

Sanford Burnham Prebys Medical Discovery Institute

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MEDICAL DISCOVERY INSTITUTE

Partnering to advance translational research

Sanford Burnham Prebys Medical Discovery Institute is an independent, nonprofit biomedical research institute that combines deep expertise in fundamental and translational science to discover innovative medicines and diagnostics.

Sanford Burnham Prebys Medical Discovery Institute (SBP) is on a mission to advance translational research that will have a meaningful impact on human health. The institute is poised to do this by implementing a model of drug R&D that focuses on partnerships with industry and clinics at the translational juncture of fundamental research and clinical science.

Key to the institute's strategy is its history of excellence in fundamental research and its extensive investment in industry-like, in-house drug discovery expertise and technology. "Given my own experience, I know that the optimal partner for pharma contributes scientists with a deep understanding of the biology of the target or pathway of interest, a line of sight to unmet patient needs, the rigor to conduct experiments right the first time, a collegial and collaborative mindset and the ability to create a complete data package," said Perry Nisen, CEO of SBP.

This unique academia-industry blend—supported by well-funded, world-class basic research and drug discovery expertise—makes SBP the partner of choice for pharmaceutical and biotech companies (**Box 1**).

Conrad Prebys Center for Chemical Genomics

Since its establishment in 2009, the Prebys Center has been progressing laboratory discoveries to clinical studies. The center's state-of-the-art, high-content and high-throughput robotic small-molecule screening platform represents a key capability in the institute's translational strategy.

The center, led by Michael Jackson, SVP of drug discovery and development, deploys a team of 80 scientists with extensive pharma experience,

focused on discovering small-molecule drugs against disease-relevant targets newly identified and validated by scientists at the institute. Equipped with the latest high-throughput screening technology and large chemical libraries, they are advancing first-in-class projects from assay development, through lead optimization, to clinical-candidate selection. This comprehensive approach has created a robust asset pipeline of high commercial interest for potential partners.

Four disease focus areas

The institute's world-class principal investigators and industry-trained drug discovery researchers focus on four therapeutic areas: cancer, autoimmunity, metabolic disorders and neuroscience.

Cancer. SBP is one of only seven National Cancer Institute (NCI)-designated Basic Cancer Research Centers in the United States, and has held this designation for over 30 years. With more than 46 faculty and \$149 million in direct funding, SBP's world-class cancer center has revealed new insights into the molecular mechanisms of cancer, including significant contributions to the understanding of tumor initiation and maintenance, the tumor microenvironment, tumor metabolism, apoptosis and the biological underpinnings of metastasis.

Findings from these investigations have led to multiple ongoing clinical trials for solid and hematologic tumors and have served as the basis for US Food and Drug Administration–approved cancer therapies.

Autoimmunity. Guided by several world-class investigators, SBP is pushing the boundaries of immune research at the intersection of cancer and the immune system. SBP is a leader in translating research discoveries regarding cytokine signaling molecules and immune-checkpoint modulators into new therapies for cancer and autoimmune disease. The recent announcement of SBP's collaboration with Eli Lilly to develop strategies to target multiple immune-checkpoint modulators for the treatment of diseases such as lupus, Sjögren's syndrome and inflammatory bowel disease shows SBP's commitment to this space and the value its assets represent for pharma partners.

Metabolic disorders. A particular focus at SBP's Orlando campus is the study of the metabolic origins of disease, including diabetes, obesity and cardiovascular disease. In addition, scientists at the institute's NCI-designated Cancer Research Center are elucidating cell pathways that disrupt tumor cell survival and proliferation, tumor response to nutrient deprivation and pro-inflammatory pathways in cancer progression.

Through a collaboration with Daiichi Sankyo, the institute also has an opportunity to develop first-in-class therapeutics for the treatment of cardiovascular-metabolic diseases. By identifying, validating and screening new drug targets and studying new mechanisms of disease, SBP was able to develop a first-in-class small molecule that was licensed to Daiichi Sankyo.

Neuroscience. SBP is investing heavily in scientific capabilities and tools in neurobiology, which has led to collaborations with Mayo Clinic and the Michael J. Fox Foundation on basic and translational research in Parkinson's disease and with the Tanz Centre for Research in Neurodegenerative Diseases in Toronto to study Alzheimer's disease.

A focus area is the generation of induced pluripotent stem cells from patients with various neuropsychiatric disorders (e.g., bipolar disorder, Alzheimer's disease and ALS) to create disease-in-a-dish assays that closely reflect the underlying biology of disease and can be used to search for effective drugs.

Valuing success

The key value of SBP's approach is its focus on translational science. The institute's unique blend of basic research and cutting-edge technology, coupled with a solid financial foundation, offers pharma and biotech companies a way to deepen their research portfolios at all stages of development.

SBP is seeking partners with complementary capabilities in clinical development and/or as clinical partners, that is, with access to patient samples. According to Nisen, "The institute brings to the table an ability to conduct killer experiments to enable unequivocal go/no-go decisions to either proceed with a project or stop and move on to the next project. At the end of the day, success needs to be measured by some sort of external metric—for example, external validation by ethics committees, institutional review boards and regulators to support a first-time-in-human experiment."

Together with pharma and clinical partners, SBP will accelerate the pace of R&D to deliver drugs for the most challenging unmet medical needs.

CONTACT DETAILS

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BOX 1: SBP'S PHARMA AND CLINICAL PARTNERS

- The Alzheimer's Association
- Boehringer Ingelheim
- Celgene
- Daiichi Sankyo
- Duke University
- Eli Lilly
- Leukemia & Lymphoma Society
- Mayo Clinic
- Michael J. Fox Foundation for Parkinson's Research
- Pfizer
- Takeda Pharmaceuticals
- U.S. Air Force