

# Sangamo (SGMO) Flash

## Two Presentations Wednesday at CROI

As a recap, Sangamo Biosciences had a busy week at CROI with four separate presentations. These covered their clinical trials and some of their pre-clinical work. The data were, while early, quite exciting. In terms of the clinical data, the vast bulk centered on aviremic patients, i.e. those with minimal viral loads. As such, issues of safety and engraftment dominated the presentations. The pre-clinical presentations focused on the hematopoietic stem cell (HSC) work and the CXCR4 deletion work.

My previous [report](#) covered the presentation from Monday, so I wanted to add a couple of impressions from Wednesday presentations. In particular, the presentations highlighted two pieces of information that have not been widely circulated. First, the pre-clinical work by Paula Cannon that is examining the HSC CCRD deletions is getting closer to the clinic and we now have a glimpse of a phase I trial design. Second, the phase I data presented by Carl June has two patients that underwent a treatment interruption (IT) that occurred after the reinfusion of the modified T-cells. As such, it provided our first glimpse of how the modified cells operated in the presence of a viral load.

## Watch the Presentations

CROI has a [website](#) dedicated to the presentations that occurred at the conference. From this page, you can access all of Sangamo's presentations, which includes the slides. Anyone interested in SGMO and the HIV program would certainly benefit from watching these.

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## Treatment Interrupted Patients

While only having data on two patients, there was certainly encouraging data that the modified T-cells survived the presences of the virus and may have even been able to reduce viral load. Of course, this needs to be verified with additional data and more patients but this is a good start.

As way of background, a number of patients with controlled viral load received the modified T-cells and then have a structured treatment

interruption before going back onto HAART. During this TI a number of events occurred. First, viral load increased, which makes sense because there were still unmodified cells in the blood. Second, this increase was delay by 10-weeks in one patient (good news). Third, and most promising, viral loads in both patients began to decrease BEFORE they went back on HAART. This is evidence (although not conclusive in any way) that the modified T-cells were able to help the body fight the virus.

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### Manufacturing?

One problem commonly associated with treatments that remove cells from the body, modify them, and then re-infuse them is that this is a complicated and difficult process. As such, can these treatments be upscaled to make them commercially viable? This is part of the problem that Dendreon (DNDN) has encounter with Provenge.

Sangamo and their partners understand this difficulty and Carl June presented data that indicated that this process has the potential to overcome these issues. He showed that the modifications for these trials were done in two different clinics and yielded similar quality products. Again, while not a decisive answer to the manufacturing question, it is certainly an encouraging start.

## HSC: Coming to the Clinic

Paula Cannon is working on the pre-clinical HSC modifications. While her presentation contains a lot of interesting information about how these cells are being modified, what seemed interesting as well was the approach they plan on taking in the clinic. The plan is to essentially piggyback on lymphoma chemotherapy. In particular, they will be using HIV positive patients who are also being treated by chemotherapy for lymphoma.

The usual chemotherapy treatment for lymphoma patients is to first remove some HSC before the treatment as the chemotherapy kills the HSC. Once the chemotherapy is finished those HSC are re-infused. As a result of this procedure, the lymphoma is treated and their HSC saved. For the phase I trial, Lymphoma patients with HIV are going to undergo the same treatment but before the HSC are re-infused they will be modified to knockout CCR5. Thus, these patients will both be cleared of their lymphoma and have a large set of HSC with the CCR5 knocked out. This will come very close to replicating what happened with the Berlin patient (although he had a bone marrow transplant). The key in this trial is to see how the HSC engraft, expand, and circulate. In addition, it will be interesting to see if the viral loads increase. Ideally, this treatment will protect the system and limit the virus thus coming close the a functional cure (assuming that the virus cannot enter through CXCR4).

It should be clear that this trial design is very preliminary and there is still some pre-clinical studies that need to be completed before an IND can even be filed. In addition, the company nor the presentation gave any guidance as to when to expect an IND for the HSC treatment. Given my personal reading of the presentation, I think they are relatively close and think than an IND would be submitted in late 2012 or early 2013.

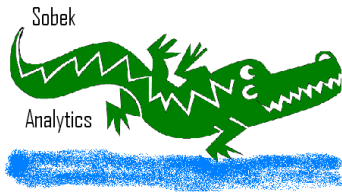
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## Disclaimer

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. In addition, I am long SGMO shares and March \$8 calls.

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