MEDSOURCE
Taking Relationships as Seriously as Science

CASE STUDY

In the face of adversity: Flexible clinical trial strategies for small biotech companies

The landscape for clinical trials is a chaotic one. Given the cost and resources required, it's vital for any company to do adequate research of competitors and existing products before even beginning its strategic plan. Moreover, when a trial is make or break for small companies with limited resources, understanding the landscape is even more important. Your first mistake could be your last!

Even with best-laid plans, the introduction of an unexpected factor can have a devastating effect on your entire strategy. In an environment like this, you need a clinical research organization (CRO) that knows the intricate details of the industry and has the flexibility and agility to make quick, strategic changes whenever necessary.

THE CHALLENGE

A new company facing a costly delay

In June 2015, a small biotech startup needed to evaluate the safety and efficacy of its first drug: a new oral CXCR4 inhibitor for patients with advanced clear cell renal cell carcinoma (RCC). Like many small startups, launching its first study was integral to future success, and it needed to show progress quickly or the whole company could fold. However, it did not have the resources or operational knowledge necessary to advance quickly and needed expert support from a CRO to help them to the next stage.

With the future of the company riding on getting its first-in-human trial up and running quickly, it sought expert help from MedSource. The MedSource team was able to provide critical operational experience and play a key role in launching the protocol, a phase I/II trial that would combine their investigational product with the current standard of care in second-line RCC, while working together with the company's limited staff.

As trial sites were preparing to open in November 2015, the company received some unexpected news: The FDA had granted an accelerated approval of a new RCC treatment indicated for use in the second-line setting and beyond. This meant that the patient population expected to enroll in the study now had a new standard of care regimen available to them. The trial investigators confirmed that the majority of the potential subjects would first need to be treated with the new standard of care before going onto the study, potentially delaying study enrollment for months or even years, which would be financially disastrous for the new company.

The company needed a way to get its investigational product into patients immediately or risk losing precious funding from its investors. With a small staff and limited experience, the company looked to MedSource for a solution.

THE STRATEGY

Developing a new strategy quickly

MedSource puts an emphasis on flexibility and can pivot procedures and staff whenever there is a need, a feat that larger CROs simply cannot match. Upon hearing the news, MedSource immediately began brainstorming with the company to develop alternative strategies that would adhere to FDA regulations and rapidly move this clinical development program forward.

The proposed solution was a sister study that would run concurrently with the original protocol. Using a subset of the sites already in startup, the sister study would target a slightly different RCC population and combine its investigational product with the newly approved second-line regimen. Time was a major factor when assessing the feasibility of launching a new study. Before aligning on the strategy, MedSource met with the company and performed a detailed forecast to project timelines and demonstrate how quickly the sister study could begin enrolling. Following this integral step, the company decided this strategy was absolutely worth pursuing, and it immediately began writing the new protocol.

Creating time efficiencies

Since 22 study sites had already been selected and qualified for the original study, MedSource knew that using a subset of those sites on the sister study would eliminate the time required to find and qualify new sites with an adequate patient population to support the study. However, the sites participating in the original protocol were large academic institutions with lengthy contracting processes, multiple scientific review committees, and local institutional review boards (IRBs). Maneuvering through study startup at these sites could typically take up to 18 months, but MedSource had planned and developed strategies that would streamline efforts across multiple channels. By reviewing the previously submitted feasibility surveys, MedSource was able to identify a subset of potential sites to participate in an abbreviated feasibility for the new study.

Startup metrics from the original study were reviewed to identify sites with an accelerated path through scientific review and IRB approval, and four sites were selected for rapid study startup. Site regulatory documents collected for the original study were utilized, and previously negotiated language was pulled into the new site contracts.

Operationally, MedSource was able to duplicate regulatory document templates, consent forms, patient diaries, study plans and tools, and case report forms from the original study, expediting all aspects of study planning and implementation. Thanks to MedSource's flexibility and strategic thinking, the first site of the sister study was activated within just four months. With the new study open and enrolling, the company was in an excellent position to share progress with its investors and secure funds to propel its clinical development program forward, including the original protocol.

For most clinical programs, the project team may not always be consistent. However, to gain further efficiencies in running the concurrent studies, MedSource was able to make quick resourcing adjustments that allowed the same team to work on both studies. To streamline workload, a single clinical research associate (CRA) was assigned to monitor the four sites on both studies. Monitoring visits were combined to save on travel time and costs. Furthermore, using one CRA allowed the knowledge and day-to-day work on the studies to be combined.

THE OUTCOME

In the face of devastating news, the small biotech company's lack of internal resources and experience could have meant the dissolution of the company. However, MedSource's ability to think creatively and work efficiently allowed both clinical studies to launch on time. By duplicating efforts on the sister study, all four sites were launched in less than six months, a remarkable achievement considering average academic institution lead times. The study enrolled a total of nine patients and obtained valuable data for steering the direction of the company's clinical development program.

With a total of 22 sites, the original protocol enrolled its first patient in May 2016. With funding secured, the company was able to open the protocol in a second country to support enrollment. The study ultimately exceeded the enrollment timeline by two months, enrolling a total of 74 patients.

With two successful phase I/II studies nearing completion, the company is ramping up efforts to continue clinical trials with this investigational product. A larger phase II protocol is currently being written to evaluate efficacy in multiple oncology indications.

An unexpected delay could have meant disaster for this small startup. It had limited funding and lacked the operational knowledge to navigate the complicated waters of the clinical trial landscape on its own. But, this company's story isn't unique. Many small companies face make-or-break decisions and changes throughout the clinical trial process that they are simply not equipped or prepared to handle. MedSource is able to give these companies the support and edge they need to not only survive, but thrive.

VISUAL SNAPSHOT

- **Clinical trial:** Combination phase I/II trial in the treatment of advanced clear cell renal cell carcinoma (RCC)
- **Condition:** RCC
- **Purpose:** Evaluate the efficacy of a new oral CXCR4 inhibitor in patients with RCC
- **Patient-type:** U.S.-based, second-line kidney cancer patients