Clinical trials go virtual, big pharma dives in

In May, UCB became the latest high-profile pharma to partner with tech startup Science 37, joining Sanofi, Novartis and Otsuka. The clinical research company Science 37 has become a key ally for pharmas that are starting to transform how they conduct clinical trials. The company is at the leading edge of a trend to bring telemedicine-based platforms to clinical trials so that companies can run ‘site-less’ trials, where patients participate from home, regardless of their geographical location. This move towards decentralized clinical trials could accelerate recruitment, and improve patient retention. And by enabling individuals to take part from home, it could also help address the lack of ethnic diversity that prevails in clinical trials. For drugmakers, the potential payoff is enormous. Faster recruitment means the companies can reach key go/no-go decisions earlier in development, rather than continue pursuing new therapies that will ultimately fail.

Pharma companies are attracted to the virtual model whereby patients feed data from their mobile phones, tablets and other telemedicine services, into a cloud-based platform. Not only is it efficient, but it also encourages patient participation. What’s more, virtual trials run from a centralized platform that enables everyone involved to share study design, protocols and clinical trial management information online. The platform used by Science 37 is the Network Oriented Research Assistant (NORA), and it is run by a network of physicians trained to work with the software across different therapeutic areas. The platform used by Science 37 is the Network Oriented Research Assistant (NORA), and it is run by a network of physicians trained to work with the software across different therapeutic areas. Noah Craft, co-founder and CEO of Science 37, says NORA functions like an all-in-one electronic medical records (EMR) system and mobile data collection tool, which makes it possible for physicians to remotely monitor and communicate with patients in trials. Science 37 has enrolled patients from as many as ten US states in a single trial. “It [the platform] makes the denominator of potential participants in trials everyone, not just people who have the means to drive to Stanford or UCLA,” Craft says.

UCB’s first virtual clinical trial with Science 37 is a phase 3b study of rotigotine for restless leg syndrome in adolescents. Rotigotine is the company’s transdermally delivered dopamine agonist that it markets as Neupro. UCB and Science 37 are recruiting 13- to 17-year-old patients, an age group that UCB had previously rejected for inclusion in clinical trials on the grounds that it wasn’t practical, says Iris Lowe-Friedrich, UCB’s chief medical officer. The company had worked out that it would have taken seven to eight years to recruit 138 patients, she says. “Our expectation is that in the virtual trial model we will be able to reach patients and their parents within 18 months.”

Speed is Science 37’s value proposition, says Craft. Faster drug development, also means a company can have a product on the market sooner, extending the time before patents run out. And for situations where multiple companies are developing therapies for the same target, being first to market has a competitive advantage, he says. “Speed is good for everyone,” he says. “The FDA [US Food and Drug Administration] likes things to be done faster; the patients like the cures to be brought to them faster.”

Virtual trials can collect data frequently and continuously from patients’ homes.

Accelerating clinical trials also means companies can kill a compound sooner, and potentially minimize the cost of pursuing therapies that are destined to fail, says Belinda Tan, Science 37 co-founder and chief medical officer. One industry partner has already benefited from the speed afforded by virtual clinical trials, she says. “Midway through the study there was an interim analysis by the sponsor, and they determined that the drug showed no efficacy,” says Tan. “And so at that stage they decided to shut down the study and move on.”

UCB tested the waters with a small virtual clinical trial two years ago, and that experience spurred the company to partner to expand into virtual clinical trials. The company chose Science 37, and plans to evaluate all of its upcoming clinical studies to determine whether they are suitable for Science 37 to run, says Lowe-Friedrich. “We are really changing our paradigm from the conventional set up to [the virtual model] whenever possible and wherever possible,” she says.

Novartis hopes virtual clinical trials can help it narrow two specific gaps. The first is between targets for patient recruitment and retention, and the realized rates. “Fewer than 5% of eligible patients participate in trials,” says Bertrand Bodson, Novartis’ chief digital officer, “so there’s a huge gap.” Also, on average, 20% of sites end up enrolling no patients at all, and 30% of patients in trials end up dropping out, he says. “Our plan overall is to rethink the model to make [study enrollment] much easier from a patient point of view, to be able to have a much broader reach,” he says. The second gap is between responses to drugs in clinical trials and what happens with recently approved drugs in the real world, says Bodson. Because virtual clinical trials lower barriers to participation, they are more representative of the real world experiences than conventional studies, he says.

Novartis is also testing Apple’s ResearchKit in a virtual clinical trial launched in April. The prospective ophthalmology trial uses Novartis’ FocalView app, which allows patients to record quantitative as well as qualitative data about
CRISPR gene therapy trial on hold

The US Food and Drug administration has delayed the start of a human study of a genetically engineered therapy for treating sickle cell disease. In May, the agency issued a clinical hold on the investigational new drug application filed by Vertex and CRISPR Therapeutics to test CTX001, a new autologous gene therapy made with CRISPR-Cas9 technology, pending resolution of questions from the agency. No other details on the reason for this hold were disclosed. CTX001 is designed to produce high levels of fetal hemoglobin (HbF) in red blood cells. HbF is a form of the oxygen-carrying hemoglobin that is naturally present at birth, and is then replaced by the adult form of hemoglobin. The hope is that elevating HbF using CTX001 can alleviate transfusion requirements and debilitating crises for sickle cell and β-thalassemia patients. In December 2017, CRISPR presented preclinical data showing greater than 90% editing of hematopoietic stem cells at the target site, leading to clinically relevant increases in HbF. According to CRISPR Therapeutics, which is headquartered in Zug, Switzerland, a European trial of CTX001 in patients with transfusion-dependent β-thalassemia remains on track to begin later this year. The company’s collaborator Vertex has also exercised an option to co-develop CTX001 for hemoglobinopathies. In February 2018, the University of Pennsylvania in Philadelphia began enrolling patients in the first CRISPR trial in the US, to treat multiple myeloma, melanoma and sarcoma, using engineered autologous NY-ESO-1 T cells, edited to remove endogenous TCR and PD-1. Several trials of cells engineered using CRISPR-Cas9 technology are recruiting in China.

“Anyone who does synthetic biology should be under surveillance, and anyone who does it without a license should be suspect.” Harvard University geneticist George Church comments on the publication of an experiment recreating a virus that has engendered fears that such information could be used to create a bioweapon. (The New York Times, 14 May 2018)

“When you put DNA and privacy together in a sentence, understandably and correctly, it makes people nervous.” Laura Hercher, of Sarah Lawrence College, comments on the recent security breach at MyHeritage.com, in which e-mails and passwords belonging to over 92 million site users were leaked. She added “I would rather give someone my DNA than my social security number, my search history, or my credit card.” (STAT, 5 June 2018)