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CONTRACT SERVICES PRIORITIZE SPEED FOR THEIR CUSTOMERS

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Virtual clinical trials, nimble CDMOs say speed is key

Lowering costs and helping clients get to market faster is a mantra for contract service organizations, who are seeing a proliferation of smaller drug companies without Big Pharma budgets seeking the competitive advantage of being first to market. Solutions such as crowdsourced clinical trial protocols, virtual patient data collection, and broad-based preclinical services by smaller, more nimble development and manufacturing partners are helping drugs get to market quicker and cheaper.

Transparency Life Sciences, for example, is the first all-digital clinical development services company—essentially removing the "clinic" from "clinical services." The company harnesses crowdsourcing and mobile health technology to advance biopharma drug candidates through clinical trials with unprecedented patient relevance and efficiency. The model is scalable and is designed to increase current industry margins of a wasteful process that often collects irrelevant data.

"We've really stripped it down to the metal," says Marc Foster, COO and co-founder of Transparency Life Sciences. "You could call us an all-digital CRO." A major downside of the current clinical trial model is the high cost of collecting data at a traditional site such as a hospital or clinic. "It's extremely expensive; you're loading the per-patient cost of a trial with the high fixed costs of an institution," he adds. This is particularly true in therapeutic areas such as central nervous system (CNS), autoimmune disorders, chronic cancers, and many rare diseases. "A lot of this data can be collected using mobile health solutions and telemedicine," Foster says. Study data, such as blood pressure, weight, and pulse rate, can be collected in a patient's home or workplace.

The Transparency Life Sciences model is well-suited for phase II trials, generally ranging from 100–300 patients. "For a smaller company, digital solutions make sense for a trial of that size," Foster says.

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Phase II is also where companies begin to experiment with end point selection, which digital solutions can help facilitate. The company also operates in the Phase IV space—after drugs get to market and insurance companies want to ensure the drugs they are paying for are having the stated therapeutic benefits over time.

Not all indications are workable for Transparency's digital data collection model, however. "Acute cancer, where you might be infusing an individual frequently or using full body scans, is not ideal for us," Foster says "But we can help design those trials. Part of our three-part digital platform is protocol crowdsourcing, to solicit input from a global crowd of patients and professionals. But if you're talking about executing a trial, we're better off focusing on trials that can be decentralized using telemedicine and mobile health."

FDA response has been encouraging, Foster says. Transparency Life Sciences worked on behalf of a small sponsor to complete multiple rounds of crowdsourcing for design of a trial for in pulmonary sarcoidosis. "We submitted a protocol and an IND to the FDA last March," Foster says. "It calls for 180 out of 200 of patients in the study to be measured completely remotely, with no interaction with the study staff at the hospital. FDA gave the goahead. That was really a big signal from a regulatory perspective that the FDA is open to this kind of innovation, and trying to push a lot of data collection out to the patient's home."

Transparency Life Sciences was launched six years ago and has focused on keeping the business lean and efficient.

BEFORE THE CLINIC

Speed and nimbleness is equally important before the clinic. The funding environment is strong, leading to a proliferation of companies pursuing niche or orphan indications, and whoever reaches the market first has a competitive advantage.

"Lots of new companies are coming into existence and the virtual biotech model is more common than ever," says Dan Smithey, CEO, of Serán Bioscience. "For virtual companies trying to take a new biology into the clinic as fast as possible, almost all of their development needs are outsourced. That includes early chemistries, analytical development, pre-clinical work, non-clinical toxicology, all the way up to formulation. There's a big need for companies that can do broad-based services."

Serán and Cascade Custom Chemistry have partnered to form a contract development and manufacturing organization specifically focused on GMP manufacturing of APIs. “Most of who we are targeting are smaller to mid-sized companies that are mostly virtual and want to work with a partner who can handle all of their CMC [chemistry, manufacturing, and control] needs. And that would include a lot of regulatory support as well as help with final IND, PIND packages, etc,” Smithey adds. Both are located in Oregon; Cascade is in Eugene, while Serán is in Bend.

CDMOs have proliferated right along with virtual biotech, but many have been acquired by larger companies—giving Serán-Cascade a unique competitive advantage. “Small, science-based, agile CDMOs are getting incorporating into larger organizations that are necessarily more bureaucratic and more complicated to work with,” Smithey says. “And if you’re an early stage company that wants to move fast and have flexibility in timing, the bigger corporations are a little bit more difficult to work with. So a lot of our customers are looking for smaller CDMOs that are easier to work with on a personal basis. Consolidation has removed a lot of those from the offering plate and there’s really an opportunity we think to fill that gap.”

Despite cost pressure along the drug development supply chain, Serán-Cascade has not seen any pressure on its price. “I think part of the reason is we are early stage focused,” says Jeremiah Marsden, president Cascade Custom Chemistry. “We don’t do any Phase III or commercial. I think downstream is where you see most of that pressure. In development,

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the research phase, it’s very difficult to compress the price because time is of the essence. Time is more valuable than money. There seems to be enough money out there in the investment world.” Serán-Cascade is also seeing a lot of interest from local companies. “We find a lot of our customers used to try and outsource to many different locations, in the US and abroad,” Marsden says. “I think that was just too difficult for them. A lot of them are pulling back more locally, especially if it’s in the same time zone and easy communication with a small, flexible company like we have in Cascade and Serán. And one big drive for this partnership was to be able to offer more, from discovery to process development, GMP and formulation. And I think a lot of places really see the benefit of that.”

Serán-Cascade is a small molecule CDMO with an ability for simultaneous synthetic process R&D and formulation development, to integrate analytical development, and to synchronize manufacturing of API and CTM in the same facility.