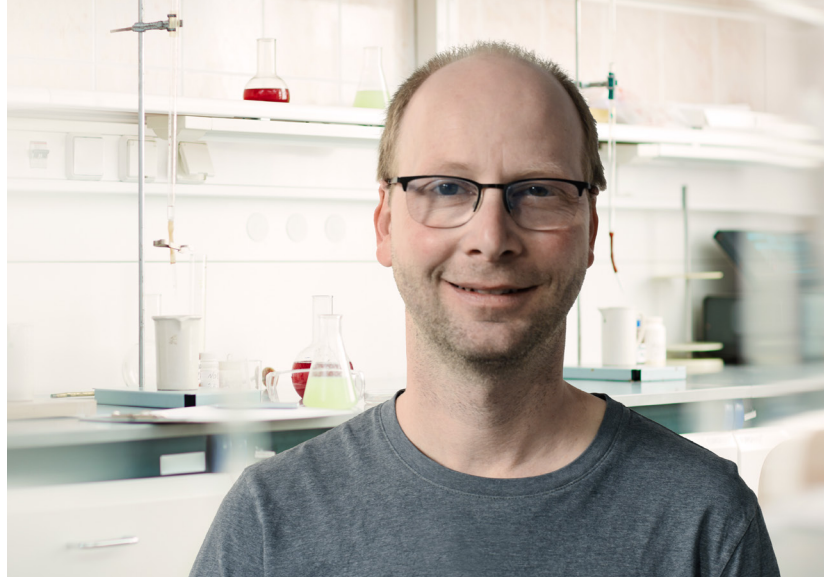


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A MUSCLE-BOUND THERAPY

TO STOP A RARE DISEASE

FOCUS: A muscle-targeted gene therapy for Kennedy's disease, a rare neuromuscular disease.

Kennedy's disease (KD), or Spinal Bulbar Muscular Atrophy, is a rare, slowly progressing adult onset neuromuscular disorder characterized by the degeneration of neurons within the spinal cord and brainstem, accompanied by atrophy of skeletal muscle. Only males are affected, developing progressive weakness and wasting of muscles in arms and legs, as well as in the facial region, resulting in difficulty with swallowing and speech. There are currently no effective treatments.

A genetic disease, KD is caused by an abnormal expansion of DNA in the androgen receptor gene (AR) found on the X chromosome. Studies have shown that silencing the androgen receptor gene in muscles is able to fully rescue the disease. However, this approach may reduce AR in other tissues and organs, inducing significant side effects.

"We are developing a virally-delivered gene therapy approach where the AR gene is silenced only in muscles,"

Dr. Fratta says. "Thus, benefits would be provided to the neuromuscular symptoms while avoiding the threat of side-effects. This method would require just one dosage, which is a godsend for such a long-term condition."

As Dr. Fratta's lab is among the largest KD clinics in the world, they are also working on providing biomarkers and outcome measures that will allow for more effective clinical trials with this disease.

"Our Harrington team is brilliant—and so involved, following the project step by step."

Harrington Discovery Institute

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