Sangamo Therapeutics Logo

Biogen and Sangamo Announce Global Collaboration to Develop Gene Regulation Therapies for Alzheimer's, Parkinson's, Neuromuscular, and Other Neurological Diseases

February 27, 2020

- Broad collaboration for gene regulation therapies in neurology, initially focused on development of ST-501 for tauopathies including Alzheimer's disease, ST-502 for synucleinopathies including Parkinson's disease, and a neuromuscular target, with exclusive rights for nine additional undisclosed neurological targets
- Biogen will pay Sangamo $350 million upfront, including a license fee and an equity investment in Sangamo stock
- Sangamo is eligible to receive up to $2.37 billion in potential milestones, as well as royalties on potential net commercial sales
- Biogen's access to Sangamo's gene regulation therapies complements its expanding efforts in gene therapy across diverse neurological diseases

CAMBRIDGE, Mass. & BRISBANE, Calif.--(BUSINESS WIRE)-- Biogen Inc. (Nasdaq: BIIB) and Sangamo Therapeutics, Inc. (Nasdaq: SGMO), a genomic medicine company, today announced that they have executed a global licensing collaboration agreement to develop and commercialize ST-501 for tauopathies including Alzheimer's disease, ST-502 for synucleinopathies including Parkinson's disease, a third undisclosed neuromuscular disease target, and up to nine additional undisclosed neurological disease targets. The companies will leverage Sangamo's proprietary zinc finger protein (ZFP) technology delivered via adeno-associated virus (AAV) to modulate the expression of key genes involved in neurological diseases.

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"As a pioneer in neuroscience, Biogen will collaborate with Sangamo on a new gene regulation therapy approach, working at the DNA level, with the potential to treat challenging neurological diseases of global significance. We aim to develop and advance these programs forward to investigational new drug applications," said Alfred Sandrock Jr., M.D., Ph.D., Executive Vice President, Research and Development at Biogen.

"There are currently no approved disease modifying treatments for patients with many devastating neurodegenerative diseases such as Alzheimer's and Parkinson's, creating an urgency for the development of medicines that will not just address symptoms like the current standards of care, but slow or stop the progression of disease," said Sandy Macrae, CEO of Sangamo. "We believe that the promise of genomic medicine in neuroscience is to provide a one-time treatment for patients to alter their disease natural history by addressing the underlying cause at the genomic level."

Sangamo's genome regulation technology, zinc finger protein transcription factors (ZFP-TFs), is currently delivered with AAVs and functions at the DNA level to selectively repress or activate the expression of specific genes to achieve a desired therapeutic effect. Highly specific, potent, and tunable repression of tau and alpha synuclein has been demonstrated in preclinical studies using AAV vectors to deliver tau-targeted (ST-501) and alpha synuclein-targeted (ST-502) ZFP-TFs.

"The combination of Sangamo's proprietary zinc finger technology, Biogen's unmatched neuroscience research, drug development, and commercialization experience and capabilities, and our shared commitment to bring innovative medicines to patients with neurological diseases establishes the foundation for a robust and compelling collaboration," said Stephane Boissel, Head of Corporate Strategy at Sangamo. "This collaboration exemplifies Sangamo's commitment to our ongoing strategy to partner programs that address substantial and diverse patient populations in disease areas requiring complex clinical trial designs and commercial pathways, therefore bringing treatments to patients faster and more efficiently, while deriving maximum value from our platform."

Under the terms of the collaboration, Biogen has exclusive global rights to ST-501 for tauopathies including Alzheimer's disease, ST-502 for synucleinopathies including Parkinson's disease, and a third undisclosed neuromuscular disease target. In addition, Biogen has exclusive rights to nominate up to nine additional undisclosed targets over a target selection period of five years. Sangamo will perform early research activities, costs for which will be shared by the companies, aimed at the development of the combination of proprietary CNS delivery vectors and ZFP-TFs targeting therapeutically relevant genes. Biogen will then assume responsibility and costs for the investigational new drug-enabling studies, clinical development, related regulatory interactions, and global commercialization.

Sangamo will be responsible for GMP manufacturing activities for the initial clinical trials for the first three products of the collaboration and plans to leverage its in-house manufacturing capacity. Biogen will assume responsibility for GMP manufacturing activities beyond the first clinical trial for each of the first three products.

Upon closing of this transaction, Sangamo will receive $350 million comprised of $125 million in a license fee payment and $225 million from the sale of new Sangamo stock, or approximately 24 million shares at $9.21 per share. In addition, Sangamo may receive up to $2.37 billion in other development, regulatory, and commercial milestone payments, including up to $925 million in pre-approval milestone payments and up to $1,445 million in first commercial sale and other sales-based milestone payments. Sangamo will also be eligible to receive from Biogen tiered high single-digit to sub-teen double-digit royalties on potential net commercial sales of products arising from the collaboration. Closing of the transaction is contingent on completion of review under antitrust laws, including the Hart-Scott-Rodino (HSR) Antitrust Improvements Act of 1976 in the U.S.

Conference call

Sangamo will host a conference call at 8:00 a.m. ET tomorrow, Friday, February 28, which will be open to the public via telephone and webcast. During the conference call, Sangamo will discuss the collaboration, review financial results for the fourth quarter and full year 2019, and provide a business update. The conference call dial-in numbers are (877) 377-7553 for domestic callers and (678) 894-3968 for international callers. The conference ID number for the call is 4609858. Participants may access the live webcast via a link on the Sangamo website in the Investors and Media section under Events and Presentations. A conference call replay will be available for one week following the conference call on Sangamo's website. The conference call replay numbers for domestic and international callers are (855) 859-2056 and (404) 537-3406, respectively. The conference ID number for the

replay is 4609858.

About Biogen

At Biogen, our mission is clear: we are pioneers in neuroscience. Biogen discovers, develops, and delivers worldwide innovative therapies for people living with serious neurological and neurodegenerative diseases as well as related therapeutic adjacencies. One of the world's first global biotechnology companies, Biogen was founded in 1978 by Charles Weissmann, Heinz Schaller, Kenneth Murray, and Nobel Prize winners Walter Gilbert and Phillip Sharp. Today Biogen has the leading portfolio of medicines to treat multiple sclerosis, has introduced the first approved treatment for spinal muscular atrophy, commercializes biosimilars of advanced biologics, and is focused on advancing research programs in multiple sclerosis and neuroimmunology, Alzheimer's disease and dementia, neuromuscular disorders, movement disorders, ophthalmology, immunology, neurocognitive disorders, acute neurology, and pain.

Biogen routinely posts information that may be important to investors on its website at www.biogen.com. To learn more, please visit www.biogen.com and follow Biogen on social media – Twitter, LinkedIn, Facebook, YouTube.

About Sangamo Therapeutics

Sangamo Therapeutics is committed to translating ground-breaking science into genomic medicines with the potential to transform patients' lives using gene therapy, *ex vivo* gene-edited cell therapy, and *in vivo* genome editing and gene regulation. For more information about Sangamo, visit www.sangamo.com.

Biogen Safe Harbor

This press release contains forward-looking statements, made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995, including statements relating to the potential benefits and results that may be achieved through Biogen's proposed collaboration with Sangamo; the anticipated completion and timing of the proposed transaction; the potential benefits, safety and efficacy of ST-501 and ST-502; the potential of Biogen's commercial business and pipeline programs; Biogen's strategy and plans; the potential treatment of neurological diseases; and risks and uncertainties associated with drug development and commercialization. These forward-looking statements may be accompanied by words such as "aim," "anticipate," "believe," "could," "estimate," "expect," "forecast," "goal," "intend," "may," "plan," "potential," "possible," "will," "would," and other words and terms of similar meaning. Drug development and commercialization involve a high degree of risk, and only a small number of research and development programs result in commercialization of a product. Results in early stage clinical trials may not be indicative of full results or results from later stage or larger scale clinical trials and do not ensure regulatory approval. You should not place undue reliance on these statements or the scientific data presented.

These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including, without limitation: risks that the proposed transaction will be completed in a timely manner or at all; the possibility that certain closing conditions to the proposed transaction will not be satisfied; uncertainty as to whether the anticipated benefits of the proposed collaboration can be achieved; risks of unexpected hurdles, costs or delays; uncertainty of success in the development and potential commercialization of ST-501 and ST-502 and other undisclosed neurological targets, which may be impacted by, among other things, unexpected concerns that may arise from additional data or analysis, the occurrence of adverse safety events, failure to obtain regulatory approvals in certain jurisdictions, failure to protect and enforce Biogen's data, intellectual property, and other proprietary rights and uncertainties relating to intellectual property claims and challenges; product liability claims; and third party collaboration risks. The foregoing sets forth many, but not all, of the factors that could cause actual results to differ from Biogen's expectations in any forward-looking statement. Investors should consider this cautionary statement, as well as the risks factors identified in Biogen's most recent annual or quarterly report and in other reports Biogen has filed with the U.S. Securities and Exchange Commission. These statements are based on Biogen's current beliefs and expectations and speak only as of the date of this press release. Biogen does not undertake any obligation to publicly update any forward-looking statements, whether as a result of new information, future developments or otherwise.

Sangamo Forward Looking Statements

This press release contains forward-looking statements regarding Sangamo's current expectations. These forward-looking statements include, without limitation, statements relating to the potential to use ZFP technology delivered via AAV to repress specific genes involved in neurological diseases, the ability of genomic medicine to provide one-time treatments, other statements regarding investigational therapies and their therapeutic benefits, statements related the anticipated effectiveness of the collaboration and the timing and benefits thereof, Sangamo's sale of shares of its common stock, receipt of an upfront payment and potential receipt of development- and sales-based milestones, as well as royalties on potential future sales, and other statements that are not historical fact. These statements are not guarantees of future performance and are subject to risks and assumptions that are difficult to predict. Factors that could cause actual results to differ include, but are not limited to, risks and uncertainties related to: the research and development process; the ability to cause the agreements to become effective on the proposed terms and schedule, the ability to obtain clearance under the HSR and to satisfy the other closing conditions, and the potential for technological developments by Sangamo's competitors that will obviate Sangamo's technologies, the new, uncertain and time consuming gene regulation therapy development and regulatory process, including the risks that Sangamo and Biogen may not be successful in their research efforts under the collaboration and that, even if successful, Biogen may be unable to successfully develop and commercialize licensed products resulting from the collaboration; Sangamo's dependence on collaborative partners, including the risks that if Biogen were to breach or terminate the agreement or otherwise fail to successfully develop and commercialize licensed products resulting from the collaboration and in a timely manner, Sangamo would not obtain the anticipated financial and other benefits of the collaboration and the development and/or commercialization of Sangamo's gene editing technology could be delayed, perhaps substantially. There can be no assurance that the necessary milestones or approvals will be obtained for any of the product candidates in this collaboration. Actual results may differ from those projected in forward-looking statements due to risks and uncertainties that exist in Sangamo's operations and business environments. These risks and uncertainties are described more fully in Sangamo's filings with the U.S. Securities and Exchange Commission, including its most recent Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q. Forward-looking statements contained in this announcement are made as of this date, and Sangamo undertakes no duty to update such information except as required under applicable law.

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Source: Sangamo Therapeutics, Inc.