BSIM Therapeutics Announces Selection of Candidate Compound for Transthyretin Amyloid Cardiomyopathy (ATTR-CM)

November 15th, 2021

BSIM Therapeutics today announced the selection of its first drug candidate, BSIM-3A05D, a compound designed to address hereditary and wild-type transthyretin amyloidosis with cardiomyopathy presentation (ATTR-CM). Driving drug discovery with its proprietary computational platform, BSIM has identified small organic molecules that exert "chaperone" activity and prevent amyloid formation by transthyretin (TTR), a clinically validated target for the treatment of several amyloidoses. Upon completion of Phase-I-enabling studies, the company plans to advance BSIM-3A05D to clinical development. As the company's first discovery program enters preclinical development, the selection of BSIM-3A05D marks a significant corporate milestone in BSIM's internal product pipeline. BSIM-3A05D is one of several compounds the company will continue to evaluate with the goal of targeting different organs and different clinical manifestations of ATTR, and as a means of addressing a range of unmet or underserved needs of ATTR patients.

Current drug treatments for ATTR-CM, both in the market and in clinical development, have paved the way for ATTR pharmacotherapy but either lack broad effectiveness across a genetically-diverse patient population or the cost-efficiency required by long-term treatments starting from initial stages of disease. BSIM believes that the combination of unmatched binding affinity and target engagement of BSIM-3A05D with its optimal pharmacokinetic properties holds high potential for a safe and efficacious treatment of ATTR-CM, and opens up new possibilities for the treatment of a growing population diagnosed with TTR-mediated cardiac amyloidosis.

"Today, it is becoming clear that the earlier the therapeutic intervention and the deeper the reduction in *de novo* deposition of TTR amyloid in the heart, the greater is the chance of attaining the best prognosis in disease management and amyloid regression. Thus, new and more effective treatments will play an essential role in maintaining ATTR-CM under check from the very initial stages of disease", said Rui Brito, CEO of BSIM Therapeutics. "Although we are in the first stages of proving the potential of this compound, we have taken a large step forward in terms of designing a versatile and cost-efficient technology for treating the ATTR-CM patient population."

Commenting on the selection, Carlos Simões, CTO of BSIM Therapeutics added: "ATTR-CM is caused by TTR wild-type and a myriad of TTR variants. Our preclinical data shows that BSIM-3A05D effectively binds to, stabilizes, and prevents amyloid formation by different TTR variants with unprecedented efficiency. Based on its unique pharmacological properties, we see BSIM-3A05D as an effective and safe therapeutic option with the potential to help a large population of ATTR-CM patients of different ages, genetic makeup and disease stages. We are looking forward to advancing BSIM-3A05D through non-clinical and clinical development."

About Transthyretin Amyloidosis with Cardiomyopathy (ATTR-CM)

Transthyretin Amyloidosis with Cardiomyopathy (ATTR-CM) is a progressive and potentially fatal disease characterized by deposition of transthyretin (TTR) amyloid in the heart's myocardium, and leading to heart failure. ATTR-CM may have a hereditary origin or may be spontaneous. In hereditary TTR amyloidosis with cardiomyopathy (hATTR-CM), amyloid deposits in the heart as a result of mutations in the TTR gene that yield less stable TTR variants. hATTR-CM is diagnosed in approximately 40,000 people worldwide, but there are estimates suggesting it may affect more than 200,000 people, and as young as 30 years old. TTR-V122I is the most common TTR variant implicated in hATTR-CM and is present in almost 3 million African Americans. Wild-type TTR amyloidosis (ATTRwt), on the other hand, is a slowly progressing disorder caused by amyloid deposits of normal TTR in the heart. It is found in ca. 80% of males over 80 years old, and it is generally believed that ATTRwt cause cardiac dysfunction and lead to restrictive cardiomyopathy, arrhythmias, and conduction defects in patients over 60 years of age.

Because BSIM-3A05D displays resilience towards the effect of mutations in TTR binding and stabilization, it may be used in the treatment of cardiomyopathies mediated by multiple TTR variants, such as TTR-V122I and TTR-WT.

About BSIM Therapeutics

BSIM Therapeutics is a biotech company focused on the design of organ-targeted, high-quality drug candidates for the treatment of transthyretin-mediated amyloidoses (ATTR). BSIM works close to patients and their physicians to understand and answer to multiple clinical manifestations of ATTR, and employs state-of-the-art computational chemistry and machine learning methods to design novel small molecule therapeutics.

BSIM's team comprises highly motivated, technology and business driven individuals, including scientists with decades of research in the transthyretin amyloidoses field, physicians who follow ATTR patients on a daily basis, and experts in the Biotech sector.

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