Clene Nanomedicine Presents Updated Blinded Efficacy Data from Ongoing Phase 2 RESCUE-ALS Study at the ENCALS 2021 Annual Meeting

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Preliminary blinded efficacy data suggest CNM-Au8 may have the potential for neuro-repair in amyotrophic lateral sclerosis (ALS) patients

Patients demonstrated improvement from baseline, as compared to the expected continuous decline seen in published data from prior observational studies

Unblinded topline data expected 2H 2021

SALT LAKE CITY, May 12, 2021 (GLOBE NEWSWIRE) -- Clene Inc. (NASDAQ: CLNN) (along with its subsidiaries, “Clene”) and its wholly owned subsidiary Clene Nanomedicine, Inc., a clinical-stage biopharmaceutical company dedicated to the treatment of neurodegenerative disease using bioenergetic nanocatalysis, today announced the presentation of updated blinded interim efficacy data from its ongoing Phase 2 RESCUE-ALS clinical trial. The Phase 2 RESCUE-ALS trial is evaluating the efficacy, safety, pharmacokinetics, and pharmacodynamics of CNM-Au8 in the treatment of early symptomatic ALS patients. The data, presented during the European Network to Cure ALS (ENCALS) 2021 Annual Meeting on May 12-14, provide further evidence supporting the disease modifying potential of CNM-Au8 for the treatment of ALS.

CNM-Au8 is the first therapeutic bioenergetic nanocatalyst in development for neurodegenerative diseases that has a unique multi-modal mechanism of action that addresses ALS disease-related bioenergetic failure, oxidative stress, and impaired proteostasis. The RESCUE-ALS study’s primary efficacy endpoint utilizes Motor Unit Number Index (MUNIX), a sophisticated electromyography technique that is a sensitive predictor of clinical decline which measures the change in the estimated number of functioning motor neurons serving specific muscle.

The data presented at the ENCALS 2021 Annual Meeting includes blinded analyses showing the enrolled cohort performed better than expected on the primary endpoint, change from baseline for the MUNIX(4)sum which represents the sum of MUNIX values for the abductor digiti minimi, abductor pollicis brevis, tibialis anterior, and biceps brachii muscles. In the overall study population (n = 45; randomized 1:1 active CNM-Au8 30 mg daily to placebo), 34%, 26%, and 18% of patients who completed Weeks 12, 24, and 36, respectively, demonstrated MUNIX(4)sum increases (improvement) from baseline, which differs from the expected continuous decline seen in published data from prior observational studies[1].

Correlation analyses showed that subjects demonstrating MUNIX(4)sum improvements also had less ALS Functional Rating Scale-Revised (ALSFRS-R) and forced vital capacity (FVC) worsening. The mean reduction in FVC for the overall study population was approximately 11% (absolute change of the % predicted) at Week 24 (n=42), which is generally less decline than anticipated based on published data sets[2].

These blinded data suggest that CNM-Au8 may have the potential for neuro-repair in ALS patients. Clene expects to report unblinded topline data from the RESCUE-ALS study in the second half of 2021.

“We are encouraged by this update from our ongoing Phase 2 trial of CNM-Au8 for the treatment of ALS. While blinded, we believe these data support CNM-Au8’s potential to become a breakthrough for this devastating disease. We look forward to delivering unblinded topline results by the end of this year,” said Rob Etherington, President and Chief Executive Officer of Clene. “Concurrent with our advancement of the RESCUE-ALS study, we are also advancing CNM-Au8 through the Phase 3 HEALEY ALS Platform trial. Through the parallel execution of these clinical programs, we aim to facilitate CNM-Au8’s clinical development and hopefully one day shift the paradigm in how we can treat and target the underlying bioenergetic deficits common to ALS patients.”

Robert Glanzman, MD, FAAN, Chief Medical Officer of Clene added, “ALS is a progressive and devastating neurodegenerative disease affecting the brain and spinal cord. Today, there is no cure and current treatments have only modest impact on maintaining quality of life for patients and their families. It is our hope that this encouraging blinded update from our RESCUE-ALS study, in the midst of ALS Awareness Month, reassures the ALS community that companies like Clene are working diligently to advance innovation in this space.”

The presentation titled, “A Blinded Interim Update on RESCUE-ALS: A Randomized, Placebo-Controlled, Phase 2 Study to Determine the Effects of CNM-Au8 to Slow Disease Progression in Amyotrophic Lateral Sclerosis,” is available as a live e-Poster at the ENCALS 2021 Annual Meeting, held online (https://www.encals.eu/meetings/encals-meeting-2021/). It is also available on the “Events and Presentations” section of the Clene website.

About RESCUE-ALS

RESCUE-ALS is a Phase 2 multi-center, randomized, double-blind, parallel-group, placebo-controlled study examining the efficacy, safety, pharmacokinetics and pharmacodynamics of CNM-Au8 in patients with early amyotrophic lateral sclerosis (ALS). The trial completed enrollment in 2H 2020. 45 subjects were randomized 1:1 to receive either active treatment with CNM-Au8 (30 mg) or placebo in addition to their current standard of care over a 36-week treatment period. The objective of the study is to assess the impact of improving neuronal bioenergetics, reducing reactive oxygen species, and promoting protein homeostasis with CNM-Au8 on disease progression in patients with early ALS. CNM-Au8 was selected by FightMND of Australia and Clene was provided a substantial grant to investigate efficacy in ALS utilizing novel electromyography endpoints at two expert sites in Australia. Topline data are expected in 2H 2021. For more information, please see ClinicalTrials.gov Identifier: NCT04098406.

About CNM-Au8

Clene’s lead drug candidate, CNM-Au8, a bioenergetic nanocatalyst, is a stable, aqueous suspension of catalytically active gold (Au) nanocrystals. Resulting from a patented manufacturing breakthrough, the self-organized, clean surfaced nanocrystals of CNM-Au8 drive critical cellular bioenergetic reactions in the brain that increase cellular energy, enhance neurorepair, and improve neuroprotection. CNM-Au8 crosses the blood-brain barrier and is not associated with the toxicities related to synthetic gold compounds or nanoparticles manufactured via alternative methods. CNM-Au8 is currently

being evaluated in a Phase 3 registration trial in amyotrophic lateral sclerosis (ALS), a Phase 2 trial examining disease progression via a novel electromyography technique in patients with early ALS, a Phase 2 trial for the treatment of chronic optic neuropathy in patients with stable relapsing multiple sclerosis (MS), and Phase 2 brain target engagement studies in patients with Parkinson's disease (PD) and MS. CNM-Au8 has demonstrated safety in Phase 1 studies in healthy volunteers and has shown both remyelination and neuroprotective 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 numerous disease-relevant neurotoxins. Oral treatment with CNM-Au8 improved functional behaviors in rodent models of ALS, MS, and PD versus vehicle (placebo).

About Clene

Clene, a clinical-stage biopharmaceutical company focused on neurodegenerative disease, is leading the way by using nanotechnology to treat bioenergetic failure, which underlies many neurological diseases. Clene has innovated a novel nanotherapeutic platform to create a new class of drugs—bioenergetic nanocatalysts. Clene's lead drug candidate, CNM-Au8, is a concentrated nanocrystalline gold (Au) suspension that drives critical cellular bioenergetic reactions in the CNS. CNM-Au8 increases cellular energy to accelerate neurorepair and improve neuroprotection. Currently, CNM-Au8 is being investigated for efficacy and safety in a Phase 3 registration trial for ALS and in Phase 2 trials for multiple sclerosis and Parkinson's disease. Clene has also advanced into the clinic an aqueous solution of ionic zinc and silver for anti-viral and anti-microbial uses. The company is based in Salt Lake City, Utah with R&D and manufacturing operations in Maryland. For more information, please visit www.clene.com.

Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Clene's actual results may differ from its expectations, estimates and projections and consequently, you should not rely on these forward-looking statements as predictions of future events. Words such as "expect," "estimate," "project," "budget," "forecast," "anticipate," "intend," "plan," "may," "will," "could," "should," "believes," "predicts," "potential," "might" and "continues," and similar expressions are intended to identify such forward-looking statements. These forward-looking statements involve significant known and unknown risks and uncertainties, many of which are beyond Clene's control and could cause actual results to differ materially and adversely from expected results. Factors that may cause such differences include Clene's ability to demonstrate the efficacy and safety of its drug candidates; the clinical results for its drug candidates, which may not support further development or marketing approval; actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials and marketing approval; Clene's ability to achieve commercial success for its marketed products and drug candidates, if approved; Clene's ability to obtain and maintain protection of intellectual property for its technology and drugs; Clene's reliance on third parties to conduct drug development, manufacturing and other services; Clene's limited operating history and its ability to obtain additional funding for operations and to complete the licensing or development and commercialization of its drug candidates; the impact of the COVID-19 pandemic on Clene's clinical development, commercial and other operations, as well as those risks more fully discussed in the section entitled "Risk Factors" in Clene's Annual Report filed on Form 10K, as well as discussions of potential risks, uncertainties, and other important factors in Clene's subsequent filings with the U.S. Securities and Exchange Commission. Clene undertakes no obligation to release publicly any updates or revisions to any forward-looking statements to reflect any change in its expectations or any change in events, conditions or circumstances on which any such statement is based, subject to applicable law. All information in this press release is as of the date of this press release. The information contained in any website referenced herein is not, and shall not be deemed to be, part of or incorporated into this press release.

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Source: Clene Inc.

[1] Neuwirth et al. J Neurol Neurosurg Psychiatry. 2015 Nov;86(11):1172-9.
[2] Andrews et al. JAMA Neurol. 2018 Jan 1;75(1):58-64.

Source: Clene Inc.