Soligenix Investor Relations

# Soligenix Achieves Significant Enrollment Milestone for its Pivotal Phase 3 Clinical Trial of SGX942 in the Treatment of Oral Mucositis in Head and Neck Cancer

## Enrollment Sufficient to Support the Interim Efficacy Analysis

**Princeton, NJ – April 18, 2019 –** 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 it has reached a significant milestone in the Phase 3 clinical study (the "DOM-INNATE" study) for SGX942 (dusquetide) in the treatment of oral mucositis in patients with head and neck cancer (HNC). Patient enrollment is sufficient to support the planned interim efficacy analysis by the independent Data Monitoring Committee (DMC).

In accordance with the clinical protocol, approximately 90 subjects have been enrolled into the study as required for the planned interim efficacy analysis. In the coming weeks, subjects that are currently enrolled will have their protocol assessments completed at participating study centers, including the primary endpoint assessment. The blinded data will be verified for accuracy by the Soligenix clinical team and the data set will be provided to an independent statistical analysis center that interacts directly with the DMC for the interim efficacy analysis. All participating subjects and study centers, as well as the Company, will remain blinded at all times.

The DMC is a group, independent of the Company, charged with safety oversight of the clinical study as well as the conduct of one, pre-specified interim efficacy analysis. One DMC member is a statistician, with the remainder consisting of clinicians knowledgeable and experienced in the disease indication being studied. The DMC convenes at pre-determined intervals (in accordance with a pre-defined charter) to review unblinded safety and efficacy data. The DMC has the power to recommend continuation or termination of the study based on the evaluation of these data. Specific recommendations include stopping the study for overwhelming efficacy, stopping the study for serious safety concern, stopping the study for futility, continuing enrollment in the study at the pre-specified sample size of approximately 190 subjects, or re-estimating sample size up or down to maintain the study's statistical power.

"Completing the required enrollment to support the DMC interim analysis is a significant milestone for the SGX942 program," stated Christopher J. Schaber, PhD, President and Chief Executive Officer of Soligenix. "We must now wait for the last enrolled subject to reach the study's primary endpoint measure, which will occur up to 16 weeks after entering the study. During this time, we will continue enrolling patients from the US and Europe into the trial, while looking forward to receiving the formal DMC recommendation in the September timeframe."

Soligenix has been working with leading oncology centers internationally, a number of which participated in the Phase 2 study, to advance this Phase 3 clinical trial referred to as the "DOM–INNATE" study (Dusquetide treatment in Oral Mucositis – by modulating INNATE immunity).

Based on the positive and previously published Phase 2 results (Study IDR-OM-01), the pivotal Phase 3 clinical trial (Study IDR-OM-02) is designed to be a highly powered, double-blind, randomized, placebo-controlled, multinational trial that seeks to enroll approximately 190 subjects with squamous cell carcinoma of the oral cavity and oropharynx who are 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/m2 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 Organisation) Grading system. Severe oral mucositis is defined as a WHO Grade of ≥3. Subjects are followed for an additional 12 months after the completion of treatment.

Patient recruitment is anticipated to be completed 2019 with top-line results available in the first half of 2020, pending the outcome of the interim analysis.

### 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.

Oral mucositis in HNC remains an area of unmet medical need where there are currently no approved drug therapies.

**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 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.

Key nonclinical and clinical findings from the dusquetide program can be found in the following publications:

- "Targeting Innate Immunity to Treat Disease: Potential Therapeutic Applications" at https://www.drugtargetreview.com/article/37410/targeting-innate-immunity/.
- "A novel approach for emerging and antibiotic resistant infections: Innate defense regulators as an agnostic therapy" at http://dx.doi.org/10.1016/j.jbiotec.2016.03.032.
- "Dusquetide: A Novel Innate Defense Regulator Demonstrating a Significant and Consistent Reduction in the Duration of Oral Mucositis in Preclinical Data and a Randomized, Placebo-Controlled Phase 2 Clinical Study" at http://dx.doi.org/10.1016/j.jbiotec.2016.10.010.
- "Dusquetide: Reduction in Oral Mucositis associated with Enduring Ancillary Benefits in Tumor Resolution and Decreased Mortality in Head and Neck Cancer Patients" at https://doi.org/10.1016/j.btre.2017.05.002.

In addition, a high level review of the dusquetide 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 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 Vaccines/BioDefense business segment includes active development programs for RiVax®, our ricin toxin vaccine 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) 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 U.S. 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 U.S. 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 planned interim analysis) or the Phase 3 clinical trial of SGX301 (synthetic hypericin) for the treatment of cutaneous T-cell lymphoma. There also can be no assurance as to timing or success of the preclinical/clinical trials of RiVax®, that RiVax® will be approved for the PRV program or the amount for which a PRV for RiVax® can be sold. 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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