Clene Nanomedicine Presents Interim Results from REPAIR-MS and REPAIR-PD Phase 2 Studies

Presentation today during Joint ACTRIMS-ECTRIMS Meeting at MSVirtual2020

Results provide first clinical evidence demonstrating catalytic effects of CNM-Au8 in multiple sclerosis and Parkinson's disease patients

SALT LAKE CITY, September 11, 2020 - Clene Nanomedicine, Inc., a clinical-stage biopharmaceutical company, today announced the presentation of interim results from the REPAIR-MS and REPAIR-PD Phase 2 studies demonstrating the effects of its lead nanocatalytic therapeutic, CNM-Au8. The preliminary data demonstrate CNM-Au8-mediated modulation of key brain bioenergetic metabolites in relapsing multiple sclerosis (MS) and Parkinson's disease (PD) patients. The data will be available starting September 11 at 8:00 a.m. ET as an on-demand e-Poster at the MSVirtual2020: 8th Joint ACTRIMS-ECTRIMS (Americas Committee for Treatment and Research in MS – European Committee for Treatment and Research in MS), held online September 11-13 (www.msvirtual2020.org).

The presentation (P0206) titled, "Effects of Nanocatalysis on CNS Bioenergetic Markers in Patients Treated with CNM-Au8: Interim Results from Two Phase 2 ^{31}Phosphorous Imaging Studies," highlights target engagement data from the ongoing REPAIR imaging trials. REPAIR-MS and REPAIR-PD are Phase 2, open-label, sequential group, investigator-blinded studies being conducted at the University of Texas Southwestern Medical Center. The study utilizes high resolution magnetic resonance spectroscopy (^{31}P-MRS) to explore the ability of orally delivered CNM-Au8 to improve the metabolic profile in the brains of patients with MS (REPAIR-MS) and PD (REPAIR-PD).

Interim results from 4 MS and 6 PD completers demonstrate significant central nervous system (CNS) target engagement of CNM-Au8. The data indicate catalytic bioenergetic improvements across important CNS bioenergetic metabolites including total nicotinamide adenine dinucleotide (NAD) levels, NAD+/NADH ratio, and adenosine triphosphate (ATP) levels, indicating a homeostatic effect of CNM-Au8 on brain bioenergetics. Full analyses will be conducted once the Phase 2 trials are complete.

"We generally receive the same questions regarding CNM-Au8: how do you know that CNM-Au8 gets to the brains of people, especially people with neurodegenerative diseases, and if it does, how do you know it will have bioenergetic effects in patients? We believe these interim data represent the first clinical evidence of CNM-Au8's ability to positively impact key bioenergetic metabolic markers in the brains of MS and PD patients, two diseases in which CNS bioenergetic failure is known to play a significant role in disease pathophysiology," said Robert Glanzman, MD, FAAN, Chief Medical Officer of Clene.

"These data from REPAIR-MS and REPAIR-PD indicate that CNM-Au8 is working mechanistically to address a foundational challenge common to many neurodegenerative diseases, namely that stressed or failing neurons need additional energy for their survival, repair, and improved function. We now have insights that CNM-Au8 is driving bioenergetics within the brain, which is a foundational insight for the further development of Clene's neurorepair clinical programs. Results from these and our other ongoing studies aim to establish Clene as a pioneer in therapeutic neurorepair and neuroprotection. We believe these preliminary data may suggest the potential for CNM-Au8 to treat millions of individuals worldwide suffering from MS and PD," said Rob Etherington, President and CEO of Clene.

About REPAIR-MS and REPAIR-PD

REPAIR-MS and REPAIR-PD are Phase 2, single-center, open label, sequential group studies examining the brain metabolic effects, safety, pharmacokinetics, and pharmacodynamics of CNM-Au8 in patients who have been diagnosed with MS within 15 years of screening or in patients with PD who have been diagnosed within three years of screening. Investigators and participants are blinded to dose, which can consist of a 15 or 30 mg orally delivered dose of the nanocrystal suspension daily each morning for 12 weeks. Participants undergo ^{31}P-MRS brain imaging scan to semi-quantitatively measure bioenergetic brain metabolites at baseline, prior to administration of drug, and at the end-of-study. The objective of this study is to demonstrate target engagement for CNM-Au8 on central nervous system biomarkers related to bioenergetics and neuronal metabolism in patients with MS and PD. The study is taking place at University of Texas Southwestern Medical Center with a team of internationally recognized experts in brain imaging and treatment of disorders of the central nervous system.

About CNM-Au8

CNM-Au8 is a concentrated, aqueous suspension of clean-surfaced faceted gold nanocrystals that act catalytically to support important intracellular biological reactions. CNM-Au8 consists solely of nanoparticles of gold, composed of clean-surfaced, faceted, geometrical crystals held in suspension in sodium bicarbonate buffered, pharmaceutical grade water. CNM-Au8 has demonstrated safety in Phase 1 studies in healthy volunteers and has shown both remyelination and neuroprotection effects in multiple preclinical (animal) models. Preclinical data, both published in peer-reviewed journals and presented at scientific congresses demonstrate that treatment of neuronal cultures with CNM-Au8 improves survival of neurons, protects neurite networks, decreases intracellular levels of reactive oxygen species, and improves mitochondrial capacity in response to cellular stresses induced by multiple disease-relevant neurotoxins. Oral treatment with CNM-Au8 improved functional behaviors in rodent models of ALS, multiple sclerosis, and Parkinson's disease versus vehicle (placebo). CNM-Au8 is currently being tested in a Phase 2 clinical study for the treatment of chronic optic neuropathy in patients with multiple sclerosis in addition to Phase 2 and Phase 3 clinical studies for disease progression in patients with amyotrophic lateral sclerosis (ALS).

About Multiple Sclerosis (MS)

MS is an inflammatory, demyelinating disease of the central nervous system and is the most common (non-traumatic) cause of neurological disability in young adults. The most common clinical presentation, relapsing MS (RMS), is characterized by sub-acute attacks of neurological disability, ranging from loss of vision to numbness and tingling, walking difficulty, dizziness, and/or paralysis. Most people with RMS are diagnosed between the ages of 20 and 40, with three times more women being affected than men. A recent study led by the National MS Society estimates that nearly 1 million people are living with MS in the United States. Despite currently available disease-modifying therapies, approximately 30% of people with MS have developed a non-active, progressive form of the disease, for which there are no approved, effective therapies, leading to significant loss of quality of life. There remains an urgent need for therapies that promote repair, neuroprotection and remyelination for all people with MS.

About Parkinson's Disease

Parkinson's disease, a progressive central nervous system disorder, is the second most common neurodegenerative disorder, affecting approximately 1.2% of the world population over the age of 70. PD predominately affects dopaminergic neurons—the neurons in the brain that make dopamine. Loss of dopaminergic neurons results in tremor, slowness, stiffness, difficulty walking, and balance problems.

PD may also be associated with non-movement related symptoms, such as constipation, depression, and memory problems.

About Clene

Clene Nanomedicine, Inc. is a privately-held, clinical-stage biopharmaceutical company focused on the development of unique therapeutics for neurodegenerative diseases. Clene has innovated a novel nanotechnology drug platform for the development of a new class of orally administered neurotherapeutic drugs. Clene has also advanced into the clinic an aqueous solution of ionic zinc and silver for anti-viral and anti-microbial uses. Founded in 2013, the company is based in Salt Lake City, Utah with R&D and manufacturing operations located in North East, Maryland. For more information, please visit www.clene.com.

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