Sage Therapeutics Announces European Medicines Agency Granted SAGE-718 Orphan Drug Designation for the Treatment of Huntington's Disease

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CAMBRIDGE, Mass.--(BUSINESS WIRE)--Feb. 22, 2023-- Sage Therapeutics, Inc. (Nasdaq: SAGE), a biopharmaceutical company leading the way to create a world with better brain health, today announced the European Medicines Agency (EMA) granted Orphan Drug Designation to SAGE-718 for the treatment of Huntington's disease (HD). SAGE-718 is in development as a potential oral therapy for cognitive disorders associated with NMDA receptor dysfunction. Multiple clinical studies are ongoing with SAGE-718 across several disease areas, including two placebo-controlled Phase 2 studies and a Phase 3 open-label safety study in the lead indication of HD-related cognitive impairment, and additionally Phase 2 placebo-controlled studies in mild cognitive impairment (MCI) associated with Parkinson's disease (PD) and MCI and mild dementia due to Alzheimer's disease (AD).

"Huntington's disease is a serious, debilitating condition that interferes with daily functioning in the prime years of life and is associated with significant morbidity and early mortality, and yet there are currently no approved therapies for the treatment of HD-related cognitive impairment," said Laura Gault, M.D., Ph.D., Chief Medical Officer, Sage Therapeutics. "With this exciting development of Orphan Drug Designation from the EMA, we have further momentum in our efforts to address this unmet need. Our goal with SAGE-718 is to provide rapid, meaningful, and sustained improvement in cognitive functioning early in the disease so that patients can maintain independence longer."

Orphan drug designation is reserved for medicines treating rare, life-threatening, or chronically debilitating diseases that meet certain specified criteria. Potential benefits for Sage as a result of this designation include Protocol Assistance or scientific advice specifically designed for orphan medicines, European centralized authorization procedure, if development is successful and a marketing authorization application is filed, and if approved, ten years of market exclusivity and protection from similar medicines with the same indication being approved.

About SAGE-718

SAGE-718, Sage's first-in-class NMDA receptor positive allosteric modulator (PAM) and lead neuropsychiatric drug candidate, is in development as a potential oral therapy for cognitive disorders associated with NMDA receptor dysfunction, potentially including HD, PD and AD. Sage is advancing a robust clinical program for SAGE-718 with multiple ongoing placebo-controlled Phase 2 studies across multiple disease areas, including its potential lead indication, cognitive impairment associated with HD, as well as cognitive impairment due to AD and PD. The Company is also conducting a Phase 3 safety extension study in HD cognitive impairment. SAGE-718 has received Fast Track Designation from the FDA for the potential treatment of HD.

About Sage Therapeutics

Sage Therapeutics is a biopharmaceutical company fearlessly leading the way to create a world with better brain health. Our mission is to pioneer solutions to deliver life-changing brain health medicines, so every person can thrive. For more information, please visit http://www.sagerx.com.

Forward-Looking Statements

Various statements in this release concern Sage's future expectations, plans and prospects, including without limitation our statements regarding: the goals, potential benefit, and potential indications for SAGE-718; the potential benefit of ODD granted to SAGE-718 by the EMA; the potential for success of this programs, and the opportunity to help patients; and the mission and goals for our business. These statements constitute forward-looking statements as that term is defined in the Private Securities Litigation Reform Act of 1995. These forward-looking statements are neither promises nor guarantees of future performance, and are subject to a variety of risks and uncertainties, many of which are beyond our control, which could cause actual results to differ materially from those contemplated in these forward-looking statements, including the risks that: we may not seek or achieve some or any of the benefits associated with orphan drug designation of SAGE-718 by the EMA; we may not be successful in the development of SAGE-718 or of any of our other product candidates; success in earlier clinical trials may not be repeated or observed in ongoing or future studies, and ongoing and future clinical trials may not meet their primary or key secondary endpoints which may substantially impair development; unexpected concerns may arise from additional data, analysis or results from any of our completed studies; we may encounter adverse events at any stage that negatively impact further development or the potential for approval or the potential for successful commercialization, if approved, or that require additional nonclinical and clinical work which may not yield positive results; we may encounter delays in initiation, conduct, completion of enrollment or completion of our ongoing and planned clinical trials, including as a result of slower than expected site initiation, slower than expected enrollment, the need or decision to expand the trials or other changes, that may impact our ability to meet our expected timelines and increase our costs; decisions or actions of the FDA or other regulatory agencies may affect the initiation, timing, design, size, progress and cost of clinical trials and our ability to proceed with further development or may impair the potential for successful development; and we may encounter technical and other unexpected hurdles in the development and manufacture of SAGE-718 or our other product candidates or the commercialization of any current or future marketed product which may delay our timing or change our plans, increase our costs or otherwise negatively impact our business; as well as those risks more fully discussed in the section entitled "Risk Factors" in our most recent quarterly report, as well as discussions of potential risks, uncertainties, and other important factors in our subsequent filings with the Securities and Exchange Commission. In addition, any forward-looking statements represent our views only as of today, and should not be relied upon as representing our views as of any subsequent date. We explicitly disclaim any obligation to update any forward-looking statements.

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