The difference is in the data.™

Full Operational Support for Rare Disease Therapies

CHALLENGE

A biotechnology firm needed assistance developing transformative breakthrough therapies for patients with severe and rare disorders. The firm focuses on high-quality, small molecule and protein-based assets in the late discovery to early clinical stages of drug development. The lead product candidate is for the prevention of a life-threatening fetal and neonatal rare disease.

MMS SOLUTION

MMS established operation functions on behalf of the firm including medical writing of the study protocol and protocol amendments, investigator's brochure, orphan drug designation request (both FDA and EMA), rare pediatric disease designation request, IMPD, CTA; provided regulatory strategy, regulatory operations including publishing and full submissions management.

In addition, MMS established all biometrics functions to support the trial needs including provision of data management, statistics, and programming expertise. Quality management systems to enable the clinical phase research were also established by MMS covering elements like SOPs, quality manuals and vendor management including vendor assessment questionnaires, business risk assessments, implementing vendor oversight plans, and adding compliance program auditing.

*"It would not have been possible without the support from all of you and the entire MMS team. Our investors expressed confidence in the **innovation and quality** of our strategy and send special thanks to you and your partnership contributions. We look forward to working with you to advance this treatment."*

OUTCOME

Experts at MMS along with the sponsor were successful in achieving early program milestones including an orphan drug designation and rare pediatric disease designation for this product. The MMS teams continue to lead three additional products for orphan drug designations for the firm.