



BH4 (KUVAN) for Treatment of Systemic Sclerosis Vasculopathy

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Technology description

Invention Summary

Systemic Sclerosis (SSc) SSc is a multisystem autoimmune disorder with a progressively devastating course. There is increased fibroblast activity resulting in abnormal growth of connective tissue causing vascular damage and fibrosis. Fibrosis occurs in skin, the gastrointestinal (GI) tract and other internal organs ;leading to digital ulcerations, blood vessel dilation, severe Raynaud' s phenomenon (narrowing of smaller blood arteries), and gangrene. Presently there is no cure, or effective therapy to improve SSc, nor even a gold standard measurement for disease progression. Delay in SSc diagnosis results in higher morbidity and mortality.

The invention describes a novel approach to treating SSc by targeting Vasculopathy that precedes fibrosis by administration of the drug BH4, tetrahydrobiopterin (KUVAN). BH4 (KUVAN) is currently approved to treat phenylketonuria (PKU), a rare hereditary disorder that leads to the overproduction of the amino acid phenylalanine. Short term study results indicate that BH4 (KUVAN) alleviates SSc symptoms in patients. These include improvement of endothelial function, decreased risk of digital ulcer, increased brachial artery dilation and improved blood flow in their forearm.

Market Opportunity

Each year, 6000 new cases of Scleroderma/Systemic Sclerosis are diagnosed. Current treatment only helps manage the disease representing SSc as an unmet clinical need. Currently SSc patients spend \$12,000 a year on treatments and therapies related to the disease.

Kuvan, also known as sapropterin dihydrochloride, is indicated and approved for the treatment of hyperphenylalaninaemia (HPA) in adult and pediatric patients of 4 years of age and over with phenylketonuria (PKU). This particular drug has been candidate for treatment of peripheral arterial disease, sickle cell anemia, pulmonary arterial hypertension, endothelial dysfunction, and autism. The projected revenue for Kuvan is \$230 million in 2016

Advantages

Repurposing BH4 (KUVAN), FDA approved drug with established safety profile, for oral use, in a new indication SSc

BH4 (KUVAN) has no effect on blood pressure unlike the current methods of treatment for SSc

Kuvan targets the cause of SSc vasculopathy while most treatments only target symptoms
May be safely used in combination with Ca²⁺ blockers and other current standards for treatment of
SSc vasculopathy

Institution

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