Axsome Therapeutics to Host R&D Day with Key Opinion Leaders Focused on AXS-05 and Unmet Needs in Alzheimer's Disease Agitation

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NEW YORK, Sept. 19, 2018 (GLOBE NEWSWIRE) -- Axsome Therapeutics, Inc. (NASDAQ:AXSM), a clinical-stage biopharmaceutical company developing novel therapies for the management of central nervous system (CNS) disorders, today announced that it will host a research and development (R&D) day with key opinion leaders (KOLs) on Thursday, October 18, 2018 in New York City. The event will highlight AXS-05, the company's most advanced CNS product candidate, and unmet needs in agitation associated with Alzheimer's disease (AD). AXS-05 is in a Phase 2/3 randomized, double-blind, controlled trial (the ADVANCE-1 study) in patients with agitation associated with AD. An interim analysis for futility of the ADVANCE-1 study is anticipated in the fourth quarter of 2018.

The event will feature presentations from Jeffrey Cummings, MD, ScD (Cleveland Clinic) who will discuss the prevalence and outcomes of neuropsychiatric symptoms of AD, and Clive Ballard, MD, MSc (University of Exeter) who will discuss the challenges in the current management of agitation associated with AD and possible future pharmacotherapeutic directions, including the potentially relevant pharmacology of AXS-05 for this indication. The presenters will be available to answer questions following their presentations.

The Axsome executive management team will also provide an update on the ongoing clinical development of AXS-05 as well as on the rest of the Company's CNS pipeline.

KOL Presenters:

- **Jeffrey Cummings, MD, ScD.** Director of the Center for Neurodegeneration and Translational Neuroscience; Director Emeritus, Cleveland Clinic Lou Ruvo Center for Brain Health; and Professor of Neurology, at the Cleveland Clinic Lerner College of Medicine

- **Clive Ballard, MD, MRCPsych, MSc.** Pro-Vice Chancellor and Executive Dean of Medicine, University of Exeter

AXS-05 is a novel, oral, investigational medicine consisting of dextromethorphan (an NMDA receptor antagonist, sigma-1 receptor agonist, and serotonin and norepinephrine reuptake inhibitor) and bupropion (a norepinephrine and dopamine reuptake inhibitor, which also increases the bioavailability of dextromethorphan), under development for the treatment of CNS disorders. In addition to the Phase 2/3 trial in agitation associated with AD, AXS-05 is also being evaluated in a Phase 3 trial in treatment resistant depression (TRD), a Phase 2 trial in major depressive disorder, and a Phase 2 trial in smoking cessation. AXS-05 has been granted U.S. Food and Drug Administration Fast Track designations for TRD and for agitation associated with AD.

This event is intended for institutional investors, sell-side analysts, and business development professionals only. Please RSVP in advance if you plan to attend, as space is limited. To reserve a seat email Kimberly Kenney at kkenney@axsome.com.

A live and archived webcast of the event, with slides, can be accessed on the investor page of Axsome's website at www.axsome.com.

KOL Credentials

- **Jeffrey Cummings, MD, ScD**

  Dr. Jeffrey Cummings is the Director of the Center for Neurodegeneration and Translational Neuroscience and Director Emeritus of the Lou Ruvo Center for Brain Health at the Cleveland Clinic Neurological Institute. He holds a faculty position as Professor of Medicine at the Cleveland Clinic Lerner College of Medicine of Case Western Reserve University. He has previously held numerous faculty and directorship positions at the UCLA school of Medicine, Boston University School of Medicine, University of Nevada College of Liberal Arts, University of Nevada Las Vegas School of Medicine, and the Department of Veterans Affairs. He is past President of the Behavioral Neurology Society and of the American Neuropsychiatric Association.

  Dr. Cummings originated the Neuropsychiatric Inventory (NPI), and he has published over 700 articles and over 40 books. He is the recipient of numerous awards including the Bengt Winblad Lifetime Achievement Award in Alzheimer's Research and the Ronald and Nancy Reagan Research Award from the national Alzheimer's Association.

- **Clive Ballard, MD, MSc**

  Professor Ballard has held faculty positions at King's College's Institute of Psychiatry as Professor of Age-Related Diseases, Co-Director of the Biomedical Research Unit for Dementia, and Co-Director of the Wolfson Center for Age-Related Disease; University of Newcastle as Professor of Old Age Psychiatry; University of Birmingham as a Lecturer in Psychiatry; and, currently, as Pro-Vice Chancellor and Executive Dean of Medicine at the University of Exeter. He has also served as Director of Research for the Alzheimer's Society.

  Author of over 500 articles and investigator on more than 25 clinical trials, Prof. Ballard is an internationally recognized researcher, lecturer, and teacher in psychiatry with subspecialty expertise in geriatric populations and dementia.

About the ADVANCE-1 Study

ADVANCE-1 (Addressing Dementia Via Agitation-Centered Evaluation 1) is a Phase 2/3 multicenter, randomized, double-blind, controlled trial to evaluate the efficacy and safety of AXS-05 in patients with agitation associated with Alzheimer's disease. Approximately 435 patients will be randomized in a 1:1:1 ratio to receive AXS-05, bupropion, or placebo for 5 weeks. The primary efficacy measure is the Cohen-Mansfield Agitation Inventory (CMAI). The trial incorporates two interim analyses to be performed by an independent data monitoring committee. The first interim analysis will be performed on the first approximately 30% of the target number of subjects to assess futility. The second interim analysis will be performed on the first approximately 60% of the target number of subjects to assess efficacy.

About Alzheimer's Disease (AD) Agitation

Alzheimer's disease (AD) is a progressive neurodegenerative disorder that manifests initially as forgetfulness advancing to severe cognitive impairment and memory loss. It afflicts an estimated 5 million individuals in the United States, a number that is anticipated to increase to approximately 14 million by 2050. In addition to cognitive decline, individuals diagnosed with AD frequently experience behavioral and psychological symptoms including agitation which is reported in approximately 45% of patients. Agitation is characterized by emotional distress, aggressive behaviors, disruptive irritability, and disinhibition. Agitation in patients with AD has been associated with increased caregiver burden, decreased functioning, earlier nursing home placement, and increased mortality. There are currently no therapies approved by the FDA for the treatment of agitation in patients with AD.

About AXS-05

AXS-05 is a novel, oral, investigational drug product under development for the treatment of central nervous system (CNS) disorders. AXS-05 consists of bupropion and dextromethorphan and utilizes Axsome's metabolic inhibition technology. Dextromethorphan is an NMDA receptor antagonist, sigma-1 receptor agonist, nicotinic acetylcholine receptor antagonist, and inhibitor of the serotonin and norepinephrine transporters. Bupropion serves to increase the bioavailability of dextromethorphan, and is a norepinephrine and dopamine reuptake inhibitor, and a nicotinic acetylcholine receptor antagonist. AXS-05 is an investigational drug product not approved by the FDA.

About Axsome Therapeutics, Inc.

Axsome Therapeutics, Inc. is a clinical-stage biopharmaceutical company developing novel therapies for the management of central nervous system (CNS) disorders for which there are limited treatment options. Axsome's product candidate portfolio includes five clinical-stage candidates, AXS-02, AXS-05, AXS-06, AXS-07, and AXS-09. AXS-05 is currently in a Phase 3 trial in treatment resistant depression (TRD) and a Phase 2/3 trial in agitation associated with Alzheimer's disease (AD), a Phase 2 trial in Major Depressive Disorder (MDD), and a Phase 2 trial in smoking cessation. AXS-02 is currently in a Phase 3 trial in knee osteoarthritis (OA) associated with bone marrow lesions (BMLs) with an additional Phase 3 trial planned in chronic low back pain (CLBP) associated with Modic changes (MCs). AXS-07 is being developed for the acute treatment of migraine. AXS-06 is being developed for the treatment of osteoarthritis and rheumatoid arthritis and for the reduction of the risk of NSAID-associated gastric ulcers. AXS-02, AXS-05, AXS-06, AXS-07, and AXS-09 are investigational drug products not approved by the FDA. For more information, please visit the company website at www.axsome.com. The company may occasionally disseminate material, nonpublic information on the company website.

Forward Looking Statements

Certain matters discussed in this press release are "forward-looking statements". The Company may, in some cases, use terms such as "predicts," "believes," "potential," "continue," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "will," "should" or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. In particular, the Company's statements regarding trends and potential future results are examples of such forward-looking statements. The forward-looking statements include risks and uncertainties, including, but not limited to, the success, timing and cost of the Company's ongoing clinical trials and anticipated clinical trials for its current product candidates, including statements regarding the timing of initiation, interim analyses and completion of the trials; the timing of and the Company's ability to obtain and maintain U.S. Food and Drug Administration or other regulatory authority approval of, or other action with respect to, its product candidates; the Company's ability to successfully defend its intellectual property or obtain the necessary licenses at a cost acceptable to the Company, if at all; the successful implementation of the Company's research and development programs and collaborations; the success of the Company's license agreements; the acceptance by the market of the Company's product candidates, if approved; and other factors, including general economic conditions and regulatory developments, not within the Company's control. The factors discussed herein could cause actual results and developments to be materially different from those expressed in or implied by such statements. The forward-looking statements are made only as of the date of this press release and the Company undertakes no obligation to publicly update such forward-looking statements to reflect subsequent events or circumstance.

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