EXCEEDING RECRUITMENT EXPECTATIONS FOR A RARE DISEASE STUDY

Small Biopharmaceutical Client

A biopharma client asked Covance to run their novel small RNA interference molecule program addressing a rare, uniformly fatal genetic disease for which there is no known cure. The disease, which primarily causes heart failure, also can lead to neuropathy and other multi-organ involvement. Covance was asked to perform an epidemiologic study of disease prevalence and a Phase III therapeutic trial followed by an open-label investigation.

Understanding the Challenge

The biopharma company was growing quickly and this molecule represented their first potential success. The client indicated that they required a CRO with innovative ideas and an unusual degree of process flexibility to expedite the program’s progress in a competitive space. But the client faced several challenges given that their study:

▶ Needed cardiovascular expertise – particularly in the area of orphan disease cardiomyopathies and a global network of heart failure experts
▶ Required operational experience in orphan disease strategies, including a robust approach to recruitment, which had been a major challenge to other companies working in this field
▶ Demanded expertise in diagnostic disease criteria, including histopathologic, hematologic and imaging-based criteria

Developing a Plan of Action

Covance, as an untried partner in the sponsor’s lead program, had to instill confidence and trust in the client so that they could feel comfortable that their needs were being met and their lead molecule could be effectively tested.

Drawing on their experience with cardiovascular exercise testing and advanced cardiovascular imaging, the Covance team offered their in-house expert who had previous experience at internationally recognized research institutions focusing on this disease as well as other rare cardiomyopathies. Working with the client, Covance also assembled a global operational team with knowledge of regional regulatory challenges, medical institutions and standards of care.

When evaluating the proposed protocol, the Covance team immediately recognized gaps in the protocol entry criteria potentially limiting study effectiveness both in recruitment and efficacy evaluation. Covance worked with the sponsor in a proactive manner to avoid future potential protocol pitfalls and expedite recruitment.

Realizing that there were other therapeutic development programs competing for the relatively few patients with this disease, Covance also developed a plan that ultimately reduced the projected recruitment time by five months – which was unprecedented for this rare disease.
This plan included:

▶ Leveraging personal contacts as well as sites within the Covance heart failure network in the U.S. and Europe to boost the visibility of the client’s study
▶ Sending out personal letters to potential investigators outlining the scientific basis of the study and the great potential for improving the treatment paradigm
▶ Travelling to sites across the U.S. where Covance’s lead physician delivered hospital Grand Rounds and dinner talks on the rare disease to create greater awareness of the clinical challenges and opportunities of the study; building connections with cardiologists and physicians that then served as referral sources
▶ Working with the sponsor’s team to develop quality site selection and realistic timelines

Value to the Client

A major goal of the client was to expedite the program while maintaining the investigational rigor required in the development of a completely unique therapy. The client was experiencing corporate growth and initially lacked in-house expertise in the disease area and some of the processes, stressing the need for a knowledgeable and understanding partner to take control and push forward with the program.

Covance delivered a collegial and malleable approach to expand the client’s comfort zone and created a strong partnership. As a result, they achieved last patient in (LPI) in the Phase III study five months ahead of schedule – an impressive result given all the challenges faced in this rare disease space.