Event Name: Soligenix Announces Topline Results from its Phase 3 Clinical Trial of SGX942 for the Treatment of Oral Mucositis in Head and Neck Cancer Patients

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Officers and Speakers
Jonathan Guarino; Soligenix, Inc.; Chief Financial Officer
Christopher Schaber; Soligenix, Inc.; Chairman, President and Chief Executive Officer
Richard Straube; Soligenix, Inc.; Chief Medical Officer
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Presentation

Operator: Welcome to the Soligenix corporate update conference call.

(Operator Instructions)

As a reminder, this is a timed conference call today and is being recorded.

I would now like to turn the conference over to Mr. Jonathan Guarino. Please go ahead, sir.

Jonathan Guarino: Good morning. This is Jonathan Guarino, Chief Financial Officer at Soligenix. Thank you all for participating in today's call.

Joining me from Soligenix are Dr. Christopher Schaber, President and Chief Executive Officer; Dr. Richard Straube, Chief Medical Officer; Dr. Oreola Donini, Chief Scientific Officer; and Mr. Daniel Ring, Vice President of Business Development & Strategic Planning.

Before we begin, I would like to caution that comments made during this conference call by management will contain forward-looking statements that involve risks and uncertainties regarding the operations and future results of Soligenix. I encourage you to review the company's past and future filings with the Securities and Exchange Commission, including, without limitation, the company's forms 10-K and 10-Q, which identify specific factors that may cause actual results or events to differ materially from those described in the forward-looking statements.

Furthermore, the content of this conference call contains time-sensitive information that is accurate only as of the date of the live broadcast, December 22, 2020. Soligenix undertakes no obligation to revise or update any statements to reflect events or circumstances after the date of this conference call.

With that said, I would like to turn the conference call over to Dr. Schaber. Dr. Schaber?

Christopher Schaber: Thank you, Jonathan. Good morning, everyone, and thank you for joining us. Although I wish the occasion for this conference call was more positive, we thought it would

be beneficial to our shareholders for us to provide an update on the top-line results from the Phase 3 DOM-INNATE study of SGX942, or dusquetide, in the treatment of oral mucositis in head and neck cancer patients.

So with that, I'll get right into the subject at hand. As many of you on the call this morning have probably seen, we announced that the DOM-INNATE study did not meet expectations. Unfortunately, after unblinding the double-blind placebo-controlled study, it was revealed that the required level of statistical significance for the predetermined primary efficacy endpoint was not achieved. This is obviously extremely disappointing to everyone working on the program: the Soligenix employees; our dedicated investigators and their staffs; you, the shareholders; as well, and most importantly, the patients suffering from this disease, and their families.

What makes this result even more frustrating is the fact that there was clear evidence that the drug had a clinically meaningful impact on the disease similar to the improvement seen in the Phase 2 study but failed to reach the predefined level of statistical significance, a p of less than or equal to 0.05. Given that the independent review committee recommendation adjusted sample size to maintain the high 90% statistical power, we were all cautiously optimistic that we had further de-risked the trial for potential success; however, this was not the case.

We aren't prepared at this time to provide a definitive reason or reasons for this unexpected result; however, the company will look to initiate analyses of the un-blinding data to obtain further clarity as to the potential cause or causes of the outcome. Depending on what we uncover following this review, we may request a meeting to discuss our findings with the FDA and/or EMA and potential next steps forward, if any. We will also provide you with an update on those findings.

Before turning the call over to Dr. Richard Straube, Chief Medical Officer of Soligenix, to provide a bit more detail, I want to reiterate that the biologic activity of SGX942 is apparent, and we remain committed to determine if there is a path forward so that we may provide treatment options for this area of high unmet medical need.

And even though this call is about the disappointing news with top-line results for SGX942 in the treatment of oral mucositis, I would be remiss if I didn't make note of our robust pipeline across our Specialized Biotherapeutics and Public Health Solutions business segments, including the positive and statistically significant Phase 3 results achieved in the FLASH study of SGX301 in the treatment of cutaneous T-cell lymphoma, or CTCL, earlier this year, where we are currently preparing a new drug application for submission to the U.S. Food and Drug Administration, or FDA, while continuing our activities towards U.S. commercialization, where we expect the SGX301 market opportunity to exceed $75 million annually. Keep in mind that this is a $250-million global market opportunity.

We also continue to pursue our Public Health Solutions business segment with an ongoing $21.2-million contract supporting RiVax, our ricin toxin vaccine development; renewed focus on our Marburg virus vaccine program; continued evaluation of SGX943 as an anti-infective agent under a DTRA subaward; and continued development of our heat-stable COVID-19 vaccine program, CiVax, which is beginning to garner added interest by the U.S. government.

Also, just a brief note on our financials. Current cash as of our call this morning is approximately $20 million. Some of you may recall that our cash burn is offset by nondilutive government funding, along with an approximate $1.1 million we anticipate receiving from our New Jersey NOL tax credit and U.K. tax incentive credit, which gives us a cash runway of about 24 months or two years. This puts us in a good position to execute on our multiple programs across our rare disease pipeline, including working towards FDA approval and commercialization of our CTCL product in the U.S. while in parallel being aggressive in evaluating all strategic options. We are also investigating additional potential assets to fill our clinical program pipeline. In addition, keep in mind that we have an ATM in place with B. Riley FBR, which we can use to supplement cash if or when the need arises.

With that said, I will now turn the call over to Dr. Richard Straube, our Chief Medical Officer, who will review the Phase 3 oral mucositis study. For those of you not familiar with Rick's background, he's had a long and distinguished career of more than 30 years in both academia and industry, most notably with the University of California at San Diego, Centocor and INO Therapeutics. He is a board-certified pediatrician with a deep research background in orphan disease development, oncology, immune modulation and infectious diseases, with a number of successful clinical trials and drug approvals under his belt. Rick?

Richard Straube: Thank you, Chris. As a background to the discussions concerning the results of this trial, I think it's important to understand the truly difficult nature of this disease. Oral mucositis (OM) is a byproduct of chemotherapy and radiation for its tumor treatment. It is initiated by the direct damage caused by the chemotherapy and the radiation that's necessary to treat the cancer. This initial damage is then amplified by the body's over-exuberant inflammatory response by the innate immune system, leading to the development of ulcers throughout the entire GI mucosal surface. They are particularly painful in the mouth. There is no approved drug for oral mucositis in head and neck cancer or any other solid tumor setting, making it an area of unmet medical need.

For most patients and doctors, managing OM is about managing through the pain, which is often not well controlled by even opioids, and the hoping that subsequent damage and inability to eat and/or drink does not result in significant dehydration, malnutrition or infection, all of which can require secondary hospitalization. Unfortunately, the pain can become so bad the patients will interrupt or stop their tumor treatment. Treatment gaps lasting more than one week have a significant impact in our ability to control the tumor, and this cannot be made up with longer treatment, so it is much preferred to treat the oral mucositis and preserve the optimal tumor treatment regime. Our drug, SGX942, does not interfere with primary tumor treatment but does modulate the downstream inflammatory response, as well as providing enhanced anti-infective activity.

In the Phase 2 study, in the subpopulation that included the same patients that were enrolled into the Phase 3 study, SGX942 statistically significantly reduced the duration of oral mucositis. In this trial, we measured the development duration of severe oral mucositis, meaning ulcers in the mouth so painful that they prevented the patient from eating, or eating and even drinking. This

level of disease has a maximal impact on the patient's prognosis with regard to secondary hospitalizations, infections and potential drop-off from all cancer therapy.

In the patients who strictly adhered to all procedures in the protocol in this trial, we saw a statistically significant 50% reduction in the median duration of severe oral mucositis from 18 days in the placebo group to 9 days in the SGX942 group. Unfortunately, in the larger patient population, the 56% reduction in median duration of severe OM from 18 days in the placebo group to 8 days in the SGX942 group did not cross the required threshold for being statistically significant, which is a p value of less than 0.05. This outcome is extremely disappointing in light of the biologic activity observed in the Phase 3 trial that provided a clinically meaningful benefit that was consistent with what we had seen prior in the Phase 2 study.

Unfortunately, we do not have an understanding of what may have occurred at this time. Over the coming weeks, we'll look to analyze the data further to determine if we can more definitively define what may have occurred. For example, there may have been subpopulations of patients that did or did not respond to SGX942 treatment, or there could have been some unexpected variability in the placebo patient population, which could have strongly impacted the statistical methods used. Based on these findings, we hope to have a clear understanding that may allow for a path forward for this program.

Although this trial demonstrated clinically meaningful benefit, the lack of statistical significance in the predefined population, as defined in the protocol, makes it unlikely to be the sole support for regulatory filings in either the U.S. or the EU. Having said that, if we do see something meaningful in the data set, we would anticipate having a discussion with the FDA and/or the European agency to better understand the potential path forward for the OM program, if any.

Given the high-level observations we have seen to date with Phase 3 data, we can say: First, we know that SGX942 has biologic activity in this patient population; second, we know that the response rates that were seen in the study are consistent with previous clinical and preclinical data; and thirdly, we know it was safe and well tolerated. It's also important to note that dusquetide's unique mechanism of action that reduces the innate immune system's response by lowering inflammation while at the same time increasing both bacteria clearance and wound healing has the potential to be used in a variety of other important indications. We also have recently shown that other routes of administration -- for example, intramuscular injection -- are feasible, and this opens the door to its use in both biodefense and even potentially home use.

In summary, although we did not demonstrate statistical significance in the study's primary endpoint, the biologic activity of SGX942 is clearly evident. In the coming weeks, we will be conducting a more thorough analysis of the results to better determine a potential path forward with the OM program.

Finally, for clarity, I would also note that although these results are disappointing, they in no way diminish the outcomes of our other Phase 3 trial earlier this year where we saw strong and efficacious response to our SGX301 drug for the treatment of cutaneous T-cell lymphoma, and where we continue to progress towards regulatory approval and commercialization.

I will now hand over to Dr. Donini to provide a brief update on our Public Health Solutions business segment. Oreola?

Oreola Donini: Thanks, Rick. While we are obviously disappointed by the data produced in this Phase 3 study, it's important to remember that our robust rare disease pipeline continues to move forward. We are continuing to develop the SGX301 product, as Rick mentioned, with its statistically significant Phase 3 results this year in cutaneous T-cell lymphoma.

We also continue to develop our Public Health Solutions assets, including our ricin toxin vaccine, RiVax. This product has been proven to be safe and efficacious in animal studies, safe in Phase 1 human studies, and has also been formulated as a thermostabilized product, stable at room temperature. Further development here will require additional manufacturing and clinical studies, as well as pivotal animal efficacy studies under the animal rule in lieu of Phase 3 clinical studies. We estimate the market for this product would include procurement contracts not only from the U.S. government but potentially from other government contracts as well. The product may also qualify for a priority review voucher, which has the potential to provide added value, significant value, to its assets.

Using this same thermostabilization technology, we are also working on a COVID-19 vaccine, which has shown very promising responses in preclinical animal studies. Relative to other vaccine candidates, our COVID vaccine, which we call CiVax, would be heat-stabilized and delivered in a single-dose vial, which could be reconstituted with sterile water for injection. This would mean the product could be stored and distributed at ambient temperatures with no fridges or freezers needed and could be used on a single-dose basis as needed after reconstitution. The vaccine platform we are using is a protein subunit platform, one of the best characterized platforms, with a well-established track record of safety. Moreover, the platform can also be used to multivalent vaccines, potentially including antigens from multiple versions of the SARS-CoV-2 virus, should that be necessary.

While our product is clearly earlier in development and will not be ready for first-generation use in the developed world, it may make significant contributions to protecting the worldwide population and to supporting any need for annual or seasonal revaccination. We continue to pursue funding for this program and to develop it in partnership with our collaborators at the University of Hawaii.

With this high-level update, I will now hand it back to Dr. Schaber to conclude the call. Chris?

Christopher Schaber: Thank you, Rick and Oreola, for those comments.

Before going to Q&A, I wanted to briefly provide some concluding remarks. As you can tell from this morning's call, we are all very disappointed in this outcome, especially in light of the biologic activity that has been observed with SGX942 in the Phase 3 and in previous trials. Over the coming weeks, we'll work to better understand what may have occurred with the trial; however, we will also be focusing on advancing towards regulatory filings and commercialization for SGX301 in CTCL.

Now, prior to going to Q&A, I would also like to again caution everyone that at this time, we are unfortunately limited in what we can say regarding the Phase 3 data.
With that, we will now take any questions. Operator?

Questions & Answers

Operator: With the time remaining, we will now open the line for electronic questions.

Jonathan Guarino: This is Jonathan Guarino again. Let's begin our Q&A, as we've received a number of questions. We will clearly not be able to address all questions but will look to address as many as possible in the time remaining.

The first question: Are therapies for OM -- other therapies for OM have failed in the past as well. Do you think there are intrinsic problems with this disease and/or patient population that impact clinical results?

Christopher Schaber: Jonathan, why don't we let Rick take this one. Rick?

Richard Straube: Thank you. It is true that OM is a multifactorial disease. It incorporates the direct damage done by chemoradiation treatment, the downstream inflammatory response and frequent bacterial infections with the ulcers. Unfortunately, we can't mediate the chemoradiation treatment directly without impacting tumor control. Several treatment regimes have failed because they've targeted only the secondary infections of ulcers, which are late in the stage of the disease.

Our treatment differed in the modulation of both the infection but also, more importantly, the inflammation, and given the biologic signals observed, we think this approach still has a great deal of merit. However, to your point, due to the multifactorial nature of the disease, there is a wide variation in the response profile among patient populations, be it both placebo as well as treated, and this certainly impacts both the study design and sample size in a very unpredictable and probably challenging manner.

Christopher Schaber: Thanks, Rick.

Jonathan Guarino: Great, thank you, Rick. Our next question: If SGX942 didn't work with OM, do you think it might still work in other indications?

Christopher Schaber: Rick, do you want to take this one as well, please?

Richard Straube: Sure. First, let me clarify that in our mind, SGX942 did work in OM. It demonstrated consistent reductions in the duration of severe OM in two clinical studies. However, unfortunately, in this Phase 3, it did not cross the statistical threshold of a p of less than 0.05 to declare it a success.

Regarding other potential indications, given the preclinical and Phase 1 clinical data showing control of inflammation while increasing the host's ability to clear bacteria and enhance wound

healing, there is definitely a potential for anti-inflammatory and antibacterial indications for dusquetide, particularly in the public health arena. With the results in the per-protocol population in the Phase 3 study, all of this shows a very strong biologic signal, which is supportive of the preclinical results in the other indications.

Christopher Schaber: Thanks, Rick.

Jonathan Guarino: Our next question: This is very disappointing and unexpected. What is your current cash position? With the cash currently in the bank, will you be able to complete the regulatory application and approval process and commercialize SGX301? Chris, do you want me to take this one?

Christopher Schaber: Yes, Jonathan, please.

Jonathan Guarino: I agree; it is an extremely disappointing outcome. The current cash as of this morning is approximately $20 million, not including our nondilutive governmental funding. This gives us a minimum cash runway of at least 24 months, so yes, with our current cash resources, we anticipate being able to complete the regulatory approval.

I'd just like to remind everyone listening that the SGX942 program uses a completely distinct drug chemical entity from SGX301 in a completely different disease indication.

Our next question: If you decide to continue to pursue SGX942, what might that cost?

Christopher Schaber: Jonathan, I'll take this one. In the short term, there are no additional significant costs as we look to complete the mandatory follow-up. Clearly, we won't be accelerating manufacturing or regulatory-related activities in the short term. Over the next several months, depending on findings from further investigations and potential discussions with the health authorities, we may propose additional activities. We'll have to wait and see.

Jonathan Guarino: Thanks, Chris. Our next question: Do you think these findings with OM impact the CTCL indication at all, or any of your other programs?

Christopher Schaber: Again, absolutely not. Our CTCL program is a completely distinct chemical entity and new drug product. This is a completely separate program. Nothing about the oral mucositis program impacts our potential approval and commercialization activities with SGX301. We plan to provide additional clarity on the CTCL commercialization activities in the new year. We've been doing a considerable amount of work.

Let me just add that we also continue to pursue our Public Health Solutions programs, including our ricin toxin vaccine, for which we have recently announced extended durability of protection; the Marburg vaccine and the COVID-19 vaccine, CiVax, which again is beginning to garner interest from the U.S. government. Thanks, Jonathan.

Jonathan Guarino: Thanks, Chris. Our next question: How do you explain the failure of the Phase 3 trial in light of the success and magnitude of response observed in the Phase 2 trial?

Christopher Schaber: Rick, do you want to take that one, please?

Richard Straube: As we noted, we don't yet have clarity on this, as it will require additional in-depth investigation of the data. However, there's a number of potential contributing factors, including the smaller size of the Phase 2 trial, possibility of having a mix of responding and nonresponding subpopulations in the Phase 3 data, and the potential variability in the placebo population. We will be exploring these parameters, as well as other aspects of patient demographics, as well as the outcome from the other secondary endpoints in the trial, over the next few months, and identify if there is a potential way forward for this program after consultation with both the FDA and/or the EMEA.

Jonathan Guarino: Thanks, Rick. Our next question: In the Phase 2 study and your preclinical data, you had seen significant anti-infective signal. Do you see that in your Phase 3 data? And what do you think it says about the mechanism of your compound?

Christopher Schaber: Oreola, would you take this one, please?

Oreola Donini: Sure. Thank you. In the Phase 2 study, we did see a reduction in the rate of infection, most prominently in the 6-mg-per-kg group but also in the 1.5-mg-per-kg group. In the Phase 3 study, where we used the more anti-inflammatory 1.5-mg-per-kg dose, we did not see a strong anti-infective signal. This is consistent with the underlying mechanism of dusquetide, where the anti-infective and anti-inflammatory signals peak at different dose levels. Nonetheless, we had been hopeful that a signal would be observed, and we were disappointed by the absence.

As you point out, however, the preclinical data and our ongoing studies continue to support the anti-infective activity of dusquetide.

Christopher Schaber: Thank you.

Jonathan Guarino: Thanks, Oreola. The next question: Given the interim analysis you conducted which advised enrolling an additional 70 subjects, it is surprising that the patient numbers were still not sufficient to see a statistically significant response. What do you think caused this?

Christopher Schaber: Oreola, would you mind taking this one as well?

Oreola Donini: Sure. Yes, we were surprised as well. It is well known that variability can be high in this patient population with respect to oral mucositis. And in comparing the groups, we had to use what's known as a comparison of distribution, and that statistical method is also more susceptible to variability. While we are still investing this, our initial thoughts are that the variability changed between the first approximately 100 subjects and the final 160 subjects in the trial. We are still investigating if this is the case, and if it is, to what extent it may have occurred.

Christopher Schaber: Thanks, Oreola.

Jonathan Guarino: Our next question: If there is a potential path forward, I don't think investors would have any confidence in the program being successful the second time around, so how would you be able to fund it? I would think strategic partnership or sale of the asset is the only logical path.

Christopher Schaber: I'll take this one, Jonathan. Obviously it's difficult to say anything further at this time without seeing all the data from the study. But you are correct that we will need to look at all possible strategic options moving forward, which may include, for example, co-development partnership, merger acquisition. However, please keep in mind that dusquetide is a platform technology and there is applicability in other indications, which we will also evaluate or are currently evaluating, such as infectious diseases.

Jonathan Guarino: Great, thanks, Chris. Our next question: Where does the company go from here?

Christopher Schaber: Where does the company go from here? Forward. While this outcome is extremely disappointing to us all, we can't forget that we announced a positive Phase 3 study with SGX301 in CTCL earlier this year that has generated compelling data throughout the extended treatment cycles. We are going to advance this program towards FDA approval and commercialization in the United States. We also have other development pipeline programs that we're actively pursuing with government funding. So again, we continue to go forward. I would also urge everyone to revisit the pipeline and development milestones in our corporate presentation. There still remains a number of important value drivers throughout 2021.

Jonathan Guarino: Great, thanks, Chris. Our next question: Can you briefly speak about your COVID-19 subunit vaccine CiVax? This is a compelling vaccine to me. Can you discuss how it compares with the mRNA vaccines from Pfizer and Moderna?

Christopher Schaber: Oreola, you're on point for this one. Would you mind responding, please?

Oreola Donini: Thank you, Chris. So our CiVax vaccine, which we are developing in collaboration with the University of Hawaii, is a subunit protein vaccine. This means that what we're doing is administering the spike protein antigen directly, ensuring everyone gets the same dose. There is no RNA or DNA involved, and no other viral vectors.

Instead, to alert the immune system to the need to develop antibodies to the spike protein, we are using an adjuvant known as CoVaccine HT. This adjuvant has been shown to produce both neutralizing antibodies, the important TH1 response in the immune system, and to stimulate some mediated immunity. Our studies to date with both our prototypes and full-length spike protein have demonstrated very good responses.

Importantly, subunit vaccines are a well-established technology in immunology and are considered one of the gold standards in vaccine safety. In addition, our work with this product has demonstrated the ability to not only produce the material rapidly and cost-efficiently, but to also formulate it so that it is heat-stable. This means it can be distributed at ambient temperatures with no need for refrigerators or freezers. We also know this technology is compatible with --

including multiple antigens, in case we need to combine it with other forms of the spike protein or other vaccines in general in the future.

Compared to the RNA and adenovirus vaccines, our product is planned to have a simpler distribution and simpler supply chain, as well as ideally providing a cost-effective solution for worldwide application. Of note, though, we do also target the spike protein antigen, which both the Moderna and Pfizer vaccines have demonstrated to be an efficacious approach.

Christopher Schaber: Thanks, Oreola.

Jonathan Guarino: Great, thanks. This is our final question, and then we'll hand it back over to the operator. Can you discuss the market potential for SGX301 in the U.S. for CTCL?

Christopher Schaber: Dan, would you mind taking this one, please?

Daniel Ring: Sure. So, no, we're excited about the opportunity for SGX301. We think it's a significant near-term commercial opportunity addressing a clear unmet need. The target population in the U.S. is about 25,000 patients, and we're estimating the peak sales in the U.S. of roughly $75 million per year, with the potential for higher sales with lifecycle management opportunities. And just to put that into context, similar competitive products that are -- have what we think are inferior product profiles, or are indicated in second line, have similar sales figures.

So again, we're excited by SGX301. We think it has a significant commercial potential opportunity in the U.S. and we'll be sharing a little bit more about the commercialization plans in the new year.

Christopher Schaber: Thanks, Dan.

Jonathan Guarino: With that, we'll hand it over, back over to the operator.

Operator: As there are no further questions at this time, I would like to turn the call back to Dr. Schaber for any closing remarks.

Christopher Schaber: Thank you, operator. As I noted, we'll continue to keep you all updated. Thank you for taking time to participate this morning. I hope everyone has a happy and healthy holiday season.