CDR3 Therapeutics

TREATMENT BEGINS HERE

EXECUTIVE SUMMARY

Stem Cell Immune Gene Immunotherapy for Curing Chronic Viral Infections and Associated Cancers

MISSION

The mission of CDR3 Therapeutics is to develop a curative stem cell gene immunotherapy, delivering chimeric antigen receptors (CARs) or T cell receptors (TCRs) to treat chronic viral infections and virus-associated cancers.

THE SCIENCE AND MEDICINE OF CDR3

CHRONIC VIRAL INFECTIONS AND ASSOCIATED CANCERS:
Chronic viral infections have drastic consequences on human health and welfare. Some have suppressive but non-curative treatments, such as HIV, herpesviruses (HSV-1/2, EBV, CMV) or Hepatitis B Virus, while others have no treatments, such as human papillomavirus (HPV). Beyond the clinical and economic burdens of chronic viral infections, many often lead directly or indirectly to cancer.

THE PROMISE:
Researchers have known for decades that T cells are critical for killing cells in the body with foreign or mutated proteins,

i.e., cells that are virus-infected or cancerous, for cure or chronic containment of disease. T cells use naturally generated T cell receptors (TCRs) to target and kill such abnormal cells. In the past few years, artificial TCRs called chimeric antigen receptors (CARs) have been inserted into patient T cells using gene therapy (T cell immunotherapy), thereby reprogramming them to target cancers. Four CAR gene therapies have been FDA cleared for treatment of blood cancers, and many more are in clinical testing.

THE CHALLENGE:
Clinical success has been achieved for a few diseases that can be rapidly cured with T cell immunotherapy, but most viral infections and cancers are resistant to clearance. Such conditions will require sustained and long-lived T cell targeting to control and/or clear disease long term, perhaps over years or decades. Current T cell immunotherapy technology involves extraction of blood T cells, performing CAR or TCR gene therapy, growing them to large numbers in the laboratory, and giving them back to patients. This is amenable to short term therapy, but this process drives the cells to become abnormal, with reduced function and poor long-term survival after reinfusion, a significant limitation of the only FDA-cleared CAR therapeutics, Yescarta from Kite, Kymriah from Novartis, and Breyanzi and Abecma from Bristol Myers Squibb.

OUR PROPRIETARY SOLUTION:
We have developed patented and proprietary technology to introduce CARs/TCRs targeting viruses and/or cancers to hematopoietic stem cells (HSCs), which are precursor cells that generate all the cells of the immune system. Administered HSCs engraft in the bone marrow and continuously develop normally into T cells in the body. Isolating HSCs from patients, applying CAR gene therapy, and reinfusing them creates a permanent, self-renewing source of healthy, normally developed T cells with the CAR/TCR, avoiding the high cost and significant limitations of expanding T cells outside the body for adoptive T cell gene therapy. Thus, HSC gene therapy provides a long-term source for fully functional T cells that have been retargeted against the virus or cancer, which can be maintained to control or prevent disease for a lifetime. Our strategy using virus-specific CARs and TCRs delivered via HSCs may also address factors related to the "cytokine storm", a severe inflammatory and sometimes fatal complication seen with the current FDA-cleared T cell immunotherapies. For experimental proof-of-concept, we have shown in humanized mice (mice transplanted with human immune systems) and monkeys that HSC CAR gene therapy can generate new antiviral T cells in the body that safely persist and have potent effects against HIV.

KEY BENEFITS OF CDR3 TECHNOLOGY

- HSC-based CAR gene therapy creates a permanent, self-renewing source of T cells directed against diseased cells over the lifetime of the patient
- These T cells are naturally produced in the body and are normal in their ability to function and persist, in contrast to current T cell immunotherapies that grow the cells outside the body
- Eliminates the high cost and biological problems associated with growing cells outside the body
- Potentially removes T cells that target normal cells during T cell development in the body, reducing the risk of the cytokine storm associated with conventional CAR therapy
- Addresses the barriers for successful treatment of chronic viral infections and associated cancers that require persistent highly functional T cells, such as HIV, Cytomegalovirus (CMV), Epstein-Barr Virus (causing post-transplant lymphoproliferative disease, nasopharyngeal carcinoma, gastric carcinoma and others), Hepatitis B Virus and Human Papillomavirus (causing cervical cancer, anal cancer, head and neck cancer, tongue/mouth cancer).

BUSINESS PLAN

The patented and proprietary technologies developed by CDR3 provide the basis to pursue gene immunotherapies harnessing stem cells to achieve success where current T cell immunotherapies have failed. CDR3 will initially focus its efforts on treatment of HIV infection using a CAR that has already undergone significant preclinical animal testing, producing immunity that can control the virus to obviate the need for life-long treatment with medications that are expensive (hundreds of thousands of dollars) and have significant side effects. This will be our prototypic first product for investigational new drug (IND) development, establishing a platform that is readily translatable to the treatment of other chronic viral infections and virus-associated cancers.

Our immediate plan is to raise the $3 million of initial funds to complete pre-IND development of our HIV treatment and begin parallel development of CAR/TCR stem cell immunotherapies for HPV-induced cancers.

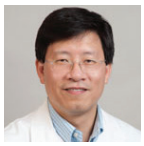
THE COMPANY

T cell CAR gene immunotherapy has had a huge impact on an extremely limited class of cancers (B cell liquid tumors) but has hit a significant roadblock in treating conditions requiring persisting immunity such as solid tumors. Nonetheless, this has been a huge area of growth and investment in the biopharmaceutical arena. CDR3 Therapeutics' uniqueness lies in its ability to address this critical issue through patented technology to harness stem cells, and we are well positioned to capitalize on the biomedicine growth trend for patients and investors alike. Based on decades of research, we have developed CARs and techniques to apply to HIV with strong pre-clinical data supporting the success of our approach.

CDR3 Therapeutics Corp., (CDR3) a Delaware Corporation, has three of its scientist-founders located at the University of California, Los Angeles and have over 20 years' experience working together on HIV research. This team has garnered more than $96 million in peer-reviewed grants relevant to research on a cure for HIV from the National Institutes of Health (NIH), the Center for AIDS Research (CFAR), The Foundation for AIDS Research (amfAR), and the California Institute for Regenerative Medicine (CIRM). They hold three core patents filed by UCLA Technology Development Group (TDG), and CDR3 holds an exclusive license from The Regents of The University of California for these proprietary technologies.

The CDR3 Company Founders and Management Team:

Scott G. Kitchen, PhD UCLA Associate Professor of Medicine. Dr. Kitchen is a biomedical, translational scientist who developed much of the platform on which the CDR3 CAR technology is based. He has vast expertise in immunotherapy development and in the development of stem cell-based technologies.



Otto O. Yang, MD UCLA Professor of Medicine. Dr. Yang is a physician-scientist whose expertise spans both clinical infectious diseases and translational laboratory research. His interests include the pathogenesis of HIV infection and the role of cellular immunity in infections, transplantation, and malignancies, with emphasis on developing immunotherapeutic strategies.

Jerome A. Zack, PhD UCLA Distinguished Professor of Medicine and Chair of Microbiology, Immunology, and Molecular Genetics. Dr. Zack is a biomedical scientist who has extensive experience in the development of new ways to understand and attack viral infection, particularly that of HIV, and in stem cell-based approaches.

Matthew C. Lorence, PhD, MBA Chief Executive Officer. Dr. Lorence is a biomedical scientist and business professional with extensive commercial experience in the genomics and molecular diagnostic industries, from start-up to highly matrixed organizations and has experience with the FDA through multiple interactions with different FDA centers. He has a track record of growing existing businesses, building new businesses, and developing strategies to enter new markets.

For more information about CDR3 Therapeutics and its quest for a curative stem cell gene immunotherapy, contact Matthew Lorence, PhD, MBA at matt@lorence.tech.