MapLight Therapeutics Announces Results from Phase 1 Trial of Novel M1/M4 Muscarinic Agonist in Development for Treatment of Schizophrenia and Alzheimer's Disease Psychosis

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- Favorable safety and tolerability profile in healthy adult and elderly volunteers at doses planned for use in the upcoming Phase 2 trials
- Exposures maintained at or above anticipated clinically relevant levels with both once- and twice-daily dosing
- Plan to initiate Phase 2 trials for schizophrenia and Alzheimer's disease psychosis in the first half of 2025

SAN FRANCISCO AND BOSTON, DECEMBER 2, 2024 – MapLight Therapeutics, a clinical-stage biopharmaceutical company focused on improving the lives of patients suffering from debilitating brain disorders, today announced results from its Phase 1 trial evaluating the safety, tolerability and pharmacokinetics of ML-007/PAC in healthy adult and elderly volunteers. With the completion of this study, ML-007 has been evaluated, both as a single agent and in combination with a peripherally acting anticholinergic (PAC), across four Phase 1 trials with 270 subjects enrolled. The company plans to initiate Phase 2 trials of ML-007/PAC in schizophrenia and Alzheimer's disease psychosis (ADP) in the first half of 2025.

"The new class of muscarinic agonists has the potential to address a broad range of brain disorders, and is the first new approved pharmacological approach to treat schizophrenia in decades. We believe there is a significant opportunity for novel entrants within the class to offer improved tolerability, convenient dosing and comprehensive symptom improvement," said Christopher Kroeger, M.D., M.B.A., the Chief Executive Officer and Founder of MapLight. "ML-007/PAC has demonstrated a favorable safety and tolerability profile with once- and twice-daily dosing, while achieving exposures anticipated to be clinically relevant, and has the potential to be a best-in-class M1/M4 muscarinic agonist."

Study Design:

The study assessed the safety, tolerability and pharmacokinetics of an extended-release, fixed-dose combination of ML-007/PAC. The study enrolled 82 healthy adult and elderly volunteers across four cohorts, with subjects dosed for up to 14 days. The study was designed to inform the dosing regimen for the company's planned Phase 2 trials in schizophrenia and ADP.

Results:

ML-007/PAC was generally well tolerated at the doses planned for the Phase 2 clinical trials. Most treatment-emergent adverse events (TEAEs) were mild and transient in nature. No severe or serious adverse events were observed. Plasma and CSF exposures were maintained above anticipated clinically relevant levels with both once- and twice-daily dosing regimens. The plasma concentration ratio between ML-007 and the PAC remained within the target range established to minimize adverse events over the duration of the intended dosing interval. Pharmacokinetic parameters were similar between adult and elderly volunteers and confirmed that the dosing in the planned Phase 2 trials will not require administration in a fasted state.

"We are encouraged by the favorable safety and tolerability profile demonstrated across our Phase 1 trials and feel confident in progressing to the next phase of clinical development," said Erin Foff, M.D., Ph.D., the Chief Medical Officer of MapLight. "The results of this study provided critical data informing the final dosing and administration protocols for our planned Phase 2 trials in schizophrenia and ADP. We are excited to move one step closer to potentially giving patients and their caregivers a better treatment option."

About ML-007/PAC

ML-007/PAC, also referred to as ML-007C-MA, is an extended-release, fixed-dose combination of the investigational M1/M4 muscarinic agonist, ML-007, co-formulated with a peripherally acting anticholinergic (PAC). Potential differentiation for ML-007/PAC is driven by an optimized pharmacokinetic synchronization of its agonist and antagonist components in the periphery, without sacrificing strong activation of both M1 and M4 receptors in the central nervous system.

About MapLight

MapLight Therapeutics is a clinical-stage biopharmaceutical company focused on improving the lives of patients suffering from debilitating central nervous system disorders. MapLight was founded by globally recognized leaders in psychiatry and neuroscience research to address the lack of circuit-specific pharmacotherapies available for patients. MapLight's discovery platform holds the potential to fill this void by identifying neural circuits causally linked to disease and targeting those circuits for therapeutic modulation. Learn more at www.maplightrx.com.

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