A gift for research

By Michael J. Haas, Senior Writer

An anonymous \$275 million gift propels the **Sanford-Burnham Medical Research Institute** more than halfway toward its funding goal for a newly announced 10-year drug development plan that involves speeding the development of preclinical therapies that it hopes to out-license.

Central to the plan will be promoting closer collaborations with existing partners and forming disease-focused teams to expand the internal cross-talk between labs and clinics.

Sanford-Burnham president Kristiina Vuori told *SciBX* that the genesis of the disease-focused teams was a 2010 collaboration between Sanford-Burnham researcher Stephen Gardell and clinician Steven Smith at **The Translational Research Institute for Metabolism and Diabetes**.

The duo have been collaborating to identify markers that reflect subsets of heterogeneous metabolic diseases better than current markers.¹

Gardell's team studies multiple animal models to link metabolic phenotypes to metabolomic profiles, thereby providing a distinct signature for the disease state of each model. Smith's team performs metabolic profiling on muscle and adipose tissues across the spectrum of human metabolic phenotypes—from marathon runners to patients with obesity, diabetes and other metabolic conditions—and looks for markers that define a particular phenotype or disease subset.

The two teams then work together to match a clinical phenotype or disease subset to an appropriate animal model based on shared metabolic markers. The two-way flow of clinical and preclinical data enables the teams to identify and develop markers of metabolic disease that are meaningful in both animal models and humans.

"Gardell and Smith are a duo whose capabilities and research complement one another," Vuori said. "We want to find more opportunities for our researchers to work together like this."

The Translational Research Institute is a 2009 joint venture between Sanford-Burnham and the **Florida Hospital**.

Two-way teams

Under Sanford-Burnham's 10-year plan, integrated disease teams initially will function as informal working groups and will include basic scientists, drug discovery experts and clinicians from Sanford-Burnham and its existing partners.

"Each integrated disease team will focus on developing small molecule therapies targeting a particular disease or pathway," said Vuori, who also is interim CEO of Sanford-Burnham and a professor at the institute's NCI-Designated Cancer Center.

When an integrated disease team evolves into a more formal unit—for instance, when it begins to acquire its own funding—the team will be called a diversified translational laboratory, she said.

Vuori said that the first integrated disease teams will form this year. The number of teams and the specific disease focus have not yet been determined, but "we expect they will focus on areas in which we are already active such as cancer, diabetes and obesity, as well as some neurological indications and autoimmune diseases."

Although the institute plans to expand its network of partnerships over the next decade, the first teams will draw on the institute's existing deals with **Sanford Research**, **Florida Hospital**, the **H. Lee Moffitt Cancer Center & Research Institute** and the **Mayo Clinic**.

In 2007, Sanford-Burnham and Sanford Research founded the Children's Health Research Center to study pediatric and congenital diseases and develop therapies to treat them. Sanford Research itself is a 1998 partnership between the not-for-profit healthcare system **Sanford Health** and the **University of South Dakota**.

In 2012, Sanford-Burnham, Florida Hospital and Moffitt formed the Personalized Medicine Partnership to discover markers that could predict individual responses to drugs for cancer, metabolic diseases and cardiovascular diseases.²

Last year, Sanford-Burnham and Mayo partnered to discover compounds to treat cancer, Alzheimer's disease (AD) and other conditions. The deal built on a year-long pilot phase in which the institute used its high throughput screening technology to analyze Mayo's compound library.

Pharmas, present and future

In addition to promoting two-way communication between the lab and clinic, the 10-year plan also will involve Sanford-Burnham using its small molecule drug discovery platform to validate preclinical candidates that could be licensed to pharmas, Vuori said.

The institute already has deals with three pharmas—**Takeda Pharmaceutical Co. Ltd.**, **Johnson & Johnson** and **Pfizer Inc.**

In 2010, Sanford-Burnham and the Translational Research Institute partnered with Takeda to support the clinical development of an undisclosed obesity compound from the pharma.¹ The two institutes renewed the deal with Takeda last year.³

In 2011, Sanford-Burnham signed a three-year partnership with the Ortho-McNeill-Janssen Pharmaceutical Inc. unit of J&J to develop therapeutics against new targets in AD and other neuropsychiatric indications.⁴

Also in 2011, the institute partnered with Pfizer to discover mechanisms and therapies for undisclosed diseases under the pharma's

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Global Centers for Therapeutic Innovation initiative.

Through that initiative, Pfizer provides its partners with funding for preclinical and clinical development programs and offers IP and ownership rights in return for options to license exclusive rights to drug candidates. Academic partners have access to the pharma's antibody libraries and research and are eligible for milestones

and royalties on advanced programs.⁵

Last year, Sanford-Burnham entered a separate collaboration with Pfizer to identify and validate new drug targets to prevent and treat insulin resistance and organ damage in obesity-related diabetes. Under the three-year deal, the pharma is using the institute's Conrad Prebys Center for Chemical Genomics to conduct high throughput screening for new targets using investigational compounds from Pfizer and a compound library from the NIH.³

To fully fund its 10-year plan, Sanford-Burnham plans to raise

another \$225 million from philanthropists, investors and other sources over the next decade, Vuori said.

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COMPANIES AND INSTITUTIONS MENTIONED

Florida Hospital, Orlando, Fla.

H. Lee Moffitt Cancer Center & Research Institute, Tampa, Fla.

Johnson & Johnson (NYSE:JNJ), New Brunswick, N.J.

Mayo Clinic, Rochester, Minn.

National Institutes of Health, Bethesda, Md.

Pfizer Inc. (NYSE:PFE), New York, N.Y.

Sanford-Burnham Medical Research Institute, La Jolla, Calif.

Sanford Health, Fargo, N.D.

Sanford Research, Sioux Falls, S.D.

Takeda Pharmaceutical Co. Ltd. (Tokyo:4502), Osaka, Japan

The Translational Research Institute for Metabolism and Diabetes, Winter Park, Fla.

University of South Dakota, Vermillion, S.D.