
# Initiating Coverage of Sangamo Biosciences

## Rating: Strong Buy

Sangamo Biosciences is a small capitalization biotechnology company with a powerful drug development technology. Not only does this allow for the development of an extensive pipeline but also provides for a hybrid business model that limits (although not eliminates) the need for dilutive capital.

The company has a busy 2011 with numerous data presentations. In particular, the upcoming Conference on Retroviruses and Opportunistic Infections (CROI) will see the company with four separate presentations (SB-728 is its HIV candidate). These will present data from two of its HIV trials. In addition, the company plans to present further data from its HIV program in the fourth quarter of 2011.

While not as proximate, but perhaps more importantly, the company also will be releasing top line data from its SB-509-901 trial in diabetic neuropathy. This phase IIb trial was designed to only incorporate moderate severity patients and represents a critical proof of concept test for SB-509. SB-509 has previously been shown to regenerate years worth of neuropathic damage but this trial is designed to demonstrate statistically significant improvements in nerve conduction velocity and neuropathic impairment score lower limb.

## Price Ranges

There are two main catalysts this year for Sangamo Biosciences: CROI presentation and top-line SB-509-901 data. This essentially creates four scenarios (highlighted is what I believe is most likely).

| | |
|---|---|
| Fail CROI, Fail SB 509-901 | Year end price target: $3.00 |
| Fail CROI, Succeed SB 509-901 | Year end price target:$8.00 |
| Succeed CROI, Fail SB 509-901 | Year end price target:$10.00 |
| Succeed CROI, Succeed SB 509-901 | Year end price target:$15.00 |

## Contents

## The Sangamo Technology

Sangamo has a lock on the intellectual property for Zinc Finger Protein (ZFP) and Zinc Finger Nuclease (ZFN) technology. In the most general sense, this platform enables Sangamo to develop therapeutic compounds that can regulate genes (ZFPs) or modify genes (ZFNs). This ability, however, extends to any living organism meaning that its technology has broad appeal to the larger life science and plant science industries. This can range from engineered cell lines for the manufacturing of protein pharmaceuticals to regulation and modification of genes in plants.

For more details on this technology, the end of this report has links to specific articles.

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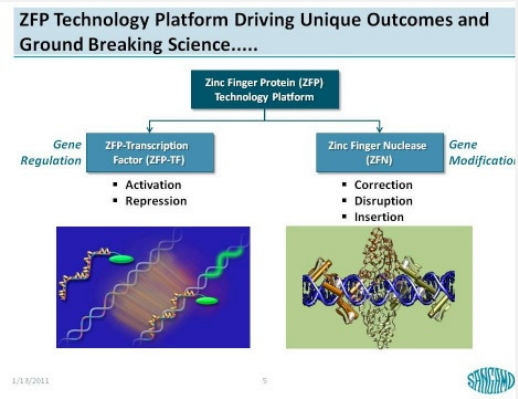


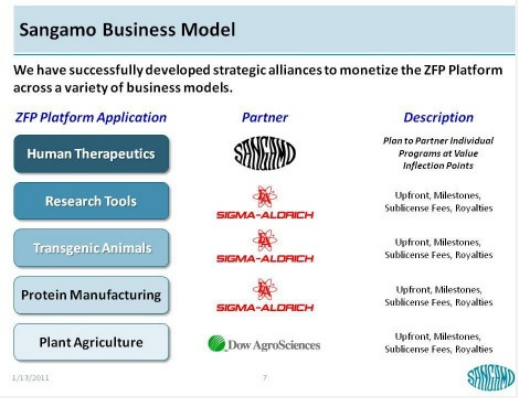


Source: 29th Annual JPM Healthcare Conference Presentation

## Sangamo Business Model

Sangamo has a relatively unique business model among small capitalization biotechnology companies. Given the applications in research tools, transgenic animals, protein manufacturing, and plant agriculture, the company is able to access non-dilutive capital. For instance, its partnership with Sigma-Aldrich not only produced a number of milestone payments but also provides Sangamo 10.5% of net sales of the licensed products. Sangamo will also receive royalties from Dow AgroScience on sales but this rate has not been released (plus, sales based on products that use Sangamo technology are year away). Overall, this hybrid business model has provided Sangamo with just under $80 million in capital.

While royalties from Sigma-Aldrich and Dow AgroSciences will likely never be the main catalyst for the stock, the key is that these relationships and the cash they produce lessens the need for dilution. This is not to say that the company has not or will not dilute with a secondary; rather, Sangamo is likely to do so at a slower pace. This ultimately provides shareholders with a larger piece of the company when drugs get approved and sales on the human therapeutic products ramp up.

At this point, the company does not have any partnerships for its human therapeutics programs. This has been a source of angst for retail shareholders who would like to see the pipeline monetized. It is unlikely that a partnership would emerge for the lead compound (SB-509) until later in the year. The company has guided that positive results from the 901 trial in diabetic neuropathy should quickly lead to a partnership for SB-509. As for the other programs, the timing of partnerships has not been mentioned except to note that the company will seek partnerships at point of value inflection.

# Table 1: Sangamo Pipeline

Source: 29th Annual JPM Healthcare Conference Presentation

## ZFP Therapeutic® Product Development Pipeline

| Therapeutic Areas | Sangamo Programs | Development Phase | | | | | |
|---|---|---|---|---|---|---|---|
| | | Research | Preclinical | Phase 1 | Phase 2 | Phase 3 | NDA / BLA |
| Diabetic Neuropathy | SB-509 | | | | | | |
| ALS | SB-509 | | | | | | |
| HIV / AIDS | SB-728-T CCR5 (HSCs, In Vivo, CXCR4) | | | | | | |
| Cancer (Glioblastoma) | SB-313 | | | | | | |
| Parkinson's Disease | Preclinical | | | | | | |
| Neuropathic Pain (Chemo Induced) | Preclinical | | | | | | |
| Spinal Cord Injury | Preclinical | | | | | | |
| Monogenic & Rare Diseases | Preclinical | | | | | | |

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# Pipeline

Sangamo's lead program is SB-509 in diabetic neuropathy (it has also been tested in ALS). There is currently an ongoing phase IIb study (901 trail) in moderately severe patients. The trial is completely enrolled and data is expected in the second half of 2011. Opinions of this program are mixed mainly given that an earlier trial (601) missed statistical significance on the nerve conduction velocity (NCV) tests and neuropathy impairment score lower limb (NIS-LL). Interestingly, however, when looking at biopsies, the company found statistically significant nerve growth (treatment reversed about 2-3 years of degeneration).

The company believes that the trial had inadvertently admitted too many mild cases of diabetic neuropathy, who have less measurable improvements. It is believed that the moderate severity patients represent a sweet spot for measurable improvements in NCV and NIS-LL. As such, the 901 trial was designed to ensure that only moderately severe cases of diabetic neuropathy made it into the study. Despite the company's direct measurement of nerve growth, many investors still feel burned by the program and ascribe a low probability to its ultimate success. Given the evidence from the biopsies, however, I am more optimistic and believe that the 901 trial will show statistically significant improvements in both NCV and NIS-LL. In many ways this is a make or break trial for the program.

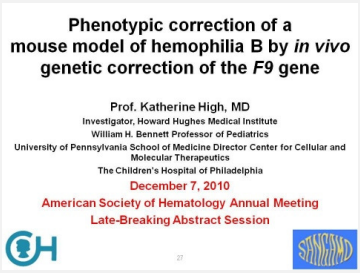
Success with this program would be a huge boon to the company first as important proof of concept and second as a large market opportunity. Currently, there are no disease modifying agents available for diabetic neuropathy. The size of the market is unclear (http://www.uptodate.com/contents/epidemiology-and-classification-of-diabetic-neuropathy ) but given the number of individuals in the United States alone with diabetes it does not take very aggressive assumptions to argue that a disease modifying treatment for diabetic neuropathy would be a multi-billion dollar blockbuster.

## HIV Trials

I previous released a short report on what to expect with the upcoming data presentation. Please view that here (Sangamo Pre-Croi Report). I will be releasing a report after the presentation as well.

## Pre-Clinical Pipeline

Sangamo also has a number of pre-clinical programs that are quite exciting. One that has recently been highlighted by the company deals with Hemophilia B. At ASH in December 2010, the company presented a paper that showed a single treatment with its drug candidate permanently corrected the F9 gene in a mouse. In other words, it seemed to permanently cure a mouse suffering from Hemophilia B with a single dose of the drug. While more testing needs to be done, this technology is powerful enough that virtually all monogenic diseases can be tackled in this way. Below is a slide that presents the title of the paper.




For additional information on recently pre-clinical presentations see:

http://investor.sangamo.com/releasedetail.cfm?ReleaseID=535403

and

http://investor.sangamo.com/releasedetail.cfm?ReleaseID=536315

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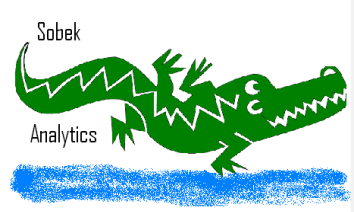


# Additional Information

## ZFP and ZFN Technology

Targeted gene knockout in mammalian cells by using engineered zinc-finger nucleases. http://www.ncbi.nlm.nih.gov/pubmed/18359850

Zinc finger nucleases: custom-designed molecular scissors for genome engineering of plant and mammalian cells.
http://www.ncbi.nlm.nih.gov/pubmed/16251401

Custom-designed zinc finger nucleases: what is next?
http://www.ncbi.nlm.nih.gov/pubmed/17763826

Zinc-finger nucleases: the next generation emerges.
http://www.ncbi.nlm.nih.gov/pubmed/18545224

## Sangamo and HIV

Human hematopoietic stem/progenitor cells modified by zinc-finger nucleases targeted to *CCR5* control HIV-1 *in vivo*
http://www.nature.com/nbt/journal/v28/n8/full/nbt.1663.html

## Previous Reports on Sangamo

Sangamo Pre-Croi Report



# Disclaimer

I am not a certified financial analyst. All the information provided in this report is my interpretation and may contain errors. Please, do not invest based solely on my opinions as it is critical for all investors to conduct their own due diligence and invest in ways that best fit their own needs. All errors (if any) in this report are mine and due to my misinterpretations. In addition, I am long shares of SGMO.