



Design Therapeutics Launches with \$45 Million to Develop a New Class of Disease-Modifying Therapies for Serious Degenerative Disorders

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SAN DIEGO--Design Therapeutics announced today that it is launching to create and develop a new class of therapies for patients with serious degenerative disorders caused by nucleotide repeat expansions. The company has closed a \$45 million Series A financing led by SR One, with participation from Cormorant Asset Management, Quan Capital and WestRiver Group, to advance its lead therapeutic candidate into clinical development for the treatment of Friedreich's ataxia, and support advancement of its discovery programs for multiple other degenerative diseases, including fragile X syndrome and myotonic dystrophy.

Nucleotide repeat disorders are a set of genetic disorders caused by repeat expansion in certain genes. Depending on where it is located, the repeat may cause a change in the regulation of gene expression or produce a toxic gene product, all of which are recognized as significant contributors to degenerative diseases. Design Therapeutics in-licensed its technology from the Wisconsin Alumni Research Foundation (WARF), and aims to take a unique approach to treatment and provide hope for patients with degenerative disorders.

Design Therapeutics was co-founded by Pratik Shah, Ph.D., and Aseem Ansari, Ph.D. Dr. Shah serves as chairman of the board at ARS Pharmaceuticals, and prior to that, until its acquisition by Sanofi, was chairman of Synthorx. Earlier, Dr. Shah was president and chief executive officer of Auspex Pharmaceuticals until its acquisition by Teva in 2015. Dr. Ansari currently serves as chair of the Department of Chemical Biology and Therapeutics at St. Jude Children's Research Hospital. Previously, Dr. Ansari was a faculty member at the University of Wisconsin, where during his tenure, he helped execute the license agreement with WARF. Design Therapeutics' leadership team also includes Sean Jeffries, Ph.D., chief business officer, formerly with the Boston Consulting Group.

"Significant industry advancements have led to the understanding of root causes of multiple nucleotide repeat disorders, however, there remain few to no therapeutic options that slow the progression or reverse the course of disease," said Dr. Shah. "Our company was founded with a goal of designing a new class of small molecule therapies that address the core etiology of diseases to deliver a biological effect typically only seen with complex molecules. The collective insights of our team, along with our financial strength, position us to transition into a clinical-stage company to explore the corrective benefit of our programs."

Joining Dr. Shah and Dr. Ansari on the Board of Directors, are lead investor Simeon George, M.D., chief executive officer of SR One, and co-lead investor Stella Xu, Ph.D., managing director at Quan Capital, as well as Rodney Lappe, Ph.D., a seasoned R&D leader and the former chairman of Mirati Therapeutics.

"Design Therapeutics has an opportunity to truly transform the way severe degenerative diseases are managed with their proprietary technology and disease-modifying approach to treatment," said Dr. George. "We are excited to have the opportunity to work with Pratik and Aseem, two experienced drug developers who have helped deliver novel therapies to patients. Their pipeline is compelling, with encouraging preclinical data in Friedreich's ataxia. I believe their unique therapeutic approach to restoring natural gene expression holds tremendous potential, and we are excited to support their future."

The company's pipeline is led by a novel program for Friedreich's ataxia, the most common form of hereditary ataxia in the U.S., affecting about 1 in every 50,000 people. Friedreich's ataxia, affects the nervous system resulting in progressive and life-altering movement problems and shortened life expectancy. Patients with Friedreich's ataxia have an expanded GAA repeat in the first intron of the *FXN* gene, which blocks transcription and limits production of the frataxin protein. Design Therapeutics has developed a novel program that unblocks transcription, thereby restoring the natural production and function of frataxin. With the use of proceeds from the Series A fundraising, Design Therapeutic intends to conduct IND-enabling studies and initiate clinical development for its program for Friedreich's ataxia.

About Design Therapeutics

Design Therapeutics is a biotechnology company developing a new class of therapies for serious degenerative disorders caused by nucleotide repeat expansions. The company's lead program is focused on the treatment of Friedreich's ataxia and discovery efforts are ongoing in other degenerative diseases. For more information, please visit designtx.com.

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