

## **Soligenix Receives Positive Scientific Advice from the European Medicines Agency for SGX942 in the Treatment of Oral Mucositis in Head and Neck Cancer Patients**

### **Follows UK Health Ministry's Granting SGX942 Promising Innovative Medicine Designation in the Treatment of Oral Mucositis**

**Princeton, NJ, January 5, 2017** – Soligenix, Inc. (Nasdaq: SNGX), 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 company had received positive Scientific Advice from the European Medicines Agency (EMA) for the development of SGX942 (dusquetide) as a treatment for oral mucositis in patients with head and neck cancer receiving chemoradiation therapy. The Scientific Advice from the EMA indicates that a single, double-blind, placebo-controlled, multinational, Phase 3 pivotal study (IDR-OM-02), if successful, in conjunction with the Phase 2 dose-ranging study IDR-OM-01, is generally considered sufficient to support a marketing authorization application (MAA) to the EMA for potential licensure in Europe. The advice also provides several constructive suggestions to strengthen the study design and data collection that will be integrated into the final protocol.

“We are pleased that the EMA agrees that the proposed Phase 3 clinical study, if positive, is sufficient to establish the efficacy of SGX942 and support an MAA filing,” stated Christopher J. Schaber, PhD, President and Chief Executive Officer of Soligenix. “We believe that this positive Scientific Advice outcome represents a significant step forward in our oral mucositis development program and has the potential to accelerate the registration timetable in Europe. We look forward to incorporating the EMA's protocol suggestions with those from the US Food and Drug Administration. Given the current timeline, we anticipate initiating this pivotal Phase 3 clinical trial in the first half of 2017.”

#### **About Scientific Advice**

Scientific Advice is a procedure offered by the EMA to stakeholders for clarification of questions arising during development of medicinal products. The scope of Scientific Advice is limited to scientific issues, i.e. to quality, non-clinical, and clinical aspects of the concerned medicinal product not yet unequivocally covered by published scientific guidelines. Scientific Advice focuses on development strategies rather than pre-evaluation of data to support an MAA. Scientific Advice is legally non-binding and is based on the current scientific knowledge which may be subject to future changes.

#### **About SGX942**

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 in that it modulates the body's reaction to both injury and infection towards an anti-inflammatory and an anti-infective response. IDRs have no direct antibiotic activity but, by modulating the host's innate immune system responses, increase survival after infections with 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, melioidosis, macrophage activation syndrome (MAS) and other bacterial infections. Some of these preclinical findings have been published in an article entitled “A novel approach for emerging and antibiotic resistant infections: Innate defense regulators as an agnostic therapy” and are available at the following link: <http://dx.doi.org/10.1016/j.jbiotec.2016.03.032>.

SGX942 has demonstrated safety in a Phase 1 clinical study in 84 healthy human volunteers. Recently, SGX942 has demonstrated preliminary efficacy and safety in an exploratory Phase 2 clinical study in 111 patients with oral mucositis due to chemoradiation (CRT) therapy for head and neck cancer. Consistent with preclinical findings, SGX942 at a dose of 1.5 mg/kg demonstrated positive improvements in decreasing the duration of severe oral mucositis by 50% overall compared to the placebo group, from 18 days to 9 days ( $p=0.099$ ). In patients at highest risk of oral mucositis (e.g., those exposed to the most aggressive concomitant chemotherapy), the reduction in the duration of severe oral mucositis was even more significant at 67% when treated with SGX942 1.5 mg/kg, from 30 days to 10 days ( $p=0.04$ ). The p-values meet the prospectively defined statistical threshold of  $p<0.1$  in the study protocol. Additional observations included an improved tumor response to CRT therapy at the one month follow-up visit, as well as decreases in infection rate. The study results are reviewed in “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 2a Clinical Study” published online in the Journal of Biotechnology and are available at the following link: <http://dx.doi.org/10.1016/j.jbiotec.2016.10.010>.

Long-term (12 month) follow-up data further indicated the safety and tolerability of SGX942 treatment, with a trend towards reduced mortality and increased tumor resolution in the 1.5 mg/kg SGX942 treatment group compared to the placebo group. Opioid pain medication use was also seen to decrease over the course of CRT in the 1.5 mg/kg SGX942 treatment group at the point of highest oral mucositis risk, while it increased in the placebo group.

The Phase 2 oral mucositis clinical study was partially funded with a grant from the National Institute of Dental and Craniofacial Research Small Business Innovation Research grant #1R43 DE024032-01 (Soligenix, Inc).

Dusquetide and related analogs have a strong intellectual property position, including composition of matter. Dusquetide was developed pursuant to discoveries made by Professors B. Brett Finlay, PhD and Robert Hancock, PhD of the University of British Columbia, Canada.

Drug products containing dusquetide have also received Fast Track Designations from the FDA for the treatment of oral mucositis as a result of radiation and/or chemotherapy treatment in head and neck cancer patients, and as an adjunctive therapy with other antibacterial drugs, for the treatment of melioidosis. Orphan Drug Designations for use of dusquetide in the treatment of MAS as well as for the treatment of acute radiation syndrome have also been granted. In addition, dusquetide has been granted Promising Innovative Medicine (PIM) designation in the United Kingdom (UK) by the Medicines and Healthcare Products Regulatory Agency (MHRA) for the treatment of severe oral mucositis in head and neck cancer patients receiving chemoradiation therapy.

### **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 head and neck cancer 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 head and neck cancer treated with chemoradiation therapy 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 head and neck cancer remains an area of unmet medical need where there are currently no approved drug therapies.

### **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, OrbeShield®, our GI acute radiation syndrome therapeutic candidate and SGX943, our melioidosis therapeutic candidate. The development of our vaccine programs incorporates the use of our proprietary heat stabilization platform technology, known as ThermoVax®. Currently, this business segment is 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](http://www.soligenix.com).

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*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. 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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