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Clinical-stage biopharmaceutical company Clene Inc. announced proof-of-concept evidence of potential neuroprotective effects of CNM-Au8, an oral therapeutic for patients with stable relapsing-remitting MS.

According to a release from Clene, data from the VISIONARY-MS trial were presented at the 14th Congress of the Pan-Asian Committee for Treatment and Research in Multiple Sclerosis Nov. 24 to 26 in Singapore.

The phase 2, randomized, double-blind, placebo-controlled trial included patients with stable relapsing-remitting MS and chronic optic neuropathy. Participants received 15 or 30 mg per day of CNM-Au8 — an oral suspension of gold nanocrystals developed to restore neuronal health and function — or placebo over 48 weeks. Ninety-two percent of participants also received disease-modifying therapy as standard of care, per the release.

According to Clene, the primary study endpoint — change in low contrast letter acuity compared with placebo at week 48 — demonstrated significant improvement (modified intent-to-treat population, least squares mean difference = 3.13; 95% CI, –0.08 to 6.33). Another outcome, the modified Multiple Sclerosis Functional Composite, also showed significant improvement (mITT population, LS mean difference = 0.28; 95% CI, 0.04-0.52).

Other improvements reported by Clene for CNM-Au8 were multifocal visual evoked potential amplitude and latency, measurements of retinal structure with optical coherence tomography and novel MRI endpoints examining myelin and axonal integrity.

In addition, placebo-treated patients generally worsened across clinical and paraclinical measures during the trial duration, while CNM-Au8 was well-tolerated and no significant safety findings were observed, the release stated.

“Remyelination and neuroprotection are key unmet needs for patients with multiple sclerosis,” **Michael Barnett, MBBS, FRACP, PhD**, of the University of Sydney in Australia, said in the release. “The phase 2 VISIONARY-MS trial results demonstrated promising efficacy of the cellular energetic nanocatalyst, CNM-Au8, across remyelination and neuroprotection domains. When these results are confirmed by a future, larger phase 3 study, CNM-Au8 would be a remarkable advance for patients with MS as an adjunct to conventional anti-inflammatory DMTs.”

Earlier this year, per the release, the trial was stopped prematurely due to COVID-19 pandemic operational challenges after enrolling just 73 of the 150 planned participants.

“Despite the operational challenges presented by COVID and the primary endpoint marginally exceeding the traditional P = 0.05 statistical threshold, Clene and its MS expert advisers believe these results strongly support the hypothesis that improving brain energetic metabolism results in improved neurological function when CNM-Au8 is administered as adjunct to standard immunomodulatory disease-modifying MS therapies,” Michael Hotchkin, Clene’s chief development officer, said in the release.

Clene plans to initiate a phase 3 study to demonstrate improved global neurological function in patients with progression independent of relapse activity after consulting with regulatory authorities, the company stated in the release.

Editor's Note: This article was updated on Dec. 5, 2022, to correct an affiliation.