

On the front lines of biodefense and more with Soligenix

BY LLOYD DUNLAP

SOLIGENIX IS DEDICATED to the development of rare disease products that address unmet medical needs in the areas of inflammation, oncology and biodefense. The company was incorporated in 1987 under the name Biological Therapeutics Inc. and has been known as Soligenix Inc. since 2009. Soligenix maintains two active business segments: BioTherapeutics and Vaccines/BioDefense. *DDNews* recently interviewed the company's president and CEO, Dr. Christopher J. Schaber, to find out more about their origin and direction.

DDNews: Tell us a bit about Soligenix's origins and how it came to develop its expertise in both therapeutics and vaccines/biodefense.

Christopher J. Schaber: Our BioTherapeutics business segment is developing several therapies at this time. Specifically, we are developing a first-in-class photodynamic therapy, SGX301, that utilizes safe, visible (fluorescent) light for the treatment of cutaneous T cell lymphoma (CTCL). This treatment avoids the risk of secondary malignancies, including melanoma, associated with the frequently employed DNA-damaging chemotherapeutic drugs and other photodynamic therapies that are dependent on ultraviolet light exposure. We are also developing proprietary formulations of oral beclomethasone 17,21-dipropionate (BDP) for the prevention and treatment of gastrointestinal disorders characterized by severe inflammation. These include pediatric Crohn's disease; the objective of developing SGX203 for pediatric Crohn's is to make available a corticosteroid option with less toxicity than the current standard therapy prednisone.

Meanwhile, we are developing SGX201 for the treatment of acute radiation enteritis, the inflammatory syndrome that develops after the intestine is exposed to radiation. In addition, we are developing our novel, first-in-class, innate defense regulator technology, SGX942, for the treatment of oral mucositis in head and neck cancer.

Our Vaccines/BioDefense business segment includes several active development programs. RiVax is our proprietary vaccine developed to protect against exposure to ricin toxin and is the most advanced vaccine product in our company's portfolio. With RiVax, we are a world leader in ricin toxin vaccine research. VeloThrax is our proprietary vaccine based on a recombinant protective antigen derivative intended for use against anthrax, and we've entered into an exclusive license option with Harvard College to license it. OrbeShield is an oral immediate- and delayed-release formulation of the topically active corticosteroid BDP, which we



Dr. Christopher J. Schaber

are developing for the treatment of gastrointestinal acute radiation syndrome (GI ARS). BDP is a corticosteroid with predominantly topical activity and less systemic absorption into the bloodstream than other corticosteroids, like prednisone. Currently, BDP is approved for use in other formulations and deliveries, such as inhalation, for asthma, psoriasis and allergic rhinitis.

The development of our vaccine programs is supported by our heat stabilization technology, known as ThermoVax, under existing and ongoing government contract funding. With the help of a recently awarded government contract from the National Institute of Allergy and Infectious Diseases (NIAID), we are attempting to advance the development of RiVax. We plan to use the funds received under our government contracts with the Biomedical Advanced Research and Development Authority (BARDA) and NIAID to advance the development of OrbeShield. Additionally, we have entered into a global and exclusive channel collaboration with Intrexon Corp. through which we intend to develop and commercialize a human monoclonal antibody therapy (SGX101) to treat melioidosis, which is an infectious disease caused by a gram-negative bacterium, *Burkholderia pseudomallei*, found in soil and water.

DDNews: You currently have two therapies in Phase 3 development for cutaneous T cell lymphoma and pediatric Crohn's disease, respectively. How are these trials progressing, and when might FDA approval be forthcoming?

Schaber: Our Phase 3 trials for CTCL and pediatric Crohn's disease are both expected to be initiated during the second half of this year. Orphan Drug and Fast Track designations have been granted by the FDA for both our therapies in these indications. We have also recently received orphan drug designation from the European Commission for SGX301 as a treatment for CTCL.

We expect to obtain initial data from our Phase 3 trial for SGX301 in CTCL—on which we are working with leading research centers, as well as with the National Organization for Rare Disorders and the Cutaneous Lymphoma Foundation—during the second half of 2016. We expect to obtain initial data for our Phase 3 trial for SGX203 in pediatric Crohn's disease during the second half of 2017.

DDNews: What do you see as the commercial potential for these two therapies?

Schaber: There is a significant worldwide market potential for these two therapies. The worldwide market for SGX301 for CTCL is estimated at approximately \$250 million, and the market for SGX203 in pediatric Crohn's disease (PCD) is estimated at approximately \$200 million. The CTCL population is esti-

mated at 40,000 patients worldwide, while the global PCD population is estimated at 160,000.

DDNews: Likewise, how is the Phase 2 program to treat oral mucositis in head and neck cancer progressing?

Schaber: SGX942 has been awarded Fast Track designation from the FDA for the treatment of oral mucositis as a result of radiation and/or chemotherapy treatment in head and neck cancer patients. Our Phase 2, double-blind, dose-ranging, placebo-controlled study in approximately 100 head and neck cancer subjects with oral mucositis is nearing enrollment completion. Based on a positive recommendation from the Data Review Committee for this study, we are enrolling 20 additional subjects randomized into a single SGX942 dose group or placebo. We expect to obtain initial data in the second half of this year.

DDNews: What precisely is the "passive immunotherapy" that you're collaborating with Intrexon to develop for melioidosis?

Schaber: This is our product candidate referred to as SGX101, which is a human monoclonal antibody therapy being developed as a potential passive immunotherapy treatment for melioidosis using Intrexon's advanced human antibody discovery, isolation and production technologies. Passive immunotherapy refers to the transfer of protective antibodies to combat diseases for which there are currently no treatments or vaccines.

This approach is robust and particularly useful for protecting individuals in situations where exposure to pathogens is expected, such as in melioidosis, a potentially fatal infection caused by the gram-negative bacillus, *Burkholderia pseudomallei* (*Bps*) that is highly resistant to many antibiotics. *Bps* and the closely related *Burkholderia mallei* are not only seen in endemic regions of the world, but are considered possible biological warfare agents by the Department of Health and Human Services because of the potential for widespread dissemination through aerosol. As data becomes available from our preclinical work, we intend to pursue grant funding to support further development of this product candidate.

Under this important collaboration, Intrexon will provide discovery and development of therapeutic antibody candidates, as well as optimize and expand production of human monoclonal antibodies targeting melioidosis by applying its proprietary platforms and technologies. Soligenix will undertake preclinical and clinical development, regulatory and government interactions, as well as the commercialization of therapeutic products.

We are also developing SGX943 as a potential treatment for melioidosis. Because SGX943 directly targets the innate immune system (and does not attempt to kill the bacteria directly), we believe it is particularly relevant for this antibiotic-resistant bacteria. In February 2014, we were awarded a one-year NIAID SBIR grant of approximately

\$300,000 to further evaluate SGX943 as a potential treatment for melioidosis. Preclinical results to date have demonstrated that SGX943 treatment, in combination with standard-of-care antibiotics such as doxycycline, can statistically significantly enhance survival in a lethal murine pneumonic melioidosis model. Assuming continued positive data with SGX943 and SGX101, there is the potential that the two could be used in conjunction with each other one day.

DDNews: Finally, how are the RiVax and OrbeShield projects progressing in your vaccines/biodefense segment?

Schaber: RiVax is Soligenix's proprietary vaccine developed to protect against exposure to ricin toxin and is the most advanced vaccine product in the company's portfolio. It has been granted Orphan Drug Designation by the FDA for the prevention of ricin intoxication and has demonstrated 100-percent protection against aerosolized ricin toxin vs. control in non-human primates. Thanks to a NIAID contract award of up to \$24.7 million, the company expects to initiate a Phase 1/2 human safety study with initial data expected during the second half of 2016. We recently initiated a development agreement with Emergent BioSolutions to implement a commercially viable, scalable production technology for RiVax. We will transfer the manufacturing processes and analytics to Emergent to conduct process development work that could potentially lead to a future commercial manufacturing collaboration.

OrbeShield, an oral immediate- and delayed-release formulation of the topically active corticosteroid BDP, is being developed for the treatment of GI ARS. OrbeShield has been awarded Orphan Drug and Fast Track Designations by the FDA for the prevention of death following a potentially lethal dose of total body irradiation during or after a radiation disaster. The company has received BARDA and NIAID awards of up to \$32 million collectively. Preclinical results indicate that dogs treated with OrbeShield demonstrated statistically significant improvement in survival with dosing 24 hours after exposure to lethal doses of total body irradiation when compared to control dogs. ■

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Dr. Christopher J. Schaber has more than 25 years of experience in the pharmaceutical and biotechnology industry and has been Soligenix's president and CEO and a director since August 2006. He has also served on the board of directors of the Biotechnology Council of New Jersey since January 2009 and the Alliance for Biosecurity since October 2014, and has been a member of the corporate councils of both the National Organization for Rare Disorders and the American Society for Blood and Marrow Transplantation since 2009.