Soligenix Investor Relations

Soligenix Identifies Biomarkers for Ricin Toxin Vaccine Testing under the FDA Animal Rule

Princeton, NJ – December 21, 2017 – 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 biomarkers for ricin toxin vaccine (RiVax®) testing have been successfully identified, facilitating potential approval under the FDA "Animal Rule". The FDA Animal Rule is applied to products where testing in clinical trials would be unethical. In the case of a ricin toxin vaccine, clinical efficacy testing of the vaccine is unethical since exposing unvaccinated humans (i.e., placebo-control group) to ricin toxin would be fatal. The Animal Rule combines safety studies in humans and efficacy testing in animals, typically non-human primates (NHPs), to facilitate approval and is generally associated with the approval of medical countermeasures for biodefense purposes. Key to the application of the Animal Rule is the requirement to establish a correlation between the response observed in clinical trials in healthy volunteers with the response demonstrated in animal efficacy studies. Identification of a biomarker to facilitate demonstrating the correlation between animal and human studies is a significant accomplishment in the RiVax® development program.

RiVax® is a ricin toxin vaccine under development by Soligenix, and originally invented at the University of Texas Southwestern. Further formulated by Soligenix to have enhanced thermostability using the Company's ThermoVax® platform, the RiVax® vaccine has demonstrated up to 100% protection in mice and NHPs subsequently exposed to lethal doses of ricin toxin either systemically or by aerosol.

Mechanistic studies, conducted in collaboration with Dr. Nicholas Mantis, Associate Professor, Department of Biomedical Sciences, School of Public Health, New York, have demonstrated the unique expression profile of ricin antibodies, which matures to combat ricin toxin as time passes. Key biomarkers of these profiles have been identified and include in order of increasing sensitivity:

1) antibody titers (the ability of antibodies to recognize ricin),

2) neutralizing antibody activity (the ability of antibodies to prevent ricin toxicity), and

3) SyH7 antibody epitope competition profile (the ability of antibodies to bind to the SyH7 binding site on the ricin protein, a key binding site that neutralizes the toxicity of the ricin protein).

These biomarkers are consistent across mice, NHPs and humans, supporting the application of the Animal Rule.

In addition to being protective and thermostable, RiVax® has demonstrated that a reduced number of vaccinations may be required to establish protection, potentially utilizing only two doses instead of three. Further efficacy studies in NHPs evaluating potential dosing regimens are anticipated in 2018, in addition to continued human safety testing evaluating the thermostable RiVax® formulation.

Key biomarker results were recently presented at the Chemical and Biological Defense Science and Technology conference and are available here.

RiVax® studies are supported by contract # HHSN272201400039C from the National Institute of Allergy and Infectious Diseases (NIAID). RiVax® has received Orphan Drug designation from the FDA and, upon approval, has the potential to qualify for a biodefense Priority Review Voucher.

About Ricin Toxin

Ricin toxin is a lethal plant-derived toxin and potential biological weapon because of its stability and high potency, and the fact it is readily extracted from by-products of castor oil production. Ricin comes in many forms including powder, mist or pellet. Ricin can also be dissolved in water and other liquids. The US Centers for Disease Control and Prevention estimates that the lethal dose in humans is about the size of a grain of salt. Ricin toxin illness causes tissue necrosis and general organ failure leading to death within several days of exposure. Ricin is especially toxic when inhaled. Ricin works by entering cells of the body and preventing the cells from making the proteins it needs. Without the proteins, cells die, which is eventually harmful to the entire body.

There are currently no effective treatments for ricin poisoning. The successful development of an effective vaccine against ricin toxin may act as a deterrent against the actual use of ricin as a biological weapon and could be used to vaccinate military personnel and civilian emergency responders at high risk of potential exposure in the event of a biological attack.

About RiVax®

RiVax® is Soligenix's proprietary heat stable recombinant subunit vaccine developed to protect against exposure to ricin toxin. With RiVax®, Soligenix is a world leader in the area of ricin toxin vaccine research.

RiVax® contains a genetically altered version of a Ricin Toxin A (RTA) chain containing two mutations that inactivate the toxicity of the ricin molecule. A Phase 1A clinical trial was conducted with a formulation of RiVax® that did not contain an adjuvant. This trial revealed dose dependent seroconversion as well as lack of toxicity of the molecule when administered intramuscularly to human volunteers. The adjuvant-free formulation of RiVax® induced toxin neutralizing antibodies that lasted up to 127 days after the third vaccination in several individuals.

To increase the longevity and magnitude of toxin neutralizing antibodies, RiVax® was subsequently formulated with an adjuvant of aluminum salts (known colloquially as Alum) for a Phase 1B clinical trial. Alum is an adjuvant that is used in many human vaccines, including most vaccines used in infants. The results of the Phase 1B study indicated that Alum-adjuvanted RiVax® was safe and well tolerated, and induced greater ricin neutralizing antibody levels in humans than adjuvant-free RiVax®. In preclinical animal studies, the Alum formulation of RiVax® also induced higher titers and longer-lasting antibodies than the adjuvant-free vaccine. Vaccination with the thermostabilized Alum-adjuvanted RiVax® formulation in a large animal model provided 100% protection ($p<0.0001$) against acute exposure to aerosolized ricin, the most lethal route of exposure for ricin. The protected animals also had no signs of gross lung damage, a serious and enduring ramification with long-term consequences for survivors of ricin exposure. These results are described in a publication available here.

Heat stabilization of RiVax® is achieved with the Company's proprietary ThermoVax® technology, designed to eliminate the cold-chain production, distribution and storage logistics required for most vaccines. The technology utilizes precise lyophilization of protein immunogens with conventional aluminum adjuvants in combination with secondary adjuvants for rapid onset of protective immunity with the fewest number of vaccinations. By employing ThermoVax® during the final formulation of RiVax®, the vaccine has demonstrated enhanced stability and the ability to withstand temperatures at least as high as 40 degrees Celsius (104 degrees Fahrenheit) for up to one year.

The development of RiVax® has been sponsored through a series of grants from both NIAID, and the FDA and ongoing development is sponsored by NIAID contract # HHSN272201400039C. RiVax® potentially would be added to the Strategic National Stockpile and dispensed in the event of a terrorist attack. RiVax® has received orphan drug designation from the FDA.

As a new chemical entity, an FDA approved RiVax® vaccine has the potential to qualify for a biodefense Priority Review Voucher (PRV), which allows the holder accelerated review of a drug application. Approved under the 21st Century Health Cures Act in late 2016, the biodefense PRV is awarded upon approval as a medical countermeasure when the active ingredient(s) have not been otherwise approved for use in any context. PRVs are transferable and can be sold, with sales in recent years ranging between $125 million to $350 million. When redeemed, PRVs entitle the user to an accelerated review period of six months, saving a median of seven months' review time as calculated in 2009. However, the FDA must be advised 90 days in advance of the use of the PRV and the use of a PRV is associated with an additional user fee ($2.7 million in 2017).

About ThermoVax®

The ThermoVax® technology is designed to eliminate the cold chain production, distribution and storage logistics required for most vaccines. The technology utilizes precise lyophilization of protein immunogens with conventional aluminum adjuvants in combination with secondary adjuvants for rapid onset of protective immunity with the fewest number of vaccinations. Cold chain requirements add considerable cost to the production and storage of current conventional vaccines. Elimination of the cold chain would also enhance the utility of these vaccines for emerging markets and for other applications requiring but lacking reliable cold chain capabilities. For vaccines that are intended for long-term stockpiling, such as for use in biodefense or in pandemic situations, the utilization of ThermoVax® has the potential to facilitate easier storage and distribution of Strategic National Stockpile vaccines in emergency situations. The underlying ThermoVax® technology has been developed by Drs. John Carpenter and Theodore Randolph at the University of Colorado.

By employing ThermoVax® during the final formulation of RiVax®, the vaccine has demonstrated enhanced stability and the ability to withstand temperatures at least as high as 40 degrees Celsius (104 degrees Fahrenheit) for up to one year. Similar stabilization at temperatures as high as 50 degrees Celsius for up to three months (maximum timepoint tested) have also been demonstrated with other antigens (e.g., human papillomavirus, Ebola and anthrax).

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 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 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.
