



MDA Awards Venture Philanthropy Grant to Locana to Develop Novel Treatment for Muscular Dystrophy

NEW YORK and SAN DIEGO – June 12, 2019 – The Muscular Dystrophy Association (MDA) and Locana, a leading RNA-targeting gene therapy company, today announced the award of an MDA Venture Philanthropy (MVP) grant totaling \$550,000 to advance Locana’s development program for myotonic dystrophy (DM).

MVP is the Muscular Dystrophy Association’s drug development program, which is exclusively focused on funding the discovery and clinical application of treatments and cures for neuromuscular disorders. MVP evaluates and makes targeted investments in for-profit and not-for-profit companies and academics developing therapeutics.

“We’ve witnessed incredible innovation with the development of the first FDA-approved gene therapy for a neuromuscular disease,” said Lynn O’Connor Vos, MDA’s president and CEO. “This gives us hope that novel approaches — such as Locana’s for designing highly specific RNA-targeting therapeutic candidates — for treatment of myotonic dystrophy can address the significant unmet needs for patients who live with this genetic disease. We have partnered with Locana to further accelerate their efforts to realize benefits in these patients.”

Locana’s Vice President of Research and Development, Ranjan (Ron) Batra, Ph.D., will serve as the principal investigator on the project. In Locana’s proof of concept studies published in [Cell](#), its approach demonstrated *in vivo* safety and efficacy, including the reversal of molecular and behavioral features of DM.

“We appreciate the support of MDA to address this devastating disease,” said Jeffrey M. Ostrove, Ph.D., chief executive officer of Locana. “DM is caused by expression of dysfunctional, repetitive RNA in diseased tissues, where application of Locana’s core RNA-targeting technology has been shown to have potential for single-dose therapeutic benefit and could provide a long-lasting approach for patients.”

About Myotonic Dystrophy

Myotonic dystrophy is an autosomal dominant genetic disorder and the most common form of adult-onset muscular dystrophy. It is a multisystemic disorder with diseases affecting skeletal muscle, cardiac muscle, the gastrointestinal tract, and the central nervous system. Patients exhibit an inability to relax muscle, termed myotonia (which is the hallmark phenotype of disease), along with muscle atrophy, weakness, excessive daytime sleepiness, cataracts, etc. The incidence of myotonic dystrophy has historically been estimated at one in 8,000 individuals worldwide or approximately 40,000 people in the United States, although more recent studies indicate the incidence may be as much as threefold higher.

About Locana, Inc.

Founded in 2016 and based in San Diego, California, Locana is an RNA-targeting gene therapy company with a platform technology to address a wide spectrum of human genetic diseases.



Locana has advanced a powerful modular RNA targeting-effector approach to optimize and advance therapeutic candidates that is distinct from DNA-targeted approaches and nucleic acid-based RNA targeting. Locana intends to build a portfolio of therapies that address the root cause of genetic diseases driven by dysfunctional RNA behavior. For more information, visit www.locanabio.com.

About the Muscular Dystrophy Association

MDA is committed to transforming the lives of people affected by muscular dystrophy, ALS, and related neuromuscular diseases. We do this through innovations in science and innovations in care. As the largest source of funding for neuromuscular disease research outside of the federal government, MDA has committed more than \$1 billion since our inception to accelerate the discovery of therapies and cures. Research we have supported is directly linked to approved, life-changing therapies across multiple neuromuscular diseases. We support the largest network of multidisciplinary clinics providing best-in-class care at more than 150 of the nation's top medical institutions, and each year thousands of children and young adults learn vital life skills and gain independence at MDA Summer Camp and through recreational programs. For more information visit mda.org.

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