



May 14, 2015

## SAGE Therapeutics Announces SAGE-547 Progress and First Quarter 2015 Financial Results

*SAGE-547 Achieves 77 Percent Response Rate in Completed Phase 1/2 Clinical Trial of SAGE-547 in SRSE*

*Conference Call Scheduled Today at 8:30 a.m. ET*

CAMBRIDGE, Mass., May 14, 2015 (GLOBE NEWSWIRE) -- SAGE Therapeutics (Nasdaq:SAGE), a clinical-stage biopharmaceutical company developing novel medicines to treat life-threatening, rare central nervous system (CNS) disorders, today reported business highlights and financial results for the first quarter ended March 31, 2015.

"This year has been transformational for SAGE. We continue to advance the development of our new medicines for CNS disorders where there are significant unmet medical needs. Our unique capabilities and innovative approach has enabled us to transition into a late-stage clinical company developing multiple potential therapies for patients facing a variety of CNS disorders," said Jeff Jonas, M.D., chief executive officer of SAGE. "This morning, we announced the successful completion of our Phase 1/2 clinical trial of SAGE-547 in SRSE, demonstrating SAGE-547's strong and robust activity and favorable safety profile. We believe this novel product candidate offers the potential to be developed as the first approved therapy for SRSE, providing hope to patients and their families affected by this rare and life-threatening seizure disorder."

Kimi Iguchi, chief financial officer of SAGE, added, "We believe that we are well-positioned to deliver on several value-creating milestones. We recently completed our successful equity financing, which raised net proceeds of approximately \$129.2 million, allowing us to continue investing in the expansion of our pipeline in 2015."

### Pipeline Updates and Upcoming Milestones

- **Positive Results in Phase 1/2 Clinical Trial of SAGE-547 in Super-Refractory Status Epilepticus (SRSE):** SAGE-547 demonstrated robust activity with 77 percent of 22 evaluable patients meeting the key efficacy endpoint of being successfully weaned off their anesthetic agents while SAGE-547 was being administered. In addition, 77 percent of the total evaluable patients were successfully weaned off SAGE-547 without recurrence of SRSE in the 24-hour period following treatment. SAGE-547 also demonstrated favorable tolerability and a benefit-risk profile supporting development in this acutely ill patient population. Overall, 64 percent of patients experienced at least one serious adverse event, though none were drug-related as determined by the Safety Review Committee. Independent of treatment response, six patient deaths occurred within the study period, all driven by underlying medical conditions.

The Phase 1/2 clinical trial results will be presented on May 15, 2015 by Stephen Kanés, M.D., Ph.D., chief medical officer of SAGE, at the Antiepileptic Drug and Device Trials XIII Conference. Additional details are available in a separate press release issued today.

- **Phase 3 STATUS Trial for SAGE-547 in SRSE Expected to Begin Enrollment by Mid-2015:** SAGE plans to initiate the STATUS Trial, a Phase 3 randomized, double-blind, placebo-controlled clinical trial of SAGE-547 for the treatment of patients with SRSE, by mid-year. SAGE believes the results from this clinical trial, along with other results from the SAGE-547 development program, could form the basis of a New Drug Application (NDA) submission for SAGE-547.
- **Initiated Enrollment in Study 302 - SAGE's Phase 3 Expanded Access Protocol for SAGE-547 in SRSE:** SAGE completed treatment for the first patient enrolled in its now initiated Phase 3 open-label expanded access protocol. Study 302 will make SAGE-547 available to patients in the United States, aged two years or older, who are affected with SRSE and is designed to evaluate the safety of SAGE-547.
- **SAGE-547 Exploratory Development Programs on Track to Report Results Mid-Year:** SAGE is using SAGE-547 to establish proof of principle in clinical trials for additional CNS disorders, including essential tremor and severe postpartum depression. These trials are designed to evaluate the safety, tolerability, pharmacokinetics and activity of SAGE-547 in these indications, and to help guide the design of second-generation molecules for the chronic treatment of these diseases. SAGE expects to report results from both exploratory trials by mid-2015.
- **Proprietary Follow-On Candidates to Enter Clinic by Late 2015:** SAGE's second-generation molecules, SAGE-689 and SAGE-217, continue to advance on track in preclinical development and SAGE plans to initiate Phase 1 clinical trials of these molecules by year-end. SAGE-689 is being developed as an adjunctive IV therapy for the treatment of status epilepticus, while SAGE-217 is being developed as an oral therapy for orphan epilepsies, such as Dravet syndrome and

Rett syndrome.

## Financial Results and Guidance

- **Cash Position:** Cash and cash equivalents as of March 31, 2015 were \$113.2 million, compared with \$127.8 million at December 31, 2014. Subsequent to March 31, 2015, the company completed a public offering that will add approximately \$129.2 million in net proceeds to this cash position.
- **R&D Expenses:** Research and development expenses were \$12.9 million, including \$0.5 million of non-cash stock-based compensation expense, in the first quarter of 2015, compared to \$4.2 million, including \$0.1 million of non-cash stock-based compensation expense, in the first quarter of 2014. The increase in R&D expenses was primarily due to increased spending on clinical activities related to the SAGE-547 development program, increased personnel-related R&D expenses to support the advancement of SAGE's pipeline of programs, and expenses associated with non-clinical and discovery efforts.
- **G&A Expenses:** General and administrative expenses were \$4.0 million, including \$0.8 million of non-cash stock-based compensation expense, in the first quarter of 2015, compared to \$1.6 million, including \$0.1 million of non-cash stock-based compensation expense, for the first quarter of 2014. The increase in G&A expenses was largely due to personnel-related costs and professional fees associated with operating as a public company.
- **Net Loss:** Net loss was \$16.9 million for the first quarter of 2015 compared to net loss of \$5.8 million for the first quarter of 2014.
- **Financial Guidance:** SAGE announced today that, under current plans, it expects that its cash and cash equivalents on hand as of the date hereof will be sufficient to fund its operations through mid-2017.

## Conference Call Information

SAGE will host a conference call and webcast today at 8:30 a.m. ET to discuss the results of the SAGE-547 Phase 1/2 clinical trial and the first quarter 2015 financial results. The event will be available on the investor page of SAGE's website at <http://investor.sagerx.com/> or by dialing 1-866-450-8683 (toll-free domestic) or 1-281-542-4847 (international) and using the conference ID 44443636. A replay of the webcast will be available on SAGE's website approximately two hours after the completion of the event.

## About SAGE-547

SAGE-547 is an allosteric modulator of both synaptic and extra-synaptic GABA<sub>A</sub> receptors. GABA<sub>A</sub> receptors are widely regarded as validated drug targets for a variety of disorders, with decades of research and multiple approved drugs targeting these receptor systems. SAGE-547 is an intravenous agent entering Phase 3 clinical development as an adjunctive therapy, a therapy combined with current therapeutic approaches, for the treatment of super-refractory status epilepticus (SRSE), as well as in exploratory Phase 2a clinical trials for the treatment of essential tremor and as an adjunctive therapy for the treatment of severe postpartum depression. SAGE plans to begin enrollment of its planned Phase 3 clinical trial, called the STATUS Trial, in mid-2015. SAGE-547 has been granted both Fast Track and orphan drug designations by the U.S. Food and Drug Administration (FDA) for the treatment of SRSE. The active pharmaceutical ingredient, treatment IND and support for emergency-use patients have been contributed under agreement by the Regents of the University of California and the University of California Davis.

## About Status Epilepticus

Status epilepticus (SE) is a life-threatening seizure condition that occurs in approximately 150,000 people each year in the U.S., of which 30,000 SE patients die.<sup>1</sup> We estimate that there are 35,000 patients with SE in the U.S. that are hospitalized in the intensive care unit (ICU) each year. An SE patient is first treated with benzodiazepines, and if no response, is then treated with other, second-line, anti-seizure drugs. If the seizure persists after the second-line therapy, the patient is diagnosed as having refractory SE (RSE), admitted to the ICU and placed into a medically induced coma.

Currently, there are no therapies that have been specifically approved for RSE; however, physicians typically use anesthetic agents to induce the coma and stop the seizure immediately. After a period of 24 hours, an attempt is made to wean the patient from the anesthetic agents to evaluate whether or not the seizure condition has resolved. Unfortunately, not all patients respond to weaning attempts, in which case the patient must be maintained in the medically induced coma. At this point, the patient is diagnosed as having SRSE. Currently, there are no therapies specifically approved for SRSE.

## About SAGE Therapeutics

SAGE Therapeutics is a clinical-stage biopharmaceutical company committed to developing and commercializing novel medicines to treat life-threatening, rare central nervous system, or CNS, disorders. SAGE's lead program, SAGE-547, is entering Phase 3 clinical development for super-refractory status epilepticus, or SRSE, and is the first of several compounds the Company is developing in its portfolio of potential anti-seizure medicines. SAGE's proprietary chemistry platform has generated multiple new compounds that target GABA<sub>A</sub> and NMDA receptors, which are broadly accepted as impacting many psychiatric and neurological disorders. For more information, please visit [www.sagerx.com](http://www.sagerx.com).

## Forward-Looking Statements

*Various statements in this release concerning SAGE's future expectations, plans and prospects, including without limitation, SAGE's expectations regarding how long its current cash and cash equivalents will last, SAGE's expectations regarding SAGE-547 as a treatment for SRSE, essential tremor and severe postpartum depression, statements concerning the potential safety and efficacy of SAGE-547 and durability of response, the final protocol design, statistical power and timing of a planned Phase 3 clinical trial and an open-label, expanded access protocol for SAGE-547, and whether the results from the planned Phase 3 clinical trial together with other available clinical data for SAGE-547 will be sufficient to support submission of an NDA for this product candidate, constitute forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995. In particular, it should be noted that FDA typically requires at least two well-controlled trials be completed prior to submission of an NDA. Whether a single Phase 3 trial of SAGE-547 will be sufficient to support submission of an NDA is typically a review issue to be discussed with FDA following completion of the trial. In addition, it should be noted that there is only limited data concerning the safety and efficacy of SAGE-547. These data may not be repeated or observed in future trials involving SAGE-547. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, SAGE's ability to successfully demonstrate the efficacy and safety of its drug candidates, the pre-clinical and clinical results for its product candidates, which may not support further development of product candidates, actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials, obtaining, maintaining and protecting intellectual property, SAGE's ability to enforce its patents against infringers and defend its patent portfolio against challenges from third parties, competition from others developing products for similar uses, SAGE's ability to manage operating expenses, SAGE's ability to obtain additional funding to support its business activities and establish and maintain strategic business alliances and new business initiatives, SAGE's dependence on third parties for development, manufacture, marketing, sales and distribution of products, the outcome of litigation, and unexpected expenditures, as well as those risks more fully discussed in the section entitled "Risk Factors" in SAGE's annual report on Form 10-K for the fiscal year ended December 31, 2014, as well as discussions of potential risks, uncertainties, and other important factors in SAGE's subsequent filings with the Securities and Exchange Commission. In addition, any forward-looking statements represent SAGE's views only as of today and should not be relied upon as representing its views as of any subsequent date. SAGE explicitly disclaims any obligation to update any forward-looking statements.*

<sup>i</sup> DeLorenzo, Robert J., Pellock, John M., Towne, Alan R., Boggs, Jane G. Epidemiology of Status Epilepticus. *J Clin Neuro* 1995; 12(4): 316-325.

### Sage Therapeutics, Inc. and Subsidiary Consolidated Balance Sheets

(in thousands, except share and per share data)

(Unaudited)

	March 31, 2015	December 31, 2014
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 113,162	\$ 127,766
Prepaid expenses and other current assets	1,367	1,056
Total current assets	114,529	128,822
Property and equipment, net	269	163
Restricted cash	39	39
Deferred tax assets	641	641
Total assets	<u>\$ 115,478</u>	<u>\$ 129,665</u>

### Liabilities and Stockholders' Equity

Current liabilities:		
Accounts payable	\$ 2,782	\$ 2,429
Accrued expenses	5,522	4,687
Deferred tax liabilities	641	641
Total current liabilities	8,945	7,757
Other liabilities	23	23
Total liabilities	8,968	7,780
Commitments and Contingencies		
Stockholders' equity:		
Preferred stock, \$0.0001 par value; 5,000,000 shares authorized at March 31, 2015 and December 31, 2014, respectively; no shares issued or outstanding at March 31, 2015 and December 31, 2014, respectively	--	--
Common stock, \$0.0001 par value; 120,000,000 shares authorized at March 31, 2015 and December 31, 2014, respectively; 25,694,560 and 25,621,791 shares issued and outstanding at March 31, 2015 and December 31, 2014, respectively	3	3
Additional paid-in capital	190,223	188,727
Accumulated deficit	(83,716)	(66,845)
Total stockholders' equity	106,510	121,885
Total liabilities and stockholders' equity	\$ 115,478	\$ 129,665

**Sage Therapeutics, Inc. and Subsidiary**  
**Consolidated Statements of Operations and Comprehensive Loss**  
(in thousands, except share and per share data)  
*(Unaudited)*

	<b>Three Months Ended March</b>	
	<b>31,</b>	
	<b>2015</b>	<b>2014</b>
Operating expenses:		
Research and development	\$ 12,900	\$ 4,173
General and administrative	3,997	1,617
Total operating expenses	16,897	5,790
Loss from operations	(16,897)	(5,790)
Interest income (expense), net	21	--
Other income (expense), net	5	--
Net loss and comprehensive loss	(16,871)	(5,790)
Accretion of redeemable convertible preferred stock to redemption value	--	(326)
Net loss attributable to common stockholders	<u>\$(16,871)</u>	<u>\$(6,116)</u>
Net loss per share attributable to common stockholders—basic and diluted	<u>\$(0.66)</u>	<u>\$(3.70)</u>
Weighted average number of common shares used in net loss per share attributable to common stockholders—basic and diluted	<u>25,655,883</u>	<u>1,652,726</u>

CONTACT: Investor Contact:

Paul Cox, SAGE Therapeutics

[paul.cox@sagerx.com](mailto:paul.cox@sagerx.com)

617-299-8377

Media Contact:

Dan Budwick, Pure Communications

[dan@purecommunicationsinc.com](mailto:dan@purecommunicationsinc.com)

973-271-6085



Source: SAGE Therapeutics

News Provided by Acquire Media