Sage Therapeutics Announces U.S. Food and Drug Administration Granted SAGE-718 Orphan Drug Designation for the Treatment of Huntington's Disease

October 18, 2023

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 18, 2023-- Sage Therapeutics, Inc. (Nasdaq: SAGE), a biopharmaceutical company leading the way to create a world with better brain health, today announced the U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation (ODD) 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 potential lead indication of HD-related cognitive impairment, as well as 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 devastating condition that often affects patients in their prime years, and it can significantly impact a patient's ability to live independently. Cognitive impairment is one of the most underrecognized aspects of this disease," said Laura Gault, M.D., Ph.D., Chief Medical Officer, Sage Therapeutics. "There are currently no approved treatments to address cognitive impairment for people with HD and a growing sense of urgency among researchers and people living with HD to address cognitive impairment early so that patients can maintain independence longer. The ODD designation from the FDA provides continued momentum in our efforts to help patients and their families impacted by this aspect of HD."

Orphan drug designation is granted by the FDA Office of Orphan Products Development to assist and encourage companies to develop safe and effective therapies for the treatment of rare diseases and disorders. Under the Orphan Drug Act, the FDA may provide grant funding towards clinical trial costs, tax advantages, FDA user-fee benefits, and the potential for seven years of market exclusivity in the United States for the drug in the orphan indication following drug approval by the FDA. The approval of an orphan designation request does not alter the standard regulatory requirements and process for obtaining marketing approval. For more information about orphan designation, please visit the FDA website at www.fda.gov.

SAGE-718 previously received Fast Track Designation from the FDA for HD, and orphan drug designation for HD by the European Medicines Agency.

About SAGE-718
SAGE-718, a first-in-class investigational NMDA receptor positive allosteric modulator (PAM), is in development as a potential oral therapy for cognitive disorders associated with NMDA receptor dysfunction, including HD, PD and AD. Sage is advancing a 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 open-label safety study in HD cognitive impairment.

About Huntington's disease and cognition
HD is a rare, inherited neurodegenerative disease that progresses over time and affects up to an estimated 40,000 adults in the U.S. each year. Cognitive impairment can severely affect people with HD. There are currently no treatment options available to improve the cognitive effects of the disease.

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 FDA; the estimated number of people with HD in the U.S.; 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 FDA; 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 or cause us to terminate further work; 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, lengthen the regulatory pathway for approval or negatively impact our ability to proceed with further development or may in other ways impair the potential for successful development; even if we are successful in our development efforts and re*ceive FDA approval of SAGE-718 in HD with orphan exclusivity that prevents others from gaining approval of the same drug in this indication during the exclusivity period, competitors may receive approval of different products for HD; our estimates of the number of people with HD and the unmet need for new options to improve treatment may prove not to be correct; 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20231018464957/en/

Investor
Ashley Kaplowitz
786-252-1419
ashley.kaplowitz@sagerx.com

Media
Matthew Henson
917-930-7147
Matthew.Henson@sagerx.com

Source: Sage Therapeutics, Inc.