

Non-Viral Aquaporin-1 Gene Therapy to Restore Salivary Flow

Clinical Need

Radiotherapy is commonly used to treat head-and-neck cancers. Because of the anatomical proximity, salivary glands often receive secondary radiation damage, resulting in xerostomia. While intensity-modulated radiotherapy significantly reduces the incidence of radiation-induced xerostomia, a need still exists for patients suffering from xerostomia.

Solution

Ultrasound-assisted gene transfer is based on sonoporation generated by ultrasound, enabling gene transfer into cells. The delivery of a water channel to glands in a large animal model restored salivary flow post-radiation to pre-treatment levels, demonstrating efficacy of our non-viral gene transfer approach.

Competitive Advantage

While a recent clinical trial using a viral-based AQP1 gene delivery demonstrated an increase in saliva production, this approach has not advanced beyond Phase I/II trial due to side-effects generated by the adenovirus vector. With our non-viral based approach, it is anticipated that enhanced safety is provided in patients with AQP1 gene therapy throughout their lifetime.

Foundational Publications & Patents

- Wang et al. Ultrasound-assisted nonviral gene transfer of AQP1 to the irradiated minipig parotid gland restores fluid secretion. [Gene Ther 2015](#)

Achievements

- Secured non-diluted funding to support GLP toxicology studies

Regulatory Pathway

- IND

Opportunities for Partnerships

- Seeking investment for first-in-human trial



Isabelle Lombaert, PhD
University of Michigan

