

THE WALL STREET TRANSCRIPT

Connecting Market Leaders with Investors

Soligenix, Inc. (NASDAQ:SNGX)



DR. CHRISTOPHER J. SCHABER is presently Chairman, President and CEO of Soligenix, Inc. (NASDAQ:SNGX), a rare disease biotechnology company developing drug therapies for multiple orphan diseases, which includes a Phase III pivotal clinical trial for cutaneous T-cell lymphoma and a Phase III pivotal trial for oral mucositis in head and neck cancer. Dr. Schaber has spent his 28-year career dedicated to the development of drug therapies in rare diseases and areas of unmet medical need.

SECTOR — PHARMACEUTICALS

TWST: Bring us up to speed on the company's late-stage drug candidates and their development status.

Dr. Schaber: As you may recall, we have two active segments at Soligenix: a therapeutic business segment focused on oncology and inflammation and a vaccines/biodefense business segment. In the therapeutic segment, we have two lead programs both enrolling patients in pivotal Phase III clinical trials.

The first is a novel photodynamic therapy for the first-line treatment of a rare cancer called cutaneous T-cell lymphoma or CTCL. In treating this disease, which consists of cancerous lesions or tumors on the skin, we apply a proprietary topical drug called synthetic hypericin — research name SGX301 — to the lesions followed by activation of the drug with a short course of safe visible fluorescent light from a specialized light device. We expect to complete our blinded interim analysis later this year with final study results in the first half of 2019.

The second Phase III study uses a new class of drugs, referred to as innate defense regulators, or IDRs, that modulate the body's own innate defense system to treat disease. Our first-in-class drug called dusquetide — research name SGX942 — is for the treatment of oral mucositis, which are ulcerations in the mouth and throat caused by chemoradiation therapy in patients being treated for head and neck cancers. Here, we are administering a short four-minute IV infusion of dusquetide — research name SGX942 — twice weekly during the patient's chemoradiation treatment to potentially reduce the duration of severe oral mucositis as well as to impact other important measures like infection and survival.

Severe is defined as ulcers so painful that the patient can no longer eat and/or drink. It is just an unimaginable pain that oftentimes can require hospitalization for dehydration and/or malnourishment. We

currently anticipate the blinded interim analysis to occur in the first half of 2019 with final study results coming in the second half of 2019.

I will briefly note that we also have a Phase III pediatric Crohn's disease program with a separate molecule, oral beclomethasone dipropionate — research name SGX203. This pivotal Phase III study has been cleared through the FDA, but we will not be initiating it until additional funding and/or a partnership is secured.

Our lead program in the vaccines/biodefense segment, which is funded entirely by the U.S. government, is our heat-stable ricin toxin vaccine called RiVax, which is the equivalent of Phase II development program. In 2018, we expect to complete a preclinical efficacy study and initiate a Phase I/II clinical safety study in healthy volunteers.

TWST: In your literature, you noted blinded interim analyses for the two lead clinical programs. For those who are not clinical-development-savvy, what is a blinded interim analysis, and why is it important?

Dr. Schaber: Yes, I have been asked this question several times since putting out our investor update in January. If you will bear with me, it's probably best if I provide a little more background before addressing its importance. Many randomized clinical trials are double blind — meaning that the participant or patient, their doctor, and even the study personnel at the organization sponsoring the trial do not know what treatment is being given to the patient. This study blind is "broken" and the true assignments disclosed to study personnel only after the trial is stopped and/or completed, and the study database is finalized or what is referred to as "locked."

Because of this double-blind approach to clinical testing, and due to the fact that often times an unproven procedure or drug treatment is being tested, many clinical trials today use what is known as a data

monitoring committee, or DMC, which is an independent group of experts who monitor patient safety and treatment efficacy data while the clinical trial is ongoing, in accordance with a prospectively defined analysis plan. The DMC is a group, typically three to as many as seven members, who are independent of the organization — also referred to as the sponsor — conducting the trial. At least one DMC member will be a statistician, with the remainder usually consisting of clinicians knowledgeable and experienced in the disease indication being studied.

The DMC will convene at predetermined intervals, depending on the type of study, to review unblinded results. The DMC has the power to recommend continuation or termination of the study based on the evaluation of these results. There are typically three reasons a DMC might recommend termination of the study: safety concerns, outstanding benefit or efficacy, and futility.

The number of patients that are to be enrolled into a clinical trial is usually determined by making estimates on the expected outcome in the control group, the expected amount of the improvement anticipated in this outcome in patients receiving the study drug treatment, and how sure the sponsor wants to be that they are unlikely to miss a clinically significant improvement. These estimates are usually based on the previous studies conducted, but they may not accurately predict what will be seen in the specific trial, even if it is structured identically to previous studies.

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As a consequence, the DMC can also be asked to provide guidance on enrollment in the study and recalculate the number of patients that need to be enrolled into the trial based on the actual outcomes rather than the “best estimates” of the original calculation. If the outcomes remain in line with those original assumptions, the DMC will recommend continuing to enroll to the original calculated number of patients. Alternatively, the DMC can propose recalculating or reestimating the sample size, if the study is not completely in line with the original assumptions. In reestimating, the DMC can recommend the sponsor enrolls additional patients to realign the study to maintain the same probability that a positive result will be statistically significant as originally planned.

In any case, the only information transmitted to the sponsor, the participating hospitals and the participants is a recommendation to halt the trial, continue the trial as planned, or continue the trial but change the total number of patients to be enrolled. As the study remains blinded, no information on outcomes or reasons for the recommendation are shared. This guidance is obviously important, as it provides added assurance regarding the quality of the trial and its probability of succeeding.

It’s important to note though that the inclusion of a DMC interim analysis does not guarantee success; however, the analysis and DMC recommendation provides a good sense of how the trial is tracking with original assumptions at a snapshot in time. Obviously, if a DMC came back with a recommendation to continue enrolling and to add a reasonable number of additional patients, that would be perceived as positive with a higher probability of success than when the study first started.

TWST: I was looking at some of your literature, and you have forecasted for everything in your pipeline a minimum market potential of \$200 million. Is that a worldwide figure and is that a conservative figure?

Dr. Schaber: Good question. That is both a worldwide figure and what we believe to be a conservative figure. We have not come out with any external formal marketing analysis or reports as yet, but intend to do so when the Phase III clinical studies are nearing or are completed.

TWST: You have orphan and/or fast track designations across your pipeline with the possible exception of the vaccine platform. Help us understand, for many people who may not understand, what that designation means in practical terms as far as benefits like approvals being expedited?

Dr. Schaber: We not only have the orphan drug designation, which typically means your drug is treating a rare disease of 200,000 or less patients in the U.S. and confers 7 years of market exclusivity upon approval, but we also have what is known as fast track designation. With fast track designation, this is defined by the FDA as an area of unmet medical need where there is typically no therapy that is currently approved or that adequately satisfies that disease indication. When you look across our pipeline, including biodefense, you have orphan diseases that are also in areas of unmet medical need that have fast track designation or potential for this

designation. We are operating in areas where there currently is no adequate or FDA approved therapy in treating the disease, so we are in a unique space across our pipeline.

TWST: Last December, the company announced news on biomarkers for the ricin toxin, RiVax. What does this mean for testing and quickening the rate of movement through development for this?

Dr. Schaber: Just as background, for medical countermeasures for biodefense purposes, the FDA approves based on what is known as the “Animal Rule,” which is applied to product candidates where testing in clinical trials would be unethical. In the case of a ricin toxin vaccine, clinical efficacy testing of the vaccine is unethical since exposing unvaccinated humans to ricin toxin would be fatal. The Animal Rule combines safety studies in humans and efficacy testing in animals, typically nonhuman primates, to facilitate approval. It is generally associated with the approval of medical countermeasures for biodefense purposes. Key to the application of the Animal Rule is the requirement to establish a correlation between the response observed in clinical trials in healthy volunteers with the response demonstrated in animal efficacy studies.

I am pleased to say that we have identified a biomarker to facilitate the demonstration of the correlation between the animal and human, which is a significant accomplishment in our RiVax development program. These biomarkers appear to be consistent across mice, nonhuman primates and humans, to support the application of the Animal Rule. So once biomarkers are identified, it does allow for the potential

acceleration of development. As I noted earlier, we plan to conduct preclinical and clinical studies in 2018 with data becoming available in late 2018 and 2019. We are currently advancing this program with the financial support of a NIH contract award of up to \$24.7 million.

TWST: Now, you have both this ricin toxin pre-exposure candidate in the pipeline as well as something called OrbeShield, which is for GI acute radiation syndrome. Can you place both of those in the context of what their approval or potential use might mean for defense or military use so a civilian can understand?

Dr. Schaber: With biodefense, you are preparing for and/or responding to a bioterrorist attack; medical countermeasures like RiVax or OrbeShield are meant to protect against or to be used after such a catastrophic event. For example, with lethal ricin exposure, you are looking to vaccinate folks to protect them from that exposure. It is important with ricin to give a vaccine because the potentially lethal effects of ricin become irreversible very quickly after exposure. A RiVax vaccine is very important here. We would initially focus on military and first-responders to make sure that they are protected from this exposure.

What you want to ultimately be able to do at the end of the day in getting FDA approval and moving this forward is have the government stockpile or procure your particular compound or product so that it is available in case there is a bioterrorist attack, so it could be distributed to those that need it. An interesting development in the area of biodefense is that, recently, over the last year, the U.S. government has put in place what is known as a priority review voucher — PRV — program. Similar to PRVs that are currently being awarded for pediatric rare diseases and tropical diseases, a biodefense PRV would apply to those medical countermeasures, like RiVax, that may be fortunate enough to receive FDA approval. Once approved, the FDA has the potential to award a PRV that could then be used to get a shorter, priority review cycle with your next new drug application — NDA — that would not normally qualify for a priority review.

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Importantly too, since these PRVs are also transferrable, is the potential to sell them for cash. PRVs in pediatric rare or tropical diseases have sold for upward of \$300 million to companies accelerating the review of their next potential blockbuster drug. Think about the additional revenue that could be generated with a reduction of six months on an NDA review cycle. This PRV potential in the biodefense sector is another very important consideration in addition to receiving government procurement contract awards.

TWST: Can you also provide an update on the platform technology called ThermoVax and remind us what it does?

Dr. Schaber: As you may know, the vast majority of vaccines today require refrigeration to maintain their protectiveness. Therefore, maintaining these storage conditions at all times is critical and also expensive, especially during shipping of the vaccine from one location to another. Our proprietary ThermoVax is a vaccine thermostabilization platform technology

that allows us to take a liquid vaccine of certain construct and freeze-dry it to a powder whereby it can then be stored outside the refrigerator at high temperatures for extended periods of time. The vaccine can be administered when needed by mixing it quickly with water.

We use the ThermoVax technology with our own ricin toxin vaccine RiVax, where we have demonstrated that once in the ThermoVax system, our vaccine can be stored at over 100 degrees Fahrenheit for one year. We have also demonstrated similar results with an anthrax vaccine, a HPV vaccine and, recently under collaboration with the University of Hawaii and Hawaii Biotech, with Ebola vaccine antigens. This collaborative work remains ongoing and is fully funded by the NIH.

TWST: And testing so far has shown that the length of time it is sitting or exposed to certain extreme temperatures does not alter the nature of the vaccine or its potency?

Dr. Schaber: Correct. So again, as an example, our ricin toxin vaccine, as a liquid, is extremely labile. As a liquid you take it out of the refrigerator, sit it on a table, and it begins to degrade within hours. However, put it into this ThermoVax system, we have demonstrated some very good data that you can store it outside the refrigerator at over 100 degree Fahrenheit for one year and, upon reconstitution of the dry powder with the commodity sterile water for injection, it maintains 100% of its activity, zero degradation. We have been very pleased with results generated thus far.

TWST: You had patent issuance on SGX942. Can you talk about what that means and the patent life for that compound?

Dr. Schaber: It is very positive as it continues to strengthen and broaden our IP protection around the innate defense regulators and dusquetide specifically in oral mucositis. In addition to issued composition of matter patents that range anywhere from 2026 to 2030, depending on the region of the world where that patent has been issued. We have patent protection for oral mucositis out into the 2030s. The

innate defense regulators and dusquetide have quite a long patent life, which as you know, is very important in drug development.

TWST: It is called an innate defense regulator, and it sounds to me like it has a potentially broad application, is that correct? What does its name mean regarding its capabilities, and also why did you go first for oral mucositis?

Dr. Schaber: As I noted earlier, innate defense regulators or IDRs are a new class of compounds that modulate the body's own innate defense system to reduce inflammation, clear infection and accelerate tissue healing. To your point, there are broad applications with this technology beyond oral mucositis, especially in emerging infectious diseases, which we are in the process of evaluating and have already completed a number of proof of concept preclinical studies. We show very positive effects for IDRs as standalone therapy or in combination with antibiotics.

Why we chose oral mucositis? As you would imagine, oral mucositis is closely aligned with the effects that the IDRs exert. It is also known now that oral mucositis is caused by dysregulation of the innate immune system when patients are receiving chemo and/or radiation therapy. So obviously if we can modulate reduce inflammation then that is very important in treating oral mucositis, as well as potentially reducing infection and healing tissue that comes along with having these open ulcerations in the mouth and throat. What we have seen thus far with our Phase II clinical data is that dusquetide or SGX942 can reduce the duration of severe oral mucositis in head and neck cancer patients, while also having a potential positive impact on infection, tumor response and long-term survival. In addition, oral mucositis is an area of unmet medical need since there are currently no approved drug therapies available; therefore, we have the potential to accelerate or fast-track development.

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TWST: What percentage of patients receiving chemotherapy has this condition?

Dr. Schaber: We focused on head and neck cancer patients because, as you would imagine, the incidence of developing severe oral mucositis is extremely high, meaning in the 70% to 80% range. When you’re doing clinical studies, you would like to show your effect in the smallest number of patients as possible. We thought this patient population was one where we could have a relatively small study at the end of the day in patients who desperately need a safe and effective treatment in managing this devastating side effect of chemoradiation for their cancer. There are other cancers for which you see oral mucositis but at a lesser amount, such as breast cancer where it usually occurs in the 40% range.

TWST: It is saying here in your literature too that anticancer therapies can cause damage to mucosal regions anywhere in the body, and so it potentially has application to other mucosal areas it sounds like?

Dr. Schaber: You are correct, as anticancer therapies can cause damage to other mucosal regions, IDRs have the potential to also treat the damage along the entire GI tract as well. Beyond oral mucositis, there are a number of disease indications where we intend to investigate and/or expand testing of the IDR platform. It is a very exciting and potentially transformational technology.

TWST: Right now your focus is developing your pipeline and not licensing assets out, correct?

Dr. Schaber: While we are advancing our two Phase III clinical programs in CTCL and oral mucositis in head and neck cancer, we remain very opportunistic on the business development front. As you may have seen, we have a collaboration in place with SciClone Pharmaceuticals for dusquetide in the Greater China market, and continue to be active in identifying and/or pursuing additional partnership opportunities across our entire pipeline. It is a high-priority focus of ours, assuming the right opportunity comes along.

TWST: What does that agreement with SciClone entail? What does that require the parties to do exactly?

Dr. Schaber: For us, we are responsible for the overall development of the program. SciClone is responsible for any additional development work that needs to be done in China as well as the regulatory filings in the territory. In China, they are also responsible for commercial build, launch, sales and marketing activities, for which Soligenix will receive a reasonable royalty.

TWST: You have been very creative with financing, particularly non-dilutive financing. Can you give us an update on your financing status and also what you are currently doing right now?

Dr. Schaber: As it relates to non-dilutive funding from government agencies, you are absolutely correct. We are very aggressive in our pursuit of non-dilutive funding. We are currently operating under a contract with the NIH for our ricin toxin vaccine, RiVax. This contract

is up to \$24.7 million. We also in 2017 received additional NIH grants of approximately \$1.5 million for each of our active Phase III clinical programs in oral mucositis and CTCL.

In 2017 alone, we received approximately \$8.5 million in non-dilutive funding. In addition, we began 2018 with approximately \$8 million in cash from equity financings. We did an equity raise in late 2017 with fundamental quality investors led by Knoll Capital Management and Act Capital Management. Completing that raise of approximately \$5 million in gross proceeds, along with the non-dilutive funding from government grants and contracts, puts the company in a nice position going into 2018, as we advance our two Phase III clinical programs.

TWST: In terms of the non-dilutive funding, and particularly the government funding that you’re receiving, what does that mean in regards to who owns the IP at the completion of the project?

Dr. Schaber: Soligenix owns the IP at the end of the day. It is like developing any other drug outside of government funding. We can commercialize it as we see fit. Obviously with biodefense, where you have one customer being the government, we do need to negotiate and make the drug available to them at a reasonable price for potential stockpiling. The only added stipulation that is in every government grant or contract is, that if a company can no longer make or no longer wants to make a product, that the government then has the right to step in and make it for themselves. To my knowledge that has never been done, and obviously if you are developing something, you want to make and sell it at the end of the day.

TWST: What do you consider to be the company’s most significant achievement in the last year?

Dr. Schaber: When looking back at the last year, I believe we had several significant achievements. We were actively enrolling patients in our pivotal Phase III study in cutaneous T-cell lymphoma. We initiated a pivotal Phase III study in oral mucositis in head and neck cancer. We uplisted to Nasdaq in December of 2016, which I think contributed to us

completing a strong financing in late 2017 with high-quality investors like Knoll and Act Capital. As I noted, we were also fortunate to bring in approximately \$8.5 million in non-dilutive funding in 2017. So I think we generated some positive momentum going into the start of 2018.

TWST: What are the company's strategic objectives for the next 12 months?

Dr. Schaber: For the next 12 months, our focus is the quality execution of our Phase III clinical programs to get to data as quickly as possible. We also want to make sure that we continue to get our story out there like we are doing today with this interview, and to be active on the business development front in pursuit of potential opportunities for collaboration across our pipeline.

TWST: Where do you envision Soligenix in five years?

Dr. Schaber: That is difficult to say. A company our size is striving to be successful, and that success can take many forms, if we have the good fortune of achieving what we expect to achieve with our pipeline programs — merger, acquisition, or going it alone and commercializing in some of our rare disease indications. For example, if we were to commercialize in cutaneous T-cell lymphoma, as that program is the closest to completing Phase III, this is a highly specialized disease area. It does not require a large commercial unit to move this forward effectively. We definitely have the potential to move this forward ourselves as we look to build shareholder value in the future. Biodefense is another example where a small company could commercialize on its own, where it would essentially have the government as its one customer.

TWST: What do you want a potential investor to know?

Dr. Schaber: Given that we are in late-stage Phase III development with multiple shots on goal along with the current price point of the company's stock, there is the potential for significant growth

and upside, assuming we achieve what we need to achieve. I truly believe we are extremely undervalued and below many investors' radars.

Just to briefly summarize, we are developing therapies in disease areas where there is high unmet medical need and currently no approved therapies for our anticipated indications. We will have Phase III data readouts in two cancer/cancer supportive care trials as soon as this year and next. We have received a good level of validation with our pipeline to date from the U.S. government through significant non-dilutive funding awards as well as the continued business interest we are garnering. Most notably we have a partnership secured with SciClone Pharmaceuticals for dusquetide for the Greater China market, which we touched upon.

It is a very exciting time for Soligenix with the potential for meaningful and positive impact to first and foremost patients but also to the stock price assuming positive outcomes in our clinical trials.

TWST: Is there anything else you wanted to mention?

Dr. Schaber: I would just maybe point out that we have a lot of good useful information on our corporate website, such as investor and medical presentations, webcasts and publications. For those that want to learn more, about the company and its programs, please go there.

TWST: Thank you. (KJL)

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