CMT Research Foundation Invests in Asha Therapeutics to Test Novel Drug for CMT2A

ATLANTA, GA, UNITED STATES, May 20, 2025 /EINPresswire.com/ -- CMT Research Foundation has invested in a research project with Asha Therapeutics to investigate the therapeutic potential of inhibiting SARM1 (Sterile alpha and TIR motif-containing protein 1) using their novel drug, ASHA-624, for the treatment of Charcot-Marie-Tooth disease type 2A.

CMT2A is caused by mutations in the MFN2 gene, which provides instructions for producing the mitofusin 2 protein — essential for mitochondrial fusion. Mutations in MFN2 lead to mitochondria dysfunction and nerve axon degeneration.

SARM1 is a driver of axonal degeneration — the breakdown of nerve fibers — which plays a role in many neurodegenerative diseases, including CMT2A. SARM1 is normally kept in an inactive state, but can become activated when stress or damage occurs, such as mitochondrial dysfunction, triggering axon degeneration in affected nerve cells.

As part of the research collaboration, Asha Therapeutics will evaluate the effectiveness of targeting SARM1 in CMT2A using patient-derived cells and a preclinical animal model of CMT2A. Recent studies have shown that genetic removal of SARM1 prevented disease development in an animal model of CMT2A, suggesting that pharmacological inhibition of SARM1 could potentially be an effective therapeutic approach.

“We deeply value the support of CMT Research Foundation in evaluating our asset’s novel method of SARM1 inactivation in CMT2A,” said Dr. Bradlee Heckmann, Asha Therapeutics’ president and chief scientific officer. “Asha’s research partnership with CMT Research Foundation will advance critical understandings into ASHA-624’s efficacy and safety for patients with CMT2A, expanding the patient impact potential of SARM1 inactivation across a growing number of neurodegenerative and rare diseases towards the design of disease modifying and curative medicines.”

ASHA-624 acts as an “intramolecular glue,” binding two different regions of the SARM1 protein to block its activation. This mechanism differs from other SARM1 inhibitors being developed and provides more selectivity, potentially overcoming key limitations seen with less targeted approaches.

"We are thrilled to partner with Asha Therapeutics to advance this promising therapeutic

candidate for CMT2A," said Dr. Riann Egusquiza, CMTRF's director of research. "Their novel approach to inhibiting SARM1 offers a highly targeted and potentially transformative strategy for halting nerve degeneration, with the potential to impact not only CMT2A, but a range of neurodegenerative diseases that include other forms of CMT."

Asha Therapeutics is currently advancing their lead candidate, ASHA-624, toward clinical trials for ALS, chemotherapy-induced peripheral neuropathy, and other neurodegenerative conditions, and has shown promising preclinical results.

About the CMT Research Foundation: The CMT Research Foundation is a patient-led, non-profit organization dedicated exclusively to funding research that will lead to treatments and a cure for Charcot-Marie-Tooth disease. By focusing on high-impact, results-driven research, CMTRF partners with leading scientists, biotech companies and investors to bring promising therapies to clinical trials faster. For more information, visit www.cmtrf.org.

About Asha Therapeutics: Asha Therapeutics (www.ashatherapeutics.com) is a life sciences company at the forefront of a new era of precision drug design, leveraging the power of its proprietary PRISM™ technology to custom design de novo compounds to create disease-modifying and curative therapeutics for neurological, neuro-oncological and rare diseases with high unmet medical need.

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