SAGE Therapeutics Presents New Data Identifying Highly Selective, Oral Compounds and Novel Approach for the Treatment of Chronic Epilepsies

Findings Presented at the American Epilepsy Society Annual Meeting

Cambridge, Mass. – December 9, 2013 – SAGE Therapeutics, a biopharmaceutical company developing novel medicines to treat central nervous system (CNS) diseases, today announced new data presented at the American Epilepsy Society (AES) Annual Meeting that reveal a novel mechanism and approach for the treatment of status epilepticus (SE) and identify new, orally bioavailable compounds that may lead to improved therapies for chronic epilepsies.

“Epilepsy is among many disorders of the CNS that have a critical unmet need,” said Al Robichaud, Ph.D., chief scientific officer of SAGE. “These data highlight SAGE’s robust chemistry platform as well as our ability to design and develop molecules that directly impact specific mechanisms involved in epilepsy and other CNS diseases. Our focus on GABA_A receptor modulation is unlike previous efforts, opening up significant potential to treat diseases with no current therapies.”

GABA_A receptors are an attractive target for the treatment of a multitude of CNS disorders, including acute and chronic seizures. Traditional approaches of inhibiting or activating the GABA pathway have been associated with significant toxicities. SAGE’s unique and proprietary approach of both positive and negative allosteric modulation of the GABA_A receptor “fine-tunes” brain activity and has the potential to more effectively treat these diseases, while limiting the harmful side effects seen with many CNS therapies.

The SAGE team has developed SAGE-547, a proprietary investigational treatment for SE that has potent activity at both synaptic and extra-synaptic GABA_A receptors and has been shown to effectively treat seizures and SE in preclinical studies. In the findings presented at the AES meeting, SAGE researchers describe new compounds that are orally bioavailable, selective for the GABA_A receptor and show robust anti-seizure activity in preclinical models. These findings may lead to new therapies for the treatment of chronic epilepsies and orphan genetic epilepsies, such as Fragile X syndrome, Dravet Syndrome and Rett Syndrome.

“We are rapidly entering the clinic with our lead program in status epilepticus and these additional novel oral compounds have the potential to quickly follow,” said Jeff Jonas, chief executive officer of SAGE. “We are committed to delivering better therapies to patients in dire
need of more effective treatments for debilitating CNS disorders.”

The research was presented in a poster titled “Generation of synthetic neuroactive steroids with potent positive modulatory activity at GABA$_A$ synaptic and extra-synaptic receptors for the treatment of epilepsy,” today at the AES annual meeting in Washington, D.C.

About SAGE Therapeutics
SAGE Therapeutics is a neuroscience-focused company developing therapies to treat CNS specialty and orphan diseases. The company has identified several product opportunities with clear and accelerated paths to regulatory approval. SAGE’s initial pipeline includes programs in status epilepticus, anesthesia, Fragile X Syndrome and traumatic brain injury, where CNS drugs poorly address the areas of most urgent patient need and are often accompanied by considerable side effects. The company’s robust allosteric modulator chemistry platform – called the Positive and Negative Allosteric Modulator (PANAM) platform – has generated multiple new chemical entities supported by promising preclinical data with the potential to lead to products with multiple indications over the next several years. SAGE Therapeutics is a private company launched in 2011 by a proven team of R&D leaders, renowned CNS experts and Third Rock Ventures. For more information, please visit www.sagerx.com.

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