Case Study

Rho and Anthera Pharmaceuticals' Collaborative Partnership in Cystic Fibrosis

By providing quality deliverables, actionable insights, and an exceptional customer experience, Rho developed a collaborative relationship with Anthera Pharmaceuticals. The small feasibility project we started with quickly grew to include global project management for Anthera's pivotal Sollpura study (SOLUTION), management of additional complex protocols, and regulatory support of their liprotamase development program.

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Director, Regulatory Strategy

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Background

Anthera's investigational project, liprotamase (Sollpura), was in development as the first soluble, stable and non-pig derived pancreatic enzyme replacement therapy (PERT) to be used to treat exocrine pancreatic insufficiency due to cystic fibrosis (CF). The unique advantage of Sollpura over other PERTs already on the market was that it would offer a novel solution to young children and adults who are either unable to swallow multiple pills, uncomfortable taking products derived from pigs, and/or forced to use gastric tubes in order to maintain appropriate nutritional health.

The Challenge

Rho was leading the SOLUTION study, which was designed to show Anthera's product was not less effective (non-inferior) than the comparator product in the study, when the scope of work expanded to include supporting Anthera through a seamless transition after they decided to change their European CRO approximately 4 months into the study.

Anthera also asked Rho to lead additional complex studies in the liprotamase development program (SIMPLICITY and EASY studies), as well as to provide regulatory support through the preparation and submission of the Sollpura Biologics License Application (BLA) with the Food and Drug Administration (FDA) and the corresponding Marketing-Authorization Application (MAA) through the European Medicines Agency (EMA).

As this collaborative relationship grew, the Rho team worked hard to ensure that timelines were met, the quality of deliverables was high, and that they continued to exceed Anthera's expectations.

How We Did It

1. **A Collaborative Partnership**
2. **Cystic Fibrosis Expertise and Advocacy**
3. **Retention Focused Strategies**
4. **Strong Site Relationships**
5. **Operational Efficiencies**
6. **Focused Project Teams**

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The Solution

A Collaborative Partnership

From the outset, Rho operated as an extension of the Anthera team, leading sites through the process of completing their study responsibilities, working alongside Anthera as they made critical decisions on how to continue their product's development pathway, and helping them plan another pivotal study (RESULT) in a reduced timeline with a limited budget. Rho collaborated with Anthera and the EU CRO on the RESULT study to complete enrollment in approximately half the time of the SOLUTION study despite a similar study design.

Cystic Fibrosis Expertise and Advocacy

Rho's expertise in cystic fibrosis was important to Anthera. Monica Gangal, former VP of Clinical Operations at Anthera, noted that choosing Rho to execute the SOLUTION study was easy because "Rho not only gave Anthera a clear picture of how their team would fulfill the study needs, but showed their knowledge and expertise of the indication as well as the protocol in a way that made them a collaborative partner rather than a service provider."

Patient advocacy groups are a crucial part of the drug development process and provide invaluable resources and input. The Cystic Fibrosis Foundation's Therapeutic Development Network (TDN) provided protocol review and sanctioning of the SOLUTION study. At the same time they started working with Anthera, Rho developed their own relationship with the TDN to become more involved in the CF community and this association continues to be a dynamic and important resource as Rho guides sponsors through the processes of working in the cystic fibrosis space.

Retention Focused Strategies

The team in place was experienced and ready to take on the unique features that SIMPLICITY and EASY called for. Based on the visit and subject schedules for the SIMPLICITY study, Rho coordinated confinement visits (held in the adult studies at the clinical site) in the subject's home, utilizing an in home nursing service for subjects that were infants or toddlers. In addition, Rho maintained good relationships with the investigators and their staff to coordinate the rolling of subjects into the long term safety extension (EASY) study. By maintaining their contacts from one study to the next, sites felt confident working with Rho as an extension of the Anthera team.

Strong Site Relationships

By working with a portion of sites Rho was already familiar with and had a working relationship with, start-up processes went smoothly and surpassed timeline expectations. The first patient first visit was held just 5 weeks after the RESULT protocol was finalized. Gangal noted that in particular, study timelines were an important factor for Anthera and that "Rho's innovative approach to study start-up was critical in assuring our team that we could achieve our study timelines as projected."

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Operational Efficiencies

Because all studies were with the same product, Sollpura, Rho realized efficiencies when adding additional studies that resulted in decreased timelines while maintaining sponsor confidence. Some of the efficiencies gained included:

- Basing new site contracts off previously negotiated contract language for the previous study at the same site
- Building program wide study plans (i.e., safety management plan) that were effective across multiple studies
- Developing a strong working relationship with the European CRO responsible for clinical monitoring and local regulatory activities in Europe to solidify team unity and decisions across the board. Rho participated in an organizational meeting with the EU CRO to identify risks and mitigating actions to implement at start up and monitor throughout studies.
- Consistent reporting and documentation practices

Focused Project Teams

Caitlin Hirschman, Director of Project Delivery at Rho, who served as Global CTL for most of the studies within the Anthera program, noted that, "As our team brought on additional Anthera studies, new Rho team members were brought on as needed, but the management team remained the same to provide a consistent stream of communication, management, and overall leadership." This was key for the small clinical operations team at Anthera as it allowed the team to focus on the conduct of the trial.

The Result

- Consistently **decreased timelines** as new studies were added; **surpassed timeline** expectations in start-up
- Completed RESULT study **enrollment** in approximately half the time of the SOLUTION study
- **First Patient First Visit (FPFV)** occurred just 5 weeks after the RESULT protocol was finalized
- Received **top rankings** on training delivery for execution of a complex protocol from attendees at the SOLUTION Investigator Meeting
- **Successfully coordinated** a complex lab specimen collection through analysis that was essential to the SOLUTION Study
- The relationship built throughout the collaborative work resulted in **successfully executed studies** where the sponsor and CRO team members felt like a **single unified team**

Conclusion

Partnering with a CRO that understands the urgency and gravity of development in the cystic fibrosis space is critical to your clinical trial journey. Rho's experts have the experience and determination necessary to help you successfully execute your program.

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Completed RESULT study enrollment in **approximately half the time of the SOLUTION study**

First Patient First Visit (FPFV) occurred **just 5 weeks** after the RESULT protocol was finalized

> ...Because team members are consistent across projects, we gain budget, timeline and communication efficiencies and as a result we have met or exceeded our timelines for every project. I appreciate the commitment, level of attention, detail, and out of the box thinking our projects receive and always look forward to any opportunity to collaborate with Rho.
>
> **Monica Gangal**
> **Former VP of Clinical Operations at Anthera**

To learn more about Rho's approach to optimizing studies and meeting timelines in a cystic fibrosis program, please contact us to consult one of our Rho experts.

Contact Us

To learn more, visit us at:
rhoworld.com/cystic-fibrosis

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