Non-Viral Aquaporin1 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.
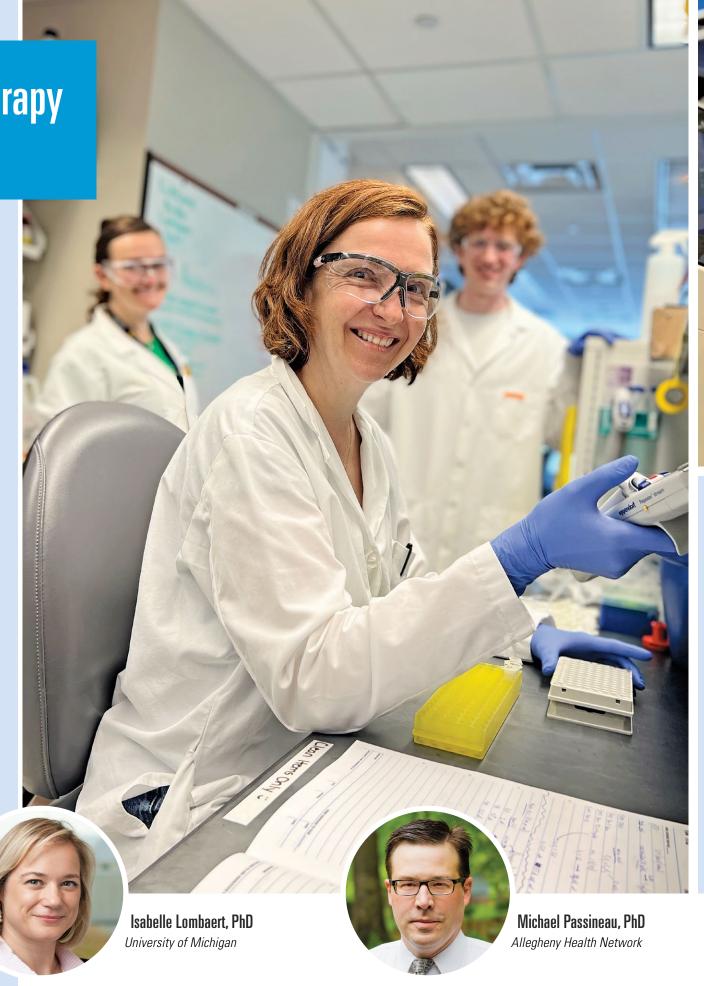
Solution

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

ITP Support

Since the start of the project in the ITP program in 2018, proof-of-concept for the anticipated product/ therapy has been established. The Resource Center Cores are supporting activities to enable the FDA IND submission. The ITP program has helped in deciphering the regulatory and commercialization path, and design/execution of studies needed for the FDA IND submission.

Key Inflection Points/ Regulatory Pathway

- IND submission anticipated in 2024

Opportunities for Partnerships

- Obtaining funds to initiate first-in-human trial

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