Sangamo Therapeutics Announces U.S. FDA Clearance of IND Application for ST-503 for the Treatment of Idiopathic Small Fiber Neuropathy, a Type of Chronic Neuropathic Pain

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Sangamo plans to initiate enrollment of patients in the Phase 1/2 study for ST-503 in mid-2025

RICHMOND, Calif.--(BUSINESS WIRE)--Nov. 19, 2024-- Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced that the U.S. Food and Drug Administration (FDA) has cleared the investigational new drug (IND) application for its ST-503 program, an investigational epigenetic regulator for the treatment of intractable pain due to idiopathic small fiber neuropathy (iSFN), a type of chronic neuropathic pain.

Neuropathic pain can be caused by a broad array of pathologies impacting the central or peripheral nervous systems, such as surgical trauma, spinal cord injury, nerve compression, neurological and infectious diseases, or metabolic and hereditary syndromes. ST-503 is not intended for sporadic or acute pain, but for chronic, intractable pain that completely dominates and often destroys the lives of patients over many years. The Phase 1/2 study will assess the safety and efficacy of ST-503 in addressing iSFN, a peripheral neuropathy that results in highly debilitating symptoms of burning, prickling, stabbing or “lightning-like” pain. iSFN has an estimated prevalence of at least 43,000 patients in the U.S., and more broadly, peripheral neuropathies are estimated to affect nearly 40 million Americans. Antidepressants, anticonvulsants, opioids and topical therapies are potential treatment options, although no long-lasting or curative therapies are currently available for iSFN patients, leading to a high unmet medical need for this patient population.

“The FDA’s clearance of the IND application to evaluate ST-503 in idiopathic small fiber neuropathy represents an important milestone for Sangamo on our journey to becoming a neurology genomic medicine company,” said Nathalie Dubois-Stringfellow, Ph.D, Chief Development Officer at Sangamo. “We strongly believe in the power of our zinc finger technology to address neurological conditions and are excited about our plans to advance this program into the clinic next year, to bring hope to patients suffering from debilitating, intractable pain, for which there are insufficient current treatment options.”

A significant body of evidence implicates sodium channels in mediating the pathophysiology of neuropathic pain. ST-503 uses an adeno-associated virus (AAV) vector carrying an engineered zinc finger repressor (ZFR) to specifically target the human gene, *SCN9A*, that encodes the Nav1.7 sodium channel and is critical for pain signaling. Developing small molecules that specifically target Nav1.7 is challenging due to the high structural similarities between different sodium channels, making it difficult to achieve selectivity and avoid off-target effects. By directly targeting the *SCN9A* gene, ST-503 was shown to selectively reduce the expression of Nav1.7 sodium channels in sensory neurons in animal models and significantly reduce pain hypersensitivity, following a single intrathecal administration of ST-503. Sangamo’s preclinical research has shown ST-503 to be well tolerated in nonhuman primates, with substantial Nav1.7 reduction observed with no off-target effects, demonstrating the promise of ST-503 as a potential therapy for chronic neuropathic pain, regardless of cause.

Sangamo is preparing for the Phase 1/2 clinical study to assess the safety, tolerability and preliminary efficacy of a one-time dose of ST-503, administered intrathecally to patients with intractable pain due to iSFN, and plans to initiate patient enrollment in mid-2025.

Sangamo believes that if this study is successful, the development of ST-503 could be broadened to patient populations suffering from other types of chronic neuropathic pain.

About Sangamo Therapeutics

Sangamo Therapeutics is a genomic medicine company dedicated to translating ground-breaking science into medicines that transform the lives of patients and families afflicted with serious neurological diseases who do not have adequate or any treatment options. Sangamo believes that its zinc finger epigenetic regulators are ideally suited to potentially address devastating neurological disorders and that its capsid discovery platform can expand delivery beyond currently available intrathecal delivery capsids, including the central nervous system. Sangamo’s pipeline also includes multiple partnered programs and programs with opportunities for partnership and investment. To learn more, visit www.sangamo.com and connect with us on LinkedIn and X.

Forward-Looking Statements

This press release contains forward-looking statements regarding our future plans and current expectations. These forward-looking statements include, without limitation, statements relating to: expectations concerning the ability of our zinc finger technology to address neurological conditions; the anticipated advancement of ST-503 to the clinic, including the timing related thereto and the potential for a Phase 1/2 study to assess its safety, tolerability and preliminary efficacy in addressing iSFN; the potential for ST-503 to treat chronic neuropathic pain, regardless of cause; the potential to broaden development of ST-503 to other patient populations; and other statements that are not historical fact. These statements are not guarantees of future performance and are subject to certain risks and uncertainties that are difficult to predict. Factors that could cause actual results to differ include, but are not limited to, risks and uncertainties related to our lack of capital resources to obtain regulatory approval for and commercialize our product candidates in a timely manner or at all; the uncertain timing and unpredictable nature of clinical trial results; our need for substantial additional funding to execute our operating plan and to continue to operate as a going concern, including the need for adequate additional funding to initiate patient enrollment in the Phase 1/2 study of ST-503; the effects of macroeconomic factors or financial challenges on the global business environment, healthcare systems and our business and operations; the research and development process; the potential for technological developments that obviate technologies used by Sangamo; and our ability to achieve expected future financial performance.

All forward-looking statements about our future plans and expectations, are subject to our ability to secure adequate additional funding. Actual results may differ materially from those projected in these forward-looking statements due to the risks and uncertainties described above and other risks and

uncertainties that exist in the operations and business environments of Sangamo and our collaborators. These risks and uncertainties are described more fully in our Securities and Exchange Commission, or SEC, filings and reports, including in our Annual Report on Form 10-K for the year ended December 31, 2024, as supplemented by our Quarterly Report on Form 10-Q for the quarter ended September 30, 2024, each filed with the SEC, and future filings and reports that Sangamo makes from time to time with the SEC. Forward-looking statements contained in this announcement are made as of this date, and we undertake no duty to update such information except as required under applicable law.

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