Soligenix Investor Relations

Soligenix Completes Enrollment in its Pivotal Phase 3 Clinical Trial of SGX942 for the Treatment of Oral Mucositis

Final top-line results expected Q4 2020

PRINCETON, N.J., June 24, 2020 /PRNewswire/ -- Soligenix, Inc. (Nasdaq: SNGX) (Soligenix or the Company), a late-stage biopharmaceutical company focused on developing and commercializing products to treat rare diseases where there is an unmet medical need, announced today it has completed patient enrollment in its Phase 3 DOM-INNATE ("Dusquetide treatment in Oral Mucositis – by modulating INNATE Immunity") study for SGX942 (dusquetide) in the treatment of oral mucositis (OM) in head and neck cancer (HNC) patients. The study successfully enrolled 268 subjects, following positive interim analysis, which included a prospectively defined, unblinded assessment of the study's primary efficacy endpoint by an independent Data Monitoring Committee (DMC). With enrollment completed, top-line results are expected in the fourth quarter of 2020.

SGX942 is a novel, first-in-class, Innate Defense Regulator (IDR) which both modulates inflammation and enhances anti-infective and tissue-healing pathways of the innate immune system. Study enrollment was temporarily extended as Soligenix assessed the potential impact of COVID-19 on the study (e.g., patient treatment compliance and completion of necessary assessments). With extra efforts by participating patients, physicians and clinical staff, the Company can now successfully report that the negative impact of the pandemic on the overall study was much less than initially anticipated. The study remains on-track to provide top-line results before the end of 2020.

"We are pleased to have completed enrollment and look forward to the top-line results in the fourth quarter, particularly in light of the DMC recommendation at the interim analysis which observed a beneficial drug effect," stated Christopher J. Schaber, PhD, President and Chief Executive Officer of Soligenix. "We continue to positively position this fast-tracked program for approval. With approximately $8 million in cash as of the end of the first quarter, not including our non-dilutive government funding, along with the at-the-market sales issuance agreement with B. Riley FBR, Inc. to judiciously supplement our cash runway as needed, we anticipate having sufficient capital to achieve multiple inflection points across our rare disease pipeline, including top-line results in the DOM-INNATE study. As there is no FDA approved drug for the treatment of oral mucositis in head and neck cancer or other solid tumor settings, we believe SGX942 has the potential to be the first approved therapy to address this unmet medical need and dramatically improve the lives of patients undergoing chemoradiation therapy (CRT)."

"SGX942 has the potential to have a significant impact on the lives of patients undergoing CRT for squamous cell carcinoma of the oral cavity and oropharynx," stated Richard Straube, MD, Senior Vice President and Chief Medical Officer of Soligenix. "We would like to thank the DMC members, our esteemed medical advisory board and our dedicated clinical investigators for their efforts in the design and conduct of this important clinical trial, as well as all the subjects that are participating in the trial. Our focus now is to complete the treatments for all subjects in both the US and Europe and to lock the study database, facilitating top-line results in the fourth quarter of 2020."

Based on the positive results demonstrated in the Phase 2 study of SGX942, the Phase 3 trial is a highly powered, double-blind, randomized, placebo-controlled, multicenter and multinational trial. The primary endpoint for the study is the median duration of severe oral mucositis, assessed by oral examination at each treatment visit and then through six weeks following completion of CRT. Oral mucositis is evaluated using the WHO (World Health Organization) Grading system. Other secondary measures, including incidence of severe oral mucositis, incidence and duration of ulcerative oral mucositis, and incidence of infection will also be assessed at topline or during the 12-month follow-up.

A prospectively defined interim analysis was conducted in August 2019 by an independent DMC and was used to verify the underlying assumptions defining the required sample size of the study to maintain its rigorous 90% statistical power. The DMC identified a beneficial SGX942 effect and accordingly adjusted the study sample size to approximately 260. The DMC did not identify any safety concerns. The interim recommendation is described in the August 2019 press release here.

About Oral Mucositis

Mucositis is the clinical term for damage done to the mucosa by anticancer therapies. It can occur in any mucosal region, but is most commonly associated with the mouth, followed by the small intestine. It is estimated, based upon review of historic published studies and reports and an interpolation of data on the incidence of mucositis, that mucositis affects approximately 500,000 people in the US per year and occurs in 40% of patients receiving chemotherapy. Mucositis can be severely debilitating and can lead to infection, sepsis, the need for parenteral nutrition and narcotic analgesia. The gastrointestinal damage causes severe

diarrhea. These symptoms can limit the doses and duration of cancer treatment, leading to sub-optimal treatment outcomes.

The mechanisms of mucositis have been extensively studied and have been recently linked to the interaction of chemotherapy and/or radiation therapy with the innate defense system. Bacterial infection of the ulcerative lesions is now regarded as a secondary consequence of dysregulated local inflammation triggered by therapy-induced cell death, rather than as the primary cause of the lesions.

It is estimated, based upon review of historic published studies and reports and an interpolation of data on the incidence of oral mucositis, that oral mucositis in HNC is a subpopulation of approximately 90,000 patients in the US, with a comparable number in Europe. Oral mucositis almost always occurs in patients with HNC treated with CRT and is severe, causing inability to eat and/or drink, in >80% of patients. It is common (40-100% incidence) in patients undergoing high dose chemotherapy and hematopoietic cell transplantation, where the incidence and severity of oral mucositis depends greatly on the nature of the conditioning regimen used for myeloablation.

In the pediatric population, head and neck cancer is a rarer occurrence and is caused by different underlying pathologies. The major types of HNC in children are lymphoma, sarcomas (including rhabdomyosarcomas), and neuroblastoma rather than squamous cell carcinoma, the major type of adult HNC cancers. Hematopoietic stem cell transplantation (HSCT), especially allogeneic transplantation with higher risk of oral mucositis, is more frequently used in the pediatric population than in adults when treating a number of primary tumor types, as seen in leukemia and lymphoma. Both treatment of HNC and HSCT are associated with high risk of oral mucositis in the pediatric population.

Oral mucositis remains an area of unmet medical need where there are currently no approved drug therapies in the context of any solid tissue tumors.

About the Phase 3 DOM-INNATE Study

This multinational, placebo-controlled, randomized study is targeted to enroll approximately 260 subjects with squamous cell carcinoma of the oral cavity and oropharynx, scheduled to receive a minimum total cumulative radiation dose of 55 Gy fractionated as 2.0-2.2 Gy per day with concomitant cisplatin chemotherapy given as a dose of 80-100 mg/m^2 every third week. Subjects are randomized to receive either 1.5 mg/kg SGX942 or placebo given twice a week during and for two weeks following completion of CRT. The primary endpoint for the study is the median duration of severe oral mucositis, assessed by oral examination at each treatment visit and then through six weeks following completion of CRT. Oral mucositis is evaluated using the WHO (World Health Organization) Grading system. Severe oral mucositis is defined as a WHO Grade of ≥3. Subjects are to be followed for an additional 12 months after the completion of treatment. Soligenix has been working with leading oncology centers internationally, a number of which participated in the Phase 2 study.

About Dusquetide

Dusquetide (the active ingredient in SGX942) is an innate defense regulator (IDR), a new class of short, synthetic peptides. It has a novel mechanism of action whereby it modulates the body's reaction to both injury and infection towards an anti-inflammatory, anti-infective and tissue healing response. IDRs have no direct antibiotic activity but, by modulating the host's innate immune system responses, increase survival after infections caused by a broad range of bacterial Gram-negative and Gram-positive pathogens. It also accelerates resolution of tissue damage following exposure to a variety of agents including bacterial pathogens, trauma and chemo- and/or radiation therapy. Preclinical efficacy and safety has been demonstrated in numerous animal disease models including mucositis, colitis, macrophage activation syndrome (MAS) as well as bacterial infections, including melioidosis.

SGX942 has demonstrated safety in a Phase 1 clinical study in 84 healthy human volunteers. Positive efficacy results were demonstrated in an exploratory Phase 2 clinical study in 111 patients with oral mucositis due to CRT for HNC. Soligenix is working with leading oncology centers in the US and Europe to advance SGX942 in oral mucositis with the conduct of a pivotal Phase 3 clinical trial referred to as the "DOM–INNATE" study (Dusquetide treatment in Oral Mucositis – by modulating INNATE immunity).

SGX942 has received Fast Track Designation from the FDA for the treatment of oral mucositis as a result of radiation and/or chemotherapy treatment in HNC patients, as well as Promising Innovative Medicine designation in the United Kingdom by the Medicines and Healthcare Products Regulatory Agency for the treatment of severe oral mucositis in HNC patients receiving CRT. In addition, products containing the same active ingredient, dusquetide, have been granted Fast Track Designation as an adjunctive therapy with other antibacterial drugs, for the treatment of melioidosis and Orphan Drug Designations in the treatment of MAS and the treatment of acute radiation syndrome.

Soligenix has a strong intellectual property position in the IDR technology platform, including composition of matter for dusquetide and related analogs. Dusquetide was developed pursuant to discoveries made by Professors B. Brett Finlay, PhD and Robert Hancock, PhD of the University of British Columbia, Canada. Soligenix has received partial funding from NIH for its oral mucositis clinical studies. The Phase 2 study was supported with a Phase I SBIR grant (#R43DE024032) award, with the Phase 3 study being supported by a Phase II SBIR grant (#R44DE024032) award.

In addition, a high level review of the IDR technology platform is available here.

About Soligenix, Inc.

Soligenix is a late-stage biopharmaceutical company focused on developing and commercializing products to treat rare diseases where there is an unmet medical need. Our Specialized BioTherapeutics business segment is developing SGX301 as a novel photodynamic therapy utilizing safe visible light for the treatment of cutaneous T-cell lymphoma, our first-in-class innate defense regulator (IDR) technology, dusquetide (SGX942) for the treatment of oral mucositis in head and neck cancer, and proprietary formulations of oral beclomethasone 17,21-dipropionate (BDP) for the prevention/treatment of gastrointestinal (GI) disorders characterized by severe inflammation including pediatric Crohn's disease (SGX203) and acute radiation enteritis (SGX201).

Our Public Health Solutions business segment includes active development programs for RiVax®, our ricin toxin vaccine candidate, OrbeShield®, our GI acute radiation syndrome therapeutic candidate and SGX943, our therapeutic candidate for antibiotic resistant and emerging infectious disease. The development of our vaccine programs incorporates the use of our proprietary heat stabilization platform technology, known as ThermoVax®. To date, this business segment has been supported with government grant and contract funding from the National Institute of Allergy and Infectious Diseases (NIAID), the Defense Threat Reduction Agents (DTRA) and the Biomedical Advanced Research and Development Authority (BARDA).

For further information regarding Soligenix, Inc., please visit the Company's website at www.soligenix.com.

This press release may contain forward-looking statements that reflect Soligenix, Inc.'s current expectations about its future results, performance, prospects and opportunities, including but not limited to, potential market sizes, patient populations and clinical trial enrollment. Statements that are not historical facts, such as "anticipates," "estimates," "believes," "hopes," "intends," "plans," "expects," "goal," "may," "suggest," "will," "potential," or similar expressions, are forward-looking statements. These statements are subject to a number of risks, uncertainties and other factors that could cause actual events or results in future periods to differ materially from what is expressed in, or implied by, these statements. Soligenix cannot assure you that it will be able to successfully develop, achieve regulatory approval for or commercialize products based on its technologies, particularly in light of the significant uncertainty inherent in developing therapeutics and vaccines against bioterror threats, conducting preclinical and clinical trials of therapeutics and vaccines, obtaining regulatory approvals and manufacturing therapeutics and vaccines, that product development and commercialization efforts will not be reduced or discontinued due to difficulties or delays in clinical trials or due to lack of progress or positive results from research and development efforts, that it will be able to successfully obtain any further funding to support product development and commercialization efforts, including grants and awards, maintain its existing grants which are subject to performance requirements, enter into any biodefense procurement contracts with the US Government or other countries, that it will be able to compete with larger and better financed competitors in the biotechnology industry, that changes in health care practice, third party reimbursement limitations and Federal and/or state health care reform initiatives will not negatively affect its business, or that the US Congress may not pass any legislation that would provide additional funding for the Project BioShield program. In addition, there can be no assurance as to timing or success of the Phase 3 clinical trial of SGX942 (dusquetide) as a treatment for oral mucositis in patients with head and neck cancer receiving chemoradiation therapy (including the outcome of the interim analysis) or the Phase 3 clinical trial of SGX301 (synthetic hypericin) for the treatment of cutaneous T-cell lymphoma. Further, there can be no assurance that RiVax® will qualify for a biodefense Priority Review Voucher (PRV) or that the prior sales of PRVs will be indicative of any potential sales price for a PRV for RiVax®. These and other risk factors are described from time to time in filings with the Securities and Exchange Commission, including, but not limited to, Soligenix's reports on Forms 10-Q and 10-K. Unless required by law, Soligenix assumes no obligation to update or revise any forward-looking statements as a result of new information or future events.

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