FDA Grants Orphan Drug Designation to AISA-021 for Systemic Sclerosis

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The FDA granted orphan drug designation to AISA-021 (cilnidipine; Aisa Pharma, Inc), a fourth-generation calcium channel antagonist, for the treatment of systemic sclerosis (SSc).[1]

"To our knowledge, this is the first time the FDA has granted orphan drug designation to a calcium channel antagonist for an autoimmune illness," Andrew Sternlicht, MD, CEO and founder of Aisa Pharma, said in a news release. "We hope this designation will accelerate our development program for AISA-021, which is designed to provide a once-daily, well-tolerated, and economical treatment that we hope can improve the lives of patients with SSc."[1]

The company also submitted data from the ongoing phase 2 study, demonstrating the improvements of the investigational drug for SSc symptoms of overall disease severity, disease-related pain, gastrointestinal dysfunction, skin ulcers, disability, and breathing-related symptoms. According to the preliminary analysis, the results showed that the 60 patients included had a lower incidence and severity of adverse events. Further, AISA-021 improved efficacy for preventing Raynaud attacks.[1]

SSc, also known as scleroderma, is a connective tissue disorder that has unknown pathogenesis, according to the National Library of Medicine. It has 2 forms—localized (including morphea and linear) and systemic (including limited or diffuse). Localized can affect the skin and subcutaneous tissue while systemic affects internal organ involvement and increased mortality. SSc had shown similarities to other autoimmune diseases, such as complex genetic regions like systemic lupus erythematosus and rheumatoid arthritis. The prevalence is estimated to range between 38 to 341 cases per a million persons with an incidence from 8 to 56 new cases per million persons annually and globally.[2]

According to Mayo Clinic, there is no treatment that can cure or stop the overproduced collagen, but there are treatments that can help control symptoms and complications. There are medications that can help dilate blood vessels to help treat Raynaud symptoms as well as medicines to suppress the immune system to reduce to progression of symptoms, including thickening of skin or worsening lung damage. Furthermore, there are treatments to reduce digestive symptoms, prevent infections, and relieve pain. Other therapies, such as stem cell transplants, can be used for those who have more serious symptoms. Additionally, organ transplants could be considered for severely damaged organs, such as lungs or kidney.[3]

Aisa Pharma Inc will also present 2 abstracts on AISA-021 at the American College of Rheumatology Convergence Scientific Meeting in November 2023 and a pre-investigational new drug application meeting with the FDA is scheduled for September 2024. The RECONNOITER study is a phase 2A trial comprising of 76 patients that assessed the safety and efficacy of the drug alone and in combination with tadalafil for SSc and secondary Raynaud. End points include Raynaud symptoms, gastrointestinal effects, endothelial dysfunction, and overall assessment and well-being. It is conducted in 2 parts—Part A, evaluating dose, safety, and efficacy and Part B, a crossover study to formally evaluate safety and efficacy.[1,4]

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