BH4 (KUVAN) FOR TREATMENT OF SYSTEMIC SCLEROSIS VASCULOPATHY

THERAPEUTICS
Treatment for systemic sclerosis vasculopathy that improves endothelial function, brachial artery dilation, and blood flow while decreasing risk of developing ulcers.

TECHNOLOGY SUMMARY
Systemic Sclerosis (SSc) is a multisystem autoimmune disorder with a progressively devastating course. Increased fibroblast activity results in abnormal growth of connective tissue that causes vascular damage and fibrosis. Fibrosis occurs in skin, the gastrointestinal (GI) tract, and other internal organs. There is no cure, effective therapy, or gold standard measurement for disease progression. Delay in diagnosis increases morbidity and mortality. BH4 is a novel approach for treating SSc by targeting vasculopathy that precedes fibrosis. BH4 (KUVAN) is currently approved to treat phenylketonuria (PKU). Short term study results indicate that BH4 (KUVAN) alleviates SSc symptoms in patients.

FEATURES AND BENEFITS
- Reduces risk of blood pressure side effects.
- Safe for use in combination with Ca2+ blockers.
- Targets the cause of SSc vasculopathy unlike most treatments that target only symptoms.
- Repurposes drug with FDA approval and established safety profile for use in new indication.

RECENT PUBLICATIONS

INVENTOR PROFILE
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