



Abata Therapeutics Launches to Usher in New Era of Cell Therapy Using Targeted Regulatory T Cells to Treat Serious Autoimmune and Inflammatory Diseases

Pipeline includes lead program in progressive multiple sclerosis and programs in inclusion body myositis and Type 1 Diabetes; plans to initiate three clinical studies by EOY 2025

Strategic partnership with ElevateBio to accelerate development of autologous Treg cell therapies

Brings together industry experts and deeply engaged pioneers in Treg biology, T cell receptor and antigen discovery, disease pathogenesis, and molecular and imaging biomarkers

\$95 million Series A financing led by Third Rock Ventures with participation from financial and strategic investors committed to advancement of cell therapy

BOSTON, June 23, 2021 – Third Rock Ventures today announced the launch of [Abata Therapeutics](#), a company focused on translating the biology of regulatory T cells (Tregs) into transformational medicines for patients living with progressive multiple sclerosis (MS) and other severe autoimmune and inflammatory diseases. The company – first seeded by Third Rock Ventures and ElevateBio – has secured \$95 million in Series A funding led by Third Rock Ventures, with participation from a diverse syndicate of investors, including ElevateBio, Lightspeed Venture Partners, Invus, Samsara BioCapital and the JDRF T1D Fund.

“Millions of people worldwide suffer from autoimmune diseases, and the majority of patients, including those with progressive MS, have inadequate or no therapeutic options,” said Samantha Singer, MS, MBA, president and chief executive officer of Abata. “The ideal treatment for autoimmune disease would be active only at the site of disease, counteract the full range of complex inflammatory mechanisms, promote tissue recovery and last for years. This is a Treg’s native role in the immune system. At Abata, we’re harnessing Tregs as targeted therapies to alter the course of serious autoimmune disease and succeed where others have failed.”

Abata’s Product Engine and Pipeline

Abata’s autologous Treg cell therapies are engineered to express T cell receptors (TCRs) that recognize antigens present in tissue where an autoimmune response has been triggered. This enables Abata’s Tregs to target the site of disease for a robust effect without imposing systemic immune suppression. Use of the native TCR signaling pathway uniquely maximizes the full effect of Abata’s Tregs, helps to maintain a stable Treg phenotype for optimal safety and stimulates tissue residency for durability, which may allow the effects of a single dose to last for years or even a lifetime.

Once activated in affected tissue, Abata’s cell therapies employ the intrinsic polypharmacy arsenal of Tregs to suppress multiple mechanisms of inflammation, including the use of soluble cytokines that act on nearby cells, even those with different antigen specificity – also referred to



as bystander suppression. Additionally, Tregs promote homeostasis in the tissue – a state of harmony – and stimulate the body’s natural repair mechanisms. Furthermore, Abata’s Tregs are autologous, meaning they are derived directly from a patient’s own cells, minimizing the risk of rejection once engineered cells are delivered back to the patient.

In conjunction with today’s launch, Abata has entered an exclusive strategic partnership with ElevateBio, leveraging ElevateBio BaseCamp, the company’s technology-enabled R&D and manufacturing center. Together, the companies will deploy a proprietary process to isolate, engineer and expand mature, thymically derived Tregs, which – unlike induced Tregs originating in the periphery – are resistant to pro-inflammatory triggers.

Abata’s lead program in progressive MS builds on well-understood technology in autologous cells to create a rapid path to the clinic. Additional programs in Abata’s pipeline include Type 1 Diabetes (T1D) and inclusion body myositis (IBM), which are also tissue-specific autoimmune diseases with substantial unmet need and a strong rationale for Abata’s Treg approach.

- **Progressive MS**: Abata’s lead program in progressive MS targets patients with non-relapsing disease, for which there are no approved therapies, with imaging evidence of ongoing inflammatory tissue injury and a genetic HLA haplotype of DR2a/b, altogether estimated to be about 45,000 patients in the U.S. Success in this program supports expansion into additional disease-associated haplotypes and ultimately to all progressive MS patients, including those who still experience relapses.
- **Type 1 Diabetes (T1D)**: Abata’s T1D program will treat patients with the genetic HLA haplotype DR3-DQ2 who are early in autoimmune pathogenesis – representing over half of addressable patients – ultimately aiming to prevent the onset of symptomatic disease and insulin dependence.
- **Inclusion Body Myositis (IBM)**: IBM is a debilitating disease characterized by inflammation that leads to muscle damage and weakness. Today, there are no therapies. Abata’s Treg cell therapy will treat patients with the genetic HLA haplotype DR3, an estimated 75% of patients.

“Our initial and urgent focus is on patients living with non-relapsing progressive MS, which remains untreatable, despite exciting advances for relapsing forms of the disease,” said Richard M. Ransohoff, M.D., co-founder and chief medical officer at Abata and venture partner at Third Rock Ventures. “At Abata, we’re taking an entirely new approach by engineering Tregs to infiltrate, disrupt and suppress inflammation that specifically drives progressive pathology. Unlike current MS therapies, which indirectly modulate inflammation by selectively altering certain cell types in the periphery, our TCR-engineered Tregs directly modulate all cellular elements of inflammation at the site of disease and may have the potential to drive remyelination. The time for Tregs is now, and we are emboldened in our approach to halt the progression of this debilitating disease and others like it.”

Leadership



Abata brings together industry experts and deeply engaged pioneers in Treg biology, TCR and antigen discovery, disease pathogenesis, and molecular and imaging biomarkers, including the following founders and scientific advisors:

- **Diane Mathis, Ph.D.**, Morton Grove-Rasmussen Professor of Immunohematology at Harvard Medical School. Dr. Mathis is a world-renowned leader in Treg biology with a lab focused on the fields of T cell differentiation, autoimmunity and inflammation. She has led landmark studies on T cell differentiation focused on maturation and selection of the T cell repertoire in the thymus, and on cellular and molecular influences on the “flavor” of T cell responses in the periphery.
- **Michael Birnbaum, Ph.D.**, assistant professor of biological engineering at Massachusetts Institute of Technology. His lab systematically examines the antigen recognition repertoire of T cell receptors and uses this information to engineer new methods to mount potent immune responses.
- **Roland Martin, M.D.**, professor of neurology and neuroimmunology at University Hospital Zürich. Dr. Martin heads one of the largest MS centers in Switzerland, where the main interests of his group include disease mechanisms of MS, cellular immunology and developing novel treatments for MS. Dr. Martin has played a seminal role in pathogenic antigen and TCR discovery in MS.
- **Richard M. Ransohoff, M.D.**, co-founder and chief medical officer; venture partner at Third Rock Ventures, collaborating scientist in the Department of Cell Biology at Harvard Medical School; adjunct professor of pathology, genetics and genome sciences at the Case Western Reserve University School of Medicine; and adjunct professor of molecular medicine at Cleveland Clinic Lerner College of Medicine. Dr. Ransohoff is a neurologist who has dedicated his career equally to patient care and to researching the role of inflammation across the spectrum of neurological disease, in particular MS.
- **Daniel Reich, M.D., Ph.D.**, senior investigator at the National Institute of Neurological Disorders and Stroke at the National Institutes of Health; and adjunct professor of radiology, neurology and biostatistics at Johns Hopkins University. Dr. Reich’s lab is focused on developing advanced MRI techniques to understand MS and adapting those techniques for clinical trials and patient care by harnessing noninvasive imaging modalities to dissect biological mechanisms of tissue damage.

Additional leadership for the company includes Samantha Singer, MS, MBA, president and chief executive officer; John Trzuppek, MBA, Ph.D., chief operating officer; Stephen Sofen, Ph.D., chief technical officer; and Andrea van Elsas, Ph.D., chief scientific officer and venture partner at Third Rock Ventures. Abata has also named its board of directors, which may be viewed [here](#).

“Abata is the culmination of a three-plus-year effort by the company’s scientific founders and the team at Third Rock Ventures to interrogate the potential for a Treg cell therapy. Identifying our first programs meant fully understanding where disease pathology and Treg biology intersect to make the biggest impact for patients, and the first clear answer is progressive MS,” said Abbie Celniker, Ph.D., executive chairman of Abata’s board of directors and partner at Third Rock



Ventures. “We look forward to the future as the company moves quickly to advance its MS program into the clinic and expand upon the potential of Treg biology in inclusion body myositis, Type 1 Diabetes and beyond.”

About Third Rock Ventures

Third Rock Ventures is a leading healthcare venture firm focused on advancing disruptive areas of science and medicine to deliver breakthroughs to patients. By starting with big ideas and fostering collaboration among people with expertise in science, medicine, business and strategy, we aim to discover, launch, and build innovative companies that can transform the lives of patients. Our mission is to be the preferred partner for entrepreneurs, investors, employees and industry to build great companies that discover and develop products that make a difference for the patients we serve. For more information, please visit www.thirdrockventures.com.

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