

Oral administration of levocarnitine for treating Sjögren's Syndrome-associated dry eye

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- **WU Reference:** VU19065

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Summary

Sjögren's syndrome (SjS) is a debilitating autoimmune disease, causing dry eye symptoms ranging from discomfort to dysfunction. Vanderbilt researchers have identified levocarnitine as a novel potential therapeutic for treating this condition.

Addressed Need

SjS is one of the most prevalent autoimmune diseases, affecting the exocrine glands that secrete tears and saliva. SjS can cause debilitating dry eye symptoms that significantly impact the patients' quality of life, including ocular discomfort and inflammation, visual dysfunction, and corneal perforation. Current treatment options are limited to topical therapies that provide only temporary relief from symptoms. The proposed technology of oral levocarnitine administration offers a novel approach for treating dry eye symptoms and may also be effective in addressing other symptoms of multiorgan system dryness often found in SjS.

TECHNOLOGY DESCRIPTION

A phenome-wide association study (PheWAS) and chart reviews identified a novel phenotype-genotype association between SjS and an SNP in the gene that encodes the OCTN2 transporter protein for carnitine. Carnitine, which helps protect ocular surfaces, is abundant in healthy tears but significantly reduced in the tears of dry eye patients. Orally administering levocarnitine could therefore potentially overcome decreased OCTN2 activity and ameliorate carnitine deficiency and systemic dryness in SjS patients.

Competitive Advantages

Systemic oral administration of a biologically active carnitine supplement could **alleviate dry eye symptoms**, potentially in both SjS and non-SjS dry eye patients, while also **preventing or slowing disease progression** through all affected organ systems. Furthermore, the SNP identified in the study could be used as a **genetic biomarker** to help identify and treat patients.

Intellectual Property Status:

Technology Development Status:

Patent: WO2021231802

This technology is supported by *in vitro* and *in vivo* data and is currently being validated in a Phase II Pilot trial (NCT03953703). An important next step in the development of this technology would be a commercially viable reformulation to enhance the bioavailability of levocarnitine, which would introduce novel intellectual property.