Soligenix Investor Relations

Soligenix Announces Japanese Patent Allowance for Use of Dusquetide in Oral Mucositis

Specific therapeutic use claims in oral mucositis adds to existing composition of matter claims

PRINCETON, N.J., Feb. 3, 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 that the Japanese Patent Office has granted the patent titled "Novel Peptides and Analogs for Use in the Treatment of Oral Mucositis." This allowance builds on similar intellectual property in the United States (US), New Zealand, Australia and Singapore and patent applications pending in other jurisdictions worldwide. The new claims cover therapeutic use of dusquetide (active ingredient in SGX942) and related innate defense regulator (IDR) analogs, and add to composition of matter claims for dusquetide and related analogs that have been granted in the US and worldwide. Dusquetide previously demonstrated positive results in a Phase 2 oral mucositis clinical trial and a pivotal Phase 3 study is ongoing, with a positive interim analysis completed in August 2019 and final topline results expected in Q2 2020.

Based on the positive and previously published Phase 2 results, the pivotal Phase 3 clinical trial is a highly powered, double-blind, randomized, placebo-controlled, multinational trial. The study, called DOM-INNATE (Dusquetide treatment in Oral Mucositis – by modulating INNATE immunity), is enrolling approximately 260 subjects with head and neck cancer (HNC) undergoing standard chemoradiation therapy (CRT) and who are therefore at high risk of developing severe oral mucositis. Enrollment is ongoing in the US and across Europe with enrollment completion expected in Q1 2020 and final topline primary endpoint results anticipated in Q2 2020.

"Soligenix continues to pursue broad patent coverage for its IDR technology, including dusquetide, first with composition of matter claims followed by therapeutic use claims in oral mucositis," stated Christopher J. Schaber, PhD, President and Chief Executive Officer of Soligenix. "Having successfully pursued composition of matter claims in jurisdictions worldwide, we continue to pursue therapeutic use claims like those issued in the US previously and in Japan with this most recent patent. The therapeutic use claims are expected to be generally valid until 2034, which provides significant patent protection and life to dusquetide and our other IDRs. This allowance is timely as we continue to have discussions with potential strategic partners and pursue all options to advance our pipeline and plan for commercial activities."

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 Dusquetide

Dusquetide (the active ingredient in SGX942) is an 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 and tolerability in a Phase 1 clinical study in 84 healthy human volunteers. Positive efficacy results were demonstrated in an exploratory Phase 2 clinical study (Study IDR-OM-01) in 111 patients with oral mucositis due to CRT for HNC, including potential long-term ancillary benefits. 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 (Study IDR-OM-02) referred to as the "DOM–INNATE" study (Dusquetide treatment in Oral Mucositis – by modulating INNATE immunity). The multinational, placebo-controlled, randomized Phase 3 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. An interim analysis for this study has been completed and identified a promising signal.

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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