eClinical
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Case Study

Biometrics Services

Accelerating clinical trials for rare diseases



The business challenge: Faster data review needed to meet urgent therapy demand

With precision medicine, there is a unique sense of urgency to have patient data quickly available through the duration of a trial. This is especially the case for Sarepta Therapeutics, which develops therapies for a patient population with urgent unmet medical needs. Duchenne Muscular Dystrophy (DMD) is a genetic disorder characterized by progressive muscle degeneration and weakness and primarily affects children.

Duchenne studies are often long, following patients' disease progression over many years. Each patient has a high volume or "avalanche" of data that is collected over the course of the treatment. Given that this is a very vulnerable patient population, it is critical for Sarepta to move quickly and rapidly analyze data to assess the safety and efficacy of its treatments. It is a common requirement from the medical team for data to be turned around within a week of a participant's last appointment. Since the company focuses primarily on DMD, they frequently run similar studies and draw data from across these studies to build comprehensive views of patients' experiences.

In order to manage these trials effectively, Sarepta needed to work with technology and service partners who understood this sense of urgency and could meet their many data demands. This included the ability to create standard, reusable libraries for case report forms and edit checks that could be used across trials to save time during setup and to reduce build costs. To do this, Sarepta chose to have one place, in the form of a Rave EDC url, for all Sarepta studies, as this provided greater efficiencies and standardization. The team also required technology platforms that would allow them to access and review data from numerous sources in a centralized, precise and clean version of data that all stakeholders could work from for data review, exploration, and visualization.

SAREPTA THERAPEUTICS

- Headquartered in Cambridge, MA
- Specializes in gene therapies for Duchenne Muscular Distrophy
- Aims to engineer precision genetic medicine for rare diseases

elluminate®

Products Utilized

→ Biometrics Services

→ elluminate Clinical Data Cloud

Success highlights

1. Rapid data cleaning and faster time to database lock
2. Instituted soft lock process and continuous data review: 95% of data is always reviewed
3. Optimized db builds with 93% reuse of forms and edit checks

The solution: data-centered partnership with excellent communication

eClinical Solutions and Rave EDC are trusted partners with a close collaboration that spans more than 12 years. Together, eClinical and Medidata help clients regain control over their data assets while increasing their ability to conduct comprehensive oversight and review activities across all data sources for faster decision making and time to analysis.

> "Having a data management-specific service provider that has standardized on the Medidata Rave technology has been a benefit to the data management organization and to the company because they are experts on the data and the Medidata Rave clinical cloud."

More than five years ago, Sarepta selected Rave EDC for its data collection and eClinical Solutions as a biometrics services provider for one study. The eClinical team configured Rave EDC for a Duchenne study and was also responsible for all data management activities. As their relationship grew, the partnership expanded to include programs of studies, additional Medidata solutions and the elluminate® Clinical Data Cloud. Along the way the team has developed a close collaboration to enable a very efficient data management process to help the company meet its goal of curing Duchenne.

Sarepta's confidence in and ability to extract maximum value from the products and services that Medidata and eClinical provide together was built on a solid foundation of direct and frequent communication across the teams. The Sarepta team chose a data services provider model giving them the in-depth expertise on the Medidata suite of products along with data management expertise to handle the high volume of longitudinal data and the continuous cleaning needs of the Duchenne population. As a result, data management has greater visibility and control over data with the combination of Medidata and the elluminate Clinical Data Cloud. This hub represents one place that ingests data from all sources and is closely integrated with Rave EDC along with patient-focused data streams, labs, biomarkers, and other data sources that are included in the trial. elluminate also provides Sarepta with centralized analytics that supports clinical, medical, and safety review. In this model, the clinical team has the ability to select the best possible clinical partner that has experience with the Duchenne indication and represents thought leaders and sites across the globe.

Increasing data visibility & standardization

As Sarepta upgrades to use the newest version of Rave EDC, leveraging the data integration capabilities of elluminate will be essential. With elluminate and Rave EDC, all legacy study data are available, enabling cross-study views and analysis, which is essential for this patient population. The eClinical team is also comprised of experts in Rave EDC's global library functionalities, where organizations can configure templates for any system and have instant and traceable deployment to new or ongoing studies.

By partnering with both eClinical and Medidata, Sarepta has increased the standardization of its trial designs and streamlined its data collection strategy. The benefits to Sarepta have included faster study starts and the ability for the Sarepta team to review information immediately with a focus on the most critical data needed for analysis. At Sarepta, trials move quickly, and the ability to accommodate protocol amendments rapidly is critical. The CRF designs and edit checks must be built with this in mind. Today, after collaborating on 20 trials, 95% of forms and 50% of edit checks are reusable, which facilitates rapid study startup.

Moving forward: quicker trials and data review

Given the nature of rare diseases, real-time analysis of data that is well-cleaned and collected is critical to the clinical trials conducted by Sarepta for DMD gene therapy. By implementing eClinical Solutions' and Medidata's products and services, Sarepta has been able to accelerate the clinical trial process. Databases are being built more efficiently and accurately, which enables Sarepta to begin collecting essential data from patients sooner. Once prepared, the data output is made more accessible for review. With the help of eClinical Solutions and Medidata, Sarepta is able to continue pursuing its mission of improving and extending the lives of patients with rare genetic-based diseases.

> "The elluminate platform helps to increase the confidence other Sarepta teams have in the data as we are all working from the same system, one centralized source of truth."

For more information, please visit us at:
eclinicalsol.com/services/clinical-data-services/

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