Issue Date: 16 July 2023

TauRx's hydromethylthionine mesylate (HMTM) demonstrates significant reduction in neurodegeneration in Alzheimer's Disease (AD)

- Results from a prespecified analysis of the Phase 3 LUCIDITY trial show reduction in neurodegeneration biomarker (NfL) in AD for people receiving 16 mg/day of HMTM compared to controls
- HMTM is an oral drug with a strong safety profile
- Results announced in an oral presentation at the Alzheimer's Association International Conference (AAIC) 2023

TauRx Pharmaceuticals Ltd., a global leader in Tau-based research in Alzheimer's disease (AD), today announced results from a prespecified analysis of the Phase 3 LUCIDITY trial that measured the impact of HMTM on neurofilament light chain (NfL), an established biomarker for brain neurodegeneration. Blood concentration of NfL showed a statistically significant 93% reduction in change over 12 months in participants receiving HMTM at a dose of 16 mg/day relative to the control group, which correlated significantly with a tau biomarker (p-tau 181) in blood.

Neurofilaments and tau proteins are essential for neuronal structure and function in the brain. In AD, tau protein aggregates to form toxic fibrils, which damage neurons. The extent of this damage can be measured by the amount of leakage of neurofilament protein into the bloodstream. NfL concentration in blood is known to correlate with tau pathology, disease severity, and therefore cognitive decline and brain atrophy in AD. HMTM, a tau aggregation inhibitor, was designed to reduce tau pathology in AD. Changes in NfL concentration by HMTM indicate a direct impact on disease pathology.

"NfL is a well-studied biomarker with wide applicability to different neurological disorders, including AD," said Henrik Zetterberg, Professor of Neurochemistry, UCL Queen Square Institute of Neurology. "Clinical practice has been waiting decades to uncover meaningful advancements to address unmet needs of people with AD. These new results further support the importance of NfL as an AD biomarker both for diagnosis and measurement of treatment effect."

"The NfL results demonstrate that a drug targeting tau pathology reduces the neurodegeneration that underlies clinical decline in AD," said Claude Wischik, Executive Chairman, TauRx. "They bring us a step closer to offering an effective new treatment option for people with AD. Because it is taken as a tablet and has a strong safety profile, HMTM would be readily accessible to people needing a disease modifying treatment."

TauRx will submit the HMTM results from LUCIDITY and earlier trials for regulatory approval in the US, UK and other territories.

For additional information, please visit: https://taurx.com/ or https://aaic.alz.org/program/scientific-sessions.asp.

THE LUCIDITY TRIAL
LUCIDITY comprised a 12-month double-blind controlled Phase 3 clinical trial followed by a 12-month period in which all participants received HMTM at 16 mg/day. The trial investigated change in various clinical and biomarker outcomes comparing HMTM 16 mg/day with methylthioninium chloride (MTC) given 4 mg twice weekly as a control over the first 12 months. NfL was the principal blood biomarker endpoint in this study. The LUCIDITY trial is now complete and TauRx is preparing publications to report both the NfL data and the full 24-month data.

TAU PATHOLOGY IN AD
Age-related factors lead to misfolding and aggregation of tau proteins, and the subsequent formation of tau tangles in AD. Tau aggregation begins many years before symptoms of dementia appear. Tau aggregation pathology correlates with the clinical decline (loss of memory and ability to care for oneself) commonly seen in people with AD, establishing it as an important target for treatment. HMTM works by inhibiting pathological aggregation of tau protein.

ABOUT TAURX PHARMACEUTICALS LTD
TauRx was founded in 2002 in Singapore, with primary research facilities and operation based in Aberdeen, UK. The company has dedicated the past two decades to developing treatments and diagnostics for Alzheimer's and other neurodegenerative diseases due to protein aggregation pathology.

Alzheimer's disease is a leading cause of disability and death throughout the world and is one of the most important public health issues that need to be addressed globally. TauRx will contribute to addressing this unmet need with data from LUCIDITY and pursuit of regulatory approvals in line with its overall plans to make HMTM available to patients and pursue clinical development in other related neurodegenerative diseases.
https://www.taurx.com

CONTACT
Rebecca Andersen
r.andersen@taurx.com
+44 (0)7979 082304