Clene Nanomedicine Announces Top-Line Results from Phase 2 RESCUE-ALS Clinical Trial

November 2, 2021

- ***RESCUE-ALS Phase 2 Trial did not meet primary MUNIX biomarker endpoint or secondary FVC endpoint at week 36; MUNIX efficacy signal was observed at week 12 (p=0.057)***
- ***MUNIX trial results demonstrated protection of lower motor neurons in the pre-specified subset of limb onset ALS subjects (Wk12, p=0.0385; Wk36, p=0.0741), which represents approximately 70% of the ALS population***
- ***Statistically significant reductions in clinically relevant outcomes including ALS disease progression (p=0.0125), ALSFRS-R responder analysis (p=0.035), and improved ALS specific quality of life (p=0.018)***
- ***Evidence for potential long-term survival benefit***
- ***Results for Healey ALS Platform Trial Expected in the Second Half of 2022***

SALT LAKE CITY, Nov. 02, 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 focused on revolutionizing the treatment of neurodegenerative disease with its potential first-in-class catalytically active nanocrystal suspension, today announced top-line data from RESCUE-ALS, a Phase 2 clinical trial evaluating CNM-Au8 as a disease modifying treatment for people with early amyotrophic lateral sclerosis (ALS).

The trial did not meet the primary or secondary endpoints – Motor Unit Number Index (MUNIX) and forced vital capacity (FVC) – at week 36. However, an efficacy signal was observed for the MUNIX endpoint at week 12 (p=0.057). Furthermore, in a pre-specified analysis in the subset of limb onset ALS, CNM-Au8 demonstrated a significant treatment effect in MUNIX at week 12 (p=0.0385) and a trend for improvement at week 36 (p=0.0741). Limb onset ALS accounts for approximately 70% of the ALS population. MUNIX is a neurophysiological biomarker that estimates the number of functioning lower motor neurons serving selected muscles.

Clinically relevant exploratory endpoints through trial week 36 demonstrated significant benefits with CNM-Au8 treatment, including slowing ALS disease progression (p=0.0125), decreasing the proportion of participants with an ALS Functional Rating Scale Revised (ALSFRS-R) 6-point decline (p=0.035), and improving quality of life as measured by the ALS Specific Quality of Life (ALSSQOL-SF) (p=0.018). In addition, RESCUE-ALS showed evidence for a potential long-term survival benefit when comparing the survival of the trial population to the validated ENCALS predictive model[1].

“These data are very encouraging to us in the ALS research and treatment community as they demonstrate clinical benefits with CNM-Au8 treatment in outcomes that matter to patients and provide evidence for improved long-term survival,” said Professor Matthew Kiernan, AM MBBS(Hons), PhD, DSc, FRACP, FAHMS, Bushell Chair of Neurology, University of Sydney and one of the trial’s clinical advisors. “RESCUE-ALS was a proof-of-concept trial intended to establish that treatment of neuronal energetic failure can provide disease-modifying effects in ALS. I am pleased to see the potential effectiveness of CNM-Au8 demonstrated in this trial, and it is important to confirm these results in a larger clinical trial.”

Rob Etherington, Clene’s Chief Executive Officer, stated, “We believe these results show the potential of CNM-Au8 to bring meaningful benefit to people living with ALS. Befitting of Lou Gehrig, whose legacy is intertwined with the disease, we swung for the fences and ended with a stand-up triple. In the second half of next year, we expect to report results from the HEALEY ALS Platform Trial with the objective of confirming CNM-Au8 as an effective disease-modifying therapy for people with ALS.”

RESCUE-ALS was a 36-week randomized, placebo-controlled Phase 2 clinical trial that enrolled 45 patients with early ALS, randomized 1:1 to treatment with CNM-Au8 at 30 mg daily or matching placebo on top of standard of care. The primary endpoint of the trial was the percent change of the sum of MUNIX from baseline to week 36. Secondary endpoints were the change in FVC and the absolute change in MUNIX values to week 36. Exploratory endpoints included multiple clinically relevant measures of ALS: disease progression, ALSFRS-R decline, and ALSSQOL-SF.

CNM-Au8 was found to be well-tolerated through 36 weeks of oral daily dosing. There were no reported serious adverse events (SAEs) related to CNM-Au8 treatment. Treatment-emergent adverse events were predominantly mild-to-moderate in severity. The most frequently reported adverse events associated with CNM-Au8 treatment included aspiration pneumonia (n=3) and transient gastrointestinal distress (n=2). Topline results from RESCUE-ALS are expected to be presented at the upcoming International Symposium on ALS/MND, which will take place Dec. 8-10, 2021.

Robert Glanzman, MD, FAAN, Clene’s Chief Medical Officer, concluded, “The results of RESCUE-ALS add to our expanding body of evidence that cellular energetic failure is an important pathophysiological mechanism in ALS. We thank the trial participants and their families for their willingness to engage in clinical research, the site investigators for their research excellence and dedication to patients, and FightMND of Australia for substantially funding the trial.”

Bec Sheean, Research Director of FightMND commented, “FightMND is committed to finding new treatment options for motor neurone disease. We awarded Clene a grant in 2019 to support the conduct of this Phase 2 clinical trial with CNM-Au8 in Australia. These clinical trial results are encouraging for people with ALS. By achieving clinically relevant endpoints, this trial demonstrates the potential for CNM-Au8 to support neuronal health. We are excited to see CNM-Au8 continue its advancement into the clinic so that we can bring this potentially transformative treatment to patients.”

Conference Call and Webcast Details
Clene will host a conference call and live audio webcast today at 8:00 a.m. ET to discuss the Phase 2 RESCUE-ALS topline clinical trial results. The live webcast may be accessed from the Investors section of the Company's website at www.clene.com. Please connect to the website prior to the start of the conference call to ensure adequate time for any software downloads that may be necessary. Individuals may participate in the conference call by dialing +1 800 309 3488 in the U.S., or +1 929 517 9012 outside the U.S., and using conference ID: 5894034.

An archived version of the webcast will be available for at least one week in the Investors section of the Company's website at www.clene.com.

About CNM-Au8®, a gold nanocrystal suspension

Clene's lead drug candidate, CNM-Au8, a catalytically active gold nanotherapeutic, is the result of a patented manufacturing breakthrough. The catalytically active nanocrystals of CNM-Au8 drive critical cellular energy producing reactions in the brain that enable neuroprotection and remyelination by increasing neuronal and glial resilience to disease-relevant stressors. 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 being evaluated in a Phase 3 registration trial for the treatment of amyotrophic lateral sclerosis (ALS). In the REPAIR Program Phase 2 open-label biomarker clinical trials, CNM-Au8 demonstrated target engagement in the treatment of Parkinson's disease (PD) and multiple sclerosis (MS). REPAIR-PD has concluded, and REPAIR-MS will continue with the initiation of a second MS dosing cohort. 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). CNM-Au8® is a federally registered trademark of Clene Nanomedicine, Inc.

About RESCUE-ALS

RESCUE-ALS is a Phase 2 multi-center, randomized, double-blind, parallel-group, placebo-controlled trial examining the efficacy, safety, pharmacokinetics and pharmacodynamics of CNM-Au8 in patients with early amyotrophic lateral sclerosis (ALS). The trial completed enrollment in the second half of 2020. In the trial, 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 trial is to assess the impact of CNM-Au8 on disease progression in patients with early-stage ALS through changes in motor unit index MUNIX. MUNIX values were evaluated for four muscles in the hand, arm, and leg: the abductor digiti minimi, abductor pollicis brevis, tibialis anterior and biceps brachii from baseline through 36 weeks of treatment. CNM-Au8 was selected by FightMND of Australia, and Clene was provided a substantial grant to investigate efficacy in ALS utilizing novel neurophysiological endpoints at two clinical sites in Australia. For more information, please see ClinicalTrials.gov Identifier: NCT04098406.

About FightMND

FightMND is a not-for-profit registered charity, founded in 2014. It was established to raise the awareness of Motor Neurone Disease (MND) in Australia, to increase funding for research to find an effective treatment and cure and to provide care equipment for MND patients. FightMND has a clear objective – to have a world free from MND.

FightMND is Australia's largest independent MND foundation focused on funding large-scale, collaborative research and clinical trials. The generous donations contributed by everyday Australians, right across the country, has enabled FightMND to raise and commit millions to cure and care initiatives.

About Clene

Clene is a clinical-stage biopharmaceutical company focused on revolutionizing the treatment of neurodegenerative disease with potential first-in-class nanotherapeutics to treat energetic failure, an underlying cause of many neurological diseases. Our lead drug candidate, CNM-Au8, is an oral suspension of gold nanocrystals that drive critical cellular energetic metabolism in the central nervous system (CNS). CNM-Au8 increases energy production and utilization to accelerate neurorepair and improve neuroprotection. CNM-Au8 is currently being evaluated in a Phase 3 registration trial in amyotrophic lateral sclerosis (ALS) and a Phase 2 trial for the treatment of chronic optic neuropathy in patients with stable relapsing multiple sclerosis (MS). 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 or follow us on Twitter, LinkedIn and Facebook.

Forward-Looking Statements

This press release contains "forward-looking statements" which are intended to be covered by 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 on Form 10-K, 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] Westeneng et al. Lancet Neurol. 2018 May;17(5):423-433.

Source: Clene Inc.