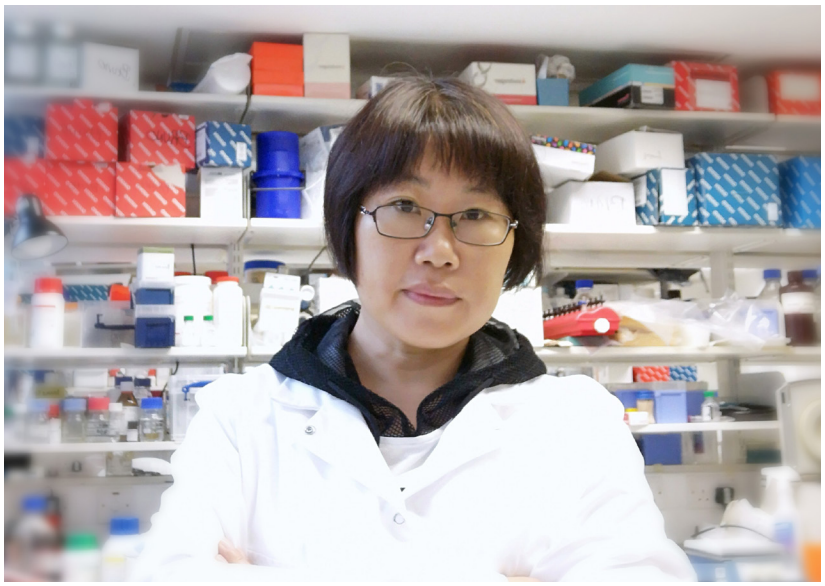

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"From the very first meeting I could feel the wonderful, professional and selfless support being provided by my Harrington Therapeutics Development team."

A NEW APPROACH

BRINGS A FEELING OF HOPE

FOCUS: Developing novel nucleic acid therapy for a hereditary nerve damage disorder.

Hereditary sensory neuropathy type I (HSN1, also known as HSAN1) is a rare disorder of peripheral nerves that results in severe loss of sensation to temperature, pressure and pain. Symptoms typically begin in the late teens and worsen over time, often leading to painless injuries and mutilating skin ulcerations, at times leading to amputation.

This condition has been reported in residents of the United Kingdom, Europe, Australia, Canada and the United States. Currently, no effective treatment for HSN1 is available.

The disorder is most commonly caused by mutations in the SPTLC1 gene, which provides instructions for making one part of an enzyme involved in making certain fats. This genetic error creates a new metabolite that is toxic to nerves and shouldn't stay in the body.

Antisense oligonucleotides (ASOs) are short, synthetic, chemically-modified chains of nucleotides (the building blocks of RNA and DNA) that have the potential to target any gene of interest.

Dr. Zhou and her team aim to develop a new therapy for HSN1 by using ASOs to selectively reduce the production of the toxic metabolites. They are testing specifically-designed RNA compounds in cells from HSN1 patients' skin. If successful, the lead compound(s) will move on to animal models and eventually into humans. Harrington Discovery Institute is helping Dr. Zhou facilitate the preclinical development of these potential therapeutics.

Harrington Discovery Institute
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