (SDTM) conversion process more efficient, thereby saving time and money.

Three Studies in One Database

The first challenge was to combine the SAS data sets and enter the data from the three separate ongoing studies. Our data management team suggested the creation of one database for all three studies instead of creating a separate database for each study. This was a new solution that we had never implemented before, but it would be far more cost-effective and efficient for our client. Any time saved in preparing a marketing application is significant.

A New Version of EDC System

We built the study database with an Electronic Data Capture (EDC) system and vendor that our team had worked with on many previous studies. However, the uploading of legacy data into the database required the use of an updated version of the uploading tool. We were the first partner to implement the functionality of this tool. Because of this, once the data was uploaded, we had our data managers take on more of the quality control (QC) work to make sure the data loaded correctly. At times, our vendor slowed down the process, so we took on more in order to improve and keep to our client's aggressive timelines. Any delays entering and cleaning the data would cause SDTM and writing delays downstream.



> *We reduced our client's database development charges by 50% by putting the data from all three programs into one database.*

Katelyn LaBombard, *Manager, Data Management, Veristat*

A High Volume of Outputs

This program produced an extremely high volume of outputs for its size. For the ongoing studies there were over 1,200 outputs, and the Integrated Summary of Safety (ISS) alone included an additional 150 outputs. We also completed the ISS for five legacy studies where we received the raw data, and then converted it to SDTM, Analysis Data Model (ADAM) and ISS Tables, Listing and Figures (TLFs).

The volume of outputs was challenging, because every time there was a change, we had to re-run and re-number the outputs. We mobilized a large project team to handle the outputs. The project team was cross-functional – biostatisticians, programmers, and medical writers – working together to ensure medical writers could start on drafts, then receive updated outputs to finalize the drafts. With the constant updating of outputs, it was critical that our biostatisticians and programmers review and quality control the updated and final draft outputs for the marketing application.

RESULT

We achieved our client's aggressive timeline! Veristat teams provided all the data in submission-ready format and all the clinical modules of the MAA by the end of October, just as we committed. It wasn't easy. Our success required a huge amount of cross-functional communication, collaboration, trust and teamwork between the Veristat and client-integrated project teams.

Our client achieved an on-time submission of their MAA to the European Medicines Agency (EMA). The MAA was accepted for review. Due to its orphan drug designation status, it was approved by the EMA in the middle of the following year to treat two debilitating rare genetic metabolic disorders.