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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 the risk of developing ulcers.

TECHNOLOGY TYPE

Rare Disease
Drug Repurposing
Imaging
Systemic Sclerosis
Vasculopathy
Rheumatic Disease
Ultrasound

STAGE OF DEVELOPMENT

Tests evaluating the short-term efficacy of BH4 in improving endothelial function of vasculature are ongoing.

IP PROTECTION

Nationalized PCT Pending in the United States and Canada

Methods of Treating
Peripheral Vascular Diseases,
Including Systemic Sclerosis
Vasculopathy
US20190076434A1

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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 the 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

Frech, T., Walker, A.E., Barrett-O'Keefe, Z., Hopkins, P.N., Richardson, R.S., Wray, D.W., Donato, A.J. (2015). Systemic sclerosis induces pronounced peripheral vascular dysfunction characterized by blunted peripheral vasoreactivity and endothelial dysfunction. *Clinical Rheumatology*. 34(5):905-913. doi: [10.1007/s10067-014-2384-5](https://doi.org/10.1007/s10067-014-2384-5)

INVENTOR PROFILE

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