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Trials In Focus: Joint Venture Aims To Simplify Cell Therapy Trials For Smaller Players

by Alaric DeArment

CTMC CEO Jason Bock talked to *Scrip* about the venture between MD Anderson and National Resilience, which provides manufacturing, process development and regulatory support for small cell therapy developers' preclinical and clinical proof-of-concept studies. Meanwhile, Verge Genomics initiated a Phase Ib study of its AI-discovered drug for ALS; EyePoint and Aviceda dosed patients in their Phase II DME trials; Parexel teamed up with a non-profit cancer research group in Japan; and BARDA selected PPD to run its Phase II platform trial for ARDS.

In the more than six years since the US Food and Drug Administration approved *Novartis AG*'s Kymriah (tisagenlecleucel) and Kite Pharma's – now part of *Gilead Sciences, Inc.* – Yescarta (axicabtagene ciloleucel) as the first CAR-T cell therapies, the number of cell therapies and new methods of developing them have skyrocketed. But developing cell therapies remains a challenge especially for smaller, earlier-stage companies and academic institutions, and in response, a joint venture from the University of Texas MD Anderson Cancer Center and biomanufacturing company *National Resilience, Inc.* aims to make that process easier and more likely to be successful.

Incorporated in May 2022, the venture, CTMC, is at the Texas Medical Center in Houston, near MD Anderson. Its purpose is to help smaller organizations reach clinical proof of concept and advance towards commercialization by providing process development, manufacturing and regulatory support.

One of CTMC's partners, *Obsidian Therapeutics, Inc.*, announced 12 December 2023 positive interim topline data from its Phase I study of OBX-115, a tumor-infiltrating lymphocyte (TIL)

cell therapy that it is developing for metastatic melanoma that relapses or is refractory to prior checkpoint inhibitor therapy, showing a 50% overall response rate (ORR), a 33% complete response (CR) rate and 100% disease control rate (DCR) in six patients.

"There's lots of academic GMP manufacturing sites at different hospitals around the country, but not to this scale," CEO Jason Bock told *Scrip*. "And there are lots of CMOs, but they're not so closely connected with clinical infrastructure or have regulatory capabilities."

CTMC began its life within MD Anderson, acquiring a 60,000-square-foot industrial facility from *Bellicum Phamaceuticals, Inc.* and hiring several dozen employees, before spinning out into the current joint venture, of which MD Anderson and National Resilience each own a 50% share. That, Bock said, gave it the flexibility to partner with groups nationwide and worldwide and develop their cell therapies at CTMC and then run clinical studies at MD Anderson.

"Mostly what we do is offer partners a pathway for their IND-enabling studies, including the CMC development, the regulatory strategy and operational support, as well as the clinical trial infrastructure of MD Anderson," Bock said. "So basically, everything a preclinical company needs to get their products from preclinical proof of concept – hey, my product works well in mice – to 15, 20, 25 patients of clinical data."

But the current focus on smaller-scale efforts doesn't mean supporting larger and pivotal trials is out of the question.

The idea is to provide groups with the means to complete those earlier stages of development and then have a pathway to bring their product through pivotal development as well. Bock added that CTMC has yet to do that, but it is on its way. The manufacturing plant is well equipped to supply material to MD Anderson given that it's only blocks away from the cancer center, which itself is a top enroller in cell therapy trials. But he added that CTMC is also capable of supporting and does support multicenter studies.

Other Clinical Trial News

Verge Genomics said 9 January that it had initiated a Phase Ib trial of VRG50635 in patients with sporadic and familial forms of amyotrophic lateral sclerosis (ALS). The drug is an inhibitor of PIKfyve, a therapeutic target for ALS discovered using the company's AI-based discovery platform, and Verge called VRG50635 one of the first drugs to enter the clinic that was entirely discovered and developed using an AI-enabled platform. The trial will use digital devices to collective measurements directly from the patient including mobility, breathing and sleeping rather than relying on physician rating scales. The announcement comes after the company already showed positive Phase I safety and tolerability data for the drug, making it an early mover in having clinical data for an AI-discovered drug. Verge has lately been forming partnerships with several large pharmaceutical companies, including a deal announced in

September 2023 with *AstraZeneca PLC*'s Alexion, which came two years after an earlier partnership with *Eli Lilly and Company*. (Also see "*Verge Builds Out Big Pharma AI Partnerships With AstraZeneca Deal*" - Scrip, 8 Sep, 2023.)

EyePoint Pharmaceuticals, Inc. announced 10 January that it had dosed the first patient in the Phase II VERONA trial of EYP-1901 in diabetic macular edema (DME). EYP-1901 is a sustained delivery therapy containing vorolanib, a tyrosine kinase inhibitor formulated in bioerodible Durasert E. VERONA is a randomized, controlled, single-masked study in DME patients who have received prior anti-VEGF therapy and comprises three arms testing two intravitreal doses of EYP-1901 and a control arm consisting of *Regeneron Pharmaceuticals, Inc.*'s Eylea (aflibercept), with the primary endpoint being time to first supplemental Eylea injection. The company expects topline data in the first quarter of 2025. It is also testing EYP-1901 in the Phase II PAVIA trial, in non-proliferative diabetic retinopathy, and plans to initiate the first pivotal Phase III trial in wet age-related macular degeneration in the second half of 2024.

Aviceda Therapeutics said 9 January that it had enrolled the first patient in its Phase II GLYCO study of AVD-104, also in DME. AVD-104 is an engineered glycan (sialic acid) nanoparticle designed to reduce inflammation by targeting the self-pattern recognition receptors on overall activated retinal neutrophils, macrophages and microglia and by repolarizing them to their resolution state. GLYCO is an open-label study that will enroll 30 patients to evaluate a low dose and a high dose of AVD-104 with three months of follow-up, the primary endpoint being incidence and severity of ocular and systemic adverse events. Macular thickness and vision will be among the secondary efficacy endpoints.

CRO *Parexel International Corp.* said 10 January the Japanese Foundation for Cancer Research (JFCR) will join Parexel's Global Site Alliance network of over 480 sites and 21,000 investigators, a move meant to expand Japanese patients' ability to participate in cancer research. Parexel will tap JFCR's expertise to help sponsors develop trial protocols that align with standards of care and regulatory approval processes in Japan. The partnership would add to Parexel's relationships with clinical trial sites in the Asia-Pacific region, including Kyoto University Hospital and the Osaka International Cancer Institute in Japan and China's Beijing Illness Challenge Foundation and the Cancer Hospital Chinese Academy of Medical Sciences.

PPD, Inc., the contract research organization (CRO) arm of Thermo Fisher Scientific Inc., said 21 December that the Biomedical Advanced Research and Development Authority had selected it to implement the first BARDA-supported Phase II platform trial to investigate therapeutic options for treating acute respiratory distress syndrome (ARDS), a condition associated with COVID-19 and other viral and bacterial infections for which there is no approved or licensed therapeutic. The purpose of the trial is to collect clinical and biomarker data on the ARDS patient population to better inform future clinical trials and contribute to the development of targeted therapies for the condition.