MyoKardia

PIONEERING PRECISION CARDIOVASCULAR MEDICINE

MyoKardia, Inc. is dedicated to revolutionizing the treatment of genetic heart diseases through the development of novel, small-molecule targeted therapeutics that address the underlying cause of disease. By combining leading-edge cardiovascular genetics with recent advances in heart muscle biochemistry, MyoKardia seeks to usher in an era of precision medicine that will dramatically improve the treatment of cardiomyopathies and make a meaningful difference in the lives of people with genetic heart disease.

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SHaRe Patient Registry

Improving Understanding of Cardiomyopathy, Paving the Way for Improved Therapies

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The Sarcomeric Human Cardiomyopathy Registry (SHaRe) is a multi-center, international repository of clinical and laboratory data on individuals and families with genetic heart disease developed as a collaboration with several world-leading cardiovascular centers.

The goal of SHaRe, a first-of-its-kind effort, is to advance the understanding of hypertrophic cardiomyopathy (HCM) and dilated cardiomyopathy (DCM), two types of heritable heart disease affecting nearly 2.4 million adults and children in the U.S. and Europe. By building the world's most robust database of its kind, cardiovascular geneticists and research-based cardiologists will be able to expand their knowledge base and generate fresh insights and research initiatives supporting the development of more targeted and effective approaches in the treatment of genetic heart disease.

The Nature of Genetic Heart Disease Creates Significant Treatment Challenges

HCM and DCM are caused by mutations in the protein genes of the sarcomere, the molecular motor and fundamental contractile unit of heart muscle, and passed on in families in an autosomal dominant pattern of inheritance. HCM, which causes a thickening of the heart walls, affects about one in 500 people. DCM produces weakening of the heart walls and enlargement of the heart chambers, and is estimated to occur in one in 2,500 people.

The discovery of treatments that can halt the progression of HCM and DCM has remained an elusive goal due to the complex and varied nature of these conditions. Currently available treatments are limited and do not target the primary cause of disease.

For these patients, a "one-size-fits-all" approach to therapy will likely not work because not all HCM and DCM patients share the same genetic mutation, or genotype.

In addition, there are many clinical characteristics, or phenotypes, that are driven in part by a patient's individual genetic makeup as well as comorbidities and environmental influences.

The SHaRe Solution

SHaRe will play a key role in helping researchers and clinicians better understand how genotypes and other disease modifiers interact to produce a characteristic range of phenotypes, which will ultimately lead to targeted therapies based on the specific disease-causing genetic mutations in individual patients.

Funded by research grants from MyoKardia, Inc., SHaRe brings together an expert group of cardiologists and geneticists from the U.S., Europe and South America with a passion for helping people with genetic heart disease. With de-identified data on thousands of patients, SHaRe is the largest and most detailed datasets of its kind.

Centers of excellence participating in SHaRe include:

- **Akureyri Hospital, Iceland**
- **Boston Children's Hospital**
- **Brigham and Women's Hospital, Boston, MA**
- **Erasmus University Medical Center, The Netherlands**
- **Florence Centre for Cardiomyopathies, Italy**
- **Laboratory of Genetics and Molecular Cardiology, Sao Paulo, Brazil**
- **Stanford University Medical Center**
- **The Heart Hospital, University College London, UK**
- **University of Michigan Medical Center**
- **Yale School of Medicine**

… and the list is growing. MyoKardia aims to grow the registry into the world's most robust database of clinical information on genetic heart disease through relationships with additional clinical investigators and cardiovascular centers of excellence.

SHaRe will provide researchers and clinicians with new insights and investigational pathways leading to promising new treatments that make a meaningful difference in the lives of patients with genetic cardiomyopathies.

For more information about SHaRe:

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