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# Sangamo Therapeutics Announces Upcoming Presentations At The 13th Annual WORLDSymposium™ Meeting

RICHMOND, Calif., Feb. 8, 2017 /PRNewswire/ -- Sangamo Therapeutics, Inc. (Nasdaq: SGM0), the leader in therapeutic genome editing, today announced upcoming oral and poster presentations at the 13<sup>th</sup> Annual WORLDSymposium™ Meeting being held in San Diego, CA from February 13 - 17, 2017.



Members of the Company's research and development team will present additional preclinical disease model data from its SB-318 and SB-913 therapeutic *in vivo* genome editing programs for MPS I and MPS II, new preclinical data on an alternative treatment modality using lipid nanoparticle delivery of zinc finger nucleases (ZFNs), as well as preclinical data on gene therapy and ZFN-mediated *in vivo* genome editing approaches for Fabry disease.

- *Liver-based expression of the human alpha-galactosidase A gene (GLA) in a murine Fabry model results in continuous supra-physiological enzyme activity and effective substrate reduction – Marshal W. Huston, PhD, Sangamo Therapeutics*  
**Oral Presentation: Basic Science I, 10:45am PT, Tuesday, Feb. 14, 2017**  
**Poster Presentation: 4:30p – 6:30pm PT, Tuesday, Feb. 14, 2017 [Poster #151]**
- *ZFN-mediated *in vivo* genome editing results in phenotypic correction in murine MPS I and MPS II models – Russell DeKolver, PhD, Sangamo Therapeutics*  
**Oral Presentation: Translational Research I, 9:15am PT, Wednesday, Feb. 15, 2017**  
**Poster Presentation: 4:30p – 6:30pm PT, Tuesday, Feb. 14, 2017 [Poster #71]**
- **In vivo* genome editing via non-viral delivery of zinc finger nucleases results in supraphysiological levels of human iduronate 2-sulfatase in adult mice – Anthony Conway, PhD, Sangamo Therapeutics*  
**Poster Presentation: 4:30p – 6:30pm PT, Wednesday, Feb. 15, 2017 [Poster #532]**

## About SB-318 and SB-913

SB-318 and SB-913 are being evaluated in Phase 1/2 clinical trials as single treatment therapies for MPS I and MPS II, lysosomal storage disorders caused by mutations in the

genes encoding the alpha-L-iduronidase (IDUA) and iduronate 2-sulfatase (IDS) enzymes, respectively. SB-318 and SB-913 are based on Sangamo's zinc finger nuclease (ZFN)-mediated *in vivo* genome editing approach and are designed to produce continuous, lifelong therapeutic levels of IDUA or IDS.

Preclinical data for the two programs demonstrated stable, continuous production of active, human IDUA (hIDUA) or hIDS enzymes from the liver, which were secreted into circulation and taken up by various secondary tissues, including the liver, spleen, kidneys, lungs, heart, muscle and brain of MPS I or MPS II mice after a single administration of SB-318 or SB-913, respectively. This resulted in a significant increase in hIDUA or hIDS enzyme activity and reduction of glycosaminoglycan (GAG) biomarkers in the plasma and secondary tissues. Treated mice also demonstrated preserved cognitive function when assessed using a Barnes Maze, which evaluates spatial learning and memory.

### **About Sangamo Therapeutics**

Sangamo Therapeutics, Inc. is focused on translating ground-breaking science into genomic therapies that transform patients' lives using the company's industry leading platform technologies in genome editing, gene therapy, gene regulation and cell therapy. The Company has Phase 1/2 clinical programs in hemophilia A and B, and lysosomal storage disorders MPS I and MPS II. Sangamo has a strategic collaboration with Bioverativ, Inc. for hemoglobinopathies, including beta thalassemia and sickle cell disease, and with Shire International GmbH to develop therapeutics for Huntington's disease. In addition, it has established strategic partnerships with companies in non-therapeutic applications of its technology, including Sigma-Aldrich Corporation and Dow AgroSciences. For more information about Sangamo, visit the Company's website at [www.sangamo.com](http://www.sangamo.com).

*This press release contains forward-looking statements regarding Sangamo's current expectations. These forward looking statements include, without limitation, references to preclinical data from SB-318 and SB-913 programs and the potential of these programs to treat MPS I and MPS II. These statements are not guarantees of future performance and are subject to certain risks, uncertainties and assumptions that are difficult to predict. Factors that could cause actual results to differ include, but are not limited to, the early stage of ZFP Therapeutic development, the lengthy and uncertain regulatory approval process, uncertainties related to the timing of initiation and completion of clinical trials, whether clinical trial results will validate and support the safety and efficacy of ZFP Therapeutics, and the ability to establish strategic partnerships. Further, there can be no assurance that the necessary regulatory approvals will be obtained or that Sangamo and its partners will be able to develop commercially viable gene-based therapeutics. 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 Annual Reports on Form 10-K and Quarterly Reports on Form 10-Q as filed with the Securities and Exchange Commission. 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.*

To view the original version on PR Newswire, visit <http://www.prnewswire.com/news-releases/sangamo-therapeutics-announces-upcoming-presentations-at-the-13th-annual-worldsymposium-meeting-300404455.html>

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