Soligenix Investor Relations

FDA Grants Soligenix Orphan Drug Designation for Dusquetide for Treatment of Macrophage Activation Syndrome

Princeton, NJ – August 18, 2016 – Soligenix, Inc. (OTCQB: 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 Office of Orphan Products Development of the US Food and Drug Administration (FDA) has granted orphan drug designation to the active ingredient dusquetide for treatment of macrophage activation syndrome (MAS). Dusquetide has previously received orphan drug designation for the treatment of acute radiation syndrome (ARS). Dusquetide is an innate defense regulator (IDR), a new class of short, synthetic peptides that accelerates bacterial clearance and resolution of tissue damage while modulating inflammation following exposure to a variety of agents including bacterial pathogens, trauma, radiation and/or chemotherapy.

The US Orphan Drug Act is intended to assist and encourage companies to develop safe and effective therapies for the treatment of rare diseases and disorders. In addition to providing a seven year term of market exclusivity upon final FDA approval, orphan drug designation also positions Soligenix to be able to leverage a wide range of financial and regulatory benefits, including government grants for conducting clinical trials, waiver of expensive FDA user fees for the potential submission of a New Drug Application, and certain tax credits.

“The FDA’s decision to grant dusquetide orphan drug designation signifies an important step for Soligenix as we continue to expand our biotherapeutics pipeline and the many potential applications of our novel IDR technology,” stated Christopher J. Schaber, PhD, President and Chief Executive Officer of Soligenix. “Dusquetide’s activity in preclinical models has demonstrated the potential to enhance mechanisms of the innate immune system to clear infection and modulate the inflammatory response, the critical attributes of this syndrome. The marketing exclusivity that orphan drug designation imparts adds significantly to the existing intellectual property surrounding dusquetide.”

About Macrophage Activation Syndrome

Macrophage activation syndrome (MAS) is characterized by a highly stimulated but ineffective immune response; however, its pathogenesis is poorly understood. MAS has many similarities with that of the other forms of hemophagocytic lymphohistiocytosis. MAS is a life-threatening complication of rheumatic disease that, for unknown reasons, occurs much more frequently in individuals with systemic juvenile idiopathic arthritis (SJIA). Besides SJIA, systemic lupus erythematosus (SLE), and Kawasaki disease are two other rheumatologic conditions in which MAS appears to occur somewhat more frequently than in other diseases. In adults, based on limited epidemiologic studies, MAS is seen most frequently in association with adult onset Still’s disease, SLE, and various vasculitic syndromes. MAS is characterized by pancytopenia, liver insufficiency, coagulopathy and neurologic symptoms and is thought to be caused by the activation and uncontrolled proliferation of T lymphocytes and well-differentiated macrophages, leading to widespread hemophagocytosis and cytokine overproduction. Despite treatment, fatalities still occur with a mortality rate in the range of 10-20%.

About Dusquetide

Dusquetide is an innate defense regulator (IDR), a new class of short, synthetic peptide. 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, MAS and other bacterial infections. In a published mouse model of MAS, dusquetide was shown to reduce the pancytopenia, reduced IL-12 responses and improve body weight maintenance.

SGX942 (the drug product containing dusquetide) 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.

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 US Food and Drug Administration (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.

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 up to $58 million in 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 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. 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.
