Clene Provides Update on Its NIH-Funded Expanded Access Program for CNM-Au8® in ALS (ACT-EAP)

May 28, 2024

- First patient, first visit is planned for early June 2024
- Enrollment has been expanded by 80% to a maximum of 180 participants
- 'Real-world' drug efficacy data will be collected by monitoring potential drug effects on survival and disease progression, as well as safety

SALT LAKE CITY, May 28, 2024 (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 improving mitochondrial health and protecting neuronal function to treat neurodegenerative diseases, including amyotrophic lateral sclerosis (ALS) and multiple sclerosis (MS), today announced several key updates on its National Institutes of Health (NIH)-funded Accelerating Access to Critical Therapies Expanded Access Program (ACT-EAP) for CNM-Au8® compassionate use in ALS.

Late last year, in collaboration with Columbia University and Synapticure, Clene announced the award of a four-year grant from the National Institute of Neurological Disorders and Stroke (NINDS), part of the NIH, to support an EAP for the Company's investigational drug, CNM-Au8 in ALS.

ALS is a deadly neurodegenerative disease with no known cure. In recognizing the need for accessibility and inclusion in such programs for individuals with ALS, Clene instituted significant manufacturing, operational, and personnel efficiencies to expand the program's accommodation to up to 180 participants.

"The Clene team recognized that there was great need in the ALS community for more access to promising treatments," Austin Rynders, VP Clinical Operations at Clene, reflected on this effort. "Everyone at Clene from Clinical Operations to Manufacturing, from Medical Affairs to HR, put forth an extraordinary effort to expand the size of the ACT-EAP without increasing the size of the budget."

In addition to meeting the requisite safety standards set by the FDA for all EAPs, Clene's ACT-EAP was designed to contribute research results to the public understanding of ALS disease progression and response to therapy. To this end, the clinical teams at Clene, Columbia, and Synapticure designed the ACT-EAP for CNM-Au8 with the following additional objectives:

1. To add 'real-world' drug exposure data to the growing clinical safety database on CNM-Au8
2. To assess 'real-world' drug efficacy data by monitoring potential drug effects on survival and disease progression with the use of natural history and control clinical trial database comparators. Biomarker data will also be collected and analyzed in parallel.

"CNM-Au8 treatment has been associated with lowered risk of death and delayed clinical worsening – all while being very well-tolerated, in both of our independent Phase 2 clinical studies," added Rob Etherington, CEO of Clene. "Data from EAP programs, which usually include a broader ALS population than clinical trials, can supplement the safety and other meaningful data gathered from clinical studies. We are grateful for the NIH's recognition to fund this important initiative and are pleased to provide this EAP for the ALS community. We are also grateful to the FDA for approving this EAP, working alongside Columbia University and Synapticure, to enable people who are living with this devastating disease access to our investigational drug CNM-Au8."

"It's important to note that this is not just a standard EAP, in which we provide investigational product to individuals with ALS through an FDA-regulated program," explained Dr. Jinsy A. Andrews, MD, MSc, FAAN, of Columbia University, and Principal Investigator on the grant. "A lot of time, careful thought, and effort have resulted in a study that will yield important insights into the real-world impact of CNM-Au8 treatment on the disease course of ALS."

"We are pleased to be the first-ever fully virtual clinical site to participate in an EAP - or any clinical study for that matter," said Peter Wallach, Chief Financial Officer and Co-Founder of Synapticure. "By leveraging our telehealth platform, Synapticure extends the reach of expanded access programs to people throughout the US. With a nationwide presence, we enable people living with ALS access to investigational medicines like CNM-Au8 who have not previously had the option."

The first patient first visit for the ACT-EAP is planned for June 2024. More information on the CNM-Au8 ACT-EAP can be accessed here: https://clenecompassionateuse.com/

This study is supported by NIH (1U01NS136023-01).

About Expanded Access Programs

An EAP is also referred to as Compassionate Use and is an FDA-regulated pathway that allows people with a serious and life-threatening disease to access an investigational drug that is not yet approved by the U.S. Food and Drug Administration (FDA). Funds supporting EAPs in ALS were made available with the passing into law by Congress and President Biden of the Accelerating Access to Critical Therapies for ALS Act (ACT for ALS) in December of 2021.

About Clene

Clene Inc., (Nasdaq: CLNN) (along with its subsidiaries, “Clene”) and its wholly owned subsidiary Clene Nanomedicine Inc., is a late clinical-stage biopharmaceutical company focused on improving mitochondrial health and protecting neuronal function to treat neurodegenerative diseases, including amyotrophic lateral sclerosis, Parkinson’s disease, and multiple sclerosis. CNM-Au8 ® is an investigational first-in-class therapy that improves central nervous system cells’ survival and function via a mechanism that targets mitochondrial function and the NAD pathway while reducing oxidative stress. CNM-Au8® is a federally registered trademark of Clene Nanomedicine, Inc. 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 X (formerly Twitter) and LinkedIn.

Forward-Looking Statements

This press release contains “forward-looking statements” within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended, and Section 27A of the Securities Act of 1933, as amended, which are intended to be covered by the “safe harbor” provisions created by those laws. Clene’s forward-looking statements include, but are not limited to, statements regarding our or our management team’s expectations, hopes, beliefs, intentions or strategies regarding our future operations. In addition, any statements that refer to projections, forecasts or other characterizations of future events or circumstances, including any underlying assumptions, are forward-looking statements. The words “anticipate,” “believe,” “contemplate,” “continue,” “estimate,” “expect,” “intends,” “may,” “might,” “plan,” “possible,” “potential,” “predict,” “project,” “should,” “will,” “would,” and similar expressions may identify forward-looking statements, but the absence of these words does not mean that a statement is not forward-looking. These forward-looking statements represent our views as of the date of this press release and involve a number of judgments, risks and uncertainties. We anticipate that subsequent events and developments will cause our views to change. We undertake no obligation to update forward-looking statements to reflect events or circumstances after the date they were made, whether as a result of new information, future events or otherwise, except as may be required under applicable securities laws. Accordingly, forward-looking statements should not be relied upon as representing our views as of any subsequent date. As a result of a number of known and unknown risks and uncertainties, our actual results or performance may be materially different from those expressed or implied by these forward-looking statements. Some factors that could cause actual results to differ include our ability to demonstrate the efficacy and safety of our drug candidates; the clinical results for our 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; our ability to achieve commercial success for our drug candidates, if approved; our limited operating history and our ability to obtain additional funding for operations and to complete the development and commercialization of our drug candidates; and other risks and uncertainties set forth in “Risk Factors” in our most recent Annual Report on Form 10-K and any subsequent Quarterly Reports on Form 10-Q. In addition, statements that “we believe” and similar statements reflect our beliefs and opinions on the relevant subject. These statements are based upon information available to us as of the date of this press release, and while we believe such information forms a reasonable basis for such statements, such information may be limited or incomplete, and our statements should not be read to indicate that we have conducted an exhaustive inquiry into, or review of, all potentially available relevant information. These statements are inherently uncertain and you are cautioned not to rely unduly upon these statements. 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.

Media Contact
Ignacio Guerrero-Ros, Ph.D., or
David Schull
Russo Partners, LLC
Ignacio.guerrero-ros@russopartnersllc.com
David.schull@russopartnersllc.com
(858) 717-2310

Investor Contact
Kevin Gardner
LifeSci Advisors
kgardner@lifesciadvisors.com
(617) 283-2856