Industrial strength for Sanford-Burnham

By Kai-Jye Lou, Senior Writer

Almost two years after its previous CEO, John Reed, left to run **Roche**'s Pharma Research and Early Development unit, Sanford-Burnham has turned to pharma for a successor. Incoming CEO Perry Nisen hopes to use what he learned in his 10 years at **GlaxoSmithKline plc** to foster partnerships and generate clinical candidates that can meet industry standards.

Nisen was SVP of science and innovation at GSK and will take the helm at the **Sanford-Burnham Medical Research Institute** on Sept. 15. President and interim CEO Kristiina Vuori will remain president. Reed, who joined Roche in January 2013, left the institute after 11 years as CEO.

The appointment of Nisen at Sanford-Burnham continues a drive that started several years ago to focus on drug discovery and engage with industry.[1] The institute refined those plans in January when Vuori laid out a 10-year strategic plan to bolster drug development and increase the out-licensing of preclinical therapies.[2] The plan includes a funding goal of $500 million. So far the institute has secured a commitment of $275 million, and it plans to raise the balance over the next decade from philanthropists, investors and other sources.

Nisen told *SciBX* that his focus as CEO will be to increase Sanford-Burnham's internal research and external partnering activities, and he thinks the institute is well placed to provide pharmas with the scientific expertise they are looking for.

"Right now, it's challenging for pharmas to justify pursuing the high-risk research programs that tend to yield the most significant breakthrough therapies," he said. Pharmas have been downsizing and externalizing early stage research efforts, he added, but "still need access to the deep scientific knowledge for studies related to human target validation and for defining patients who will and will not benefit from a particular treatment and those who might be at risk for adverse effects."

Nisen said that he brings to the institute experience in discovering and developing therapies and in engaging with regulators and payers. Nisen said that he had a hand in several recent regulatory approvals at GSK, including those involving Tafinlar dabrafenib and Mekinist trametinib.

In May 2013, the FDA approved NDAs for the two drugs to treat metastatic or unresectable melanoma individually and an sNDA this January for their combined use in the same indication. Last month, the European Commission also approved Mekinist for this type of melanoma. Tafinlar is an oral BRAF inhibitor and Mekinist is a small molecule inhibitor of MAP kinase kinase 1 (MAP2K1; MEK1) and MEK2 (MAP2K2).

To expand Sanford-Burnham's internal research, Nisen wants to foster projects that advance cross-cutting science from multiple therapeutic areas, such as combining epigenetics, metabolism and immunomodulation. Other areas of interest include personalized medicine and regenerative medicine with projects, for example, to predict a patient's response to therapy and risk for adverse effects or to promote hair cell regeneration to restore hearing.

Nisen also wants to leverage his pharma ties and partnering experience to expand Sanford-Burnham's existing alliances and form new ones. He said that the strength of the institute's chemical screening platform is a major draw for partners.

At GSK, Nisen said that he established and managed more than a dozen partnerships including those with cancer epigenetics company **Epizyme Inc.** and several other biotechs. He also helped set up the pharma's global R&D center in Shanghai in 2007.

> **"We're looking to advance our drug discovery enterprise and plan to establish a variety of creative partnerships with pharma and other entities."**
> ***—Perry Nisen, Sanford-Burnham Medical Research Institute***

"We're looking to advance our drug discovery enterprise and plan to establish a variety of creative partnerships with pharma and other entities to do this," he said. This could include biotechs, startups, other research institutes and even payers, he noted.

Although the institute has a track record of partnering and generating high-quality data, Nisen thinks that he can bolster it because he understands pharma's expectations and knows how to develop "compounds at a quality level and scale that pharma would accept." He added that "intimate collaboration between institute scientists and partners will ensure that experiments are done right the first time and to a reproducible standard necessary to progress meaningful drug and diagnostics discovery and development."

Sanford-Burnham already has partnerships with four pharmas. Its most recent pharma deal—announced in May—is with **Daiichi Sankyo Co. Ltd.** and already encompasses elements of the institute's 10-year plan.[3] The three-year deal is Sanford-Burnham's broadest industry partnership to date based on the range of projects and number of faculty members. Institute scientists will carry out preclinical studies to identify and validate new targets for cardiovascular and metabolic diseases. The partners will then work together to screen for new compounds against those targets.

Sanford-Burnham's other pharma partners are **Johnson & Johnson**, **Pfizer Inc.** and **Takeda Pharmaceutical Co. Ltd.** The institute partnered with Takeda in 2010 to support clinical development of an undisclosed compound to treat obesity.[4] The deal was renewed last year.[5]

The institute partnered with J&J's Ortho-McNeil-Janssen Pharmaceutical Inc. unit in 2011 to develop therapeutics against new targets for Alzheimer's disease (AD) and other neuropsychiatric indications. Also in 2011, Sanford-Burnham partnered with Pfizer to

discover mechanisms and therapies for undisclosed indications under the pharma's Global Center for Therapeutic Innovation initiative.[6] In 2013, the partners announced a second deal to identify and validate new drug targets for prevention and treatment of insulin resistance in obesity and diabetes.

In the last 5 years, the institute has formed more than 40 partnerships with various companies and research organizations and more than 100 research collaboration agreements.

Nisen thinks that a major challenge for the institute going forward will be to address the decline in NIH funding for not-for-profit research organizations. "One way that Sanford-Burnham will address this challenge is by establishing additional creative partnerships with pharma and other stakeholders by leveraging the institute's strengths in basic science and tapping into its in-house professional drug discovery platform," he said.

Nisen said that another challenge involves bridging the gap between basic science and meaningful clinical applications. "Sanford-Burnham must develop a 'line of sight' to clinical unmet need by arranging for our scientists to interact directly with patients and providers so that their research is informed and influenced by patients' needs, wants and expectations," he said. "The pipeline will be prioritized with targeted investment to accelerate key projects toward clinical candidate selection, and we will recruit additional top talent and establish more clinical and pharma partnerships."

He added, "A third area of opportunity is to tighten the connections between the research institute and clinical partners to enhance translational research."

Lou, K.-J. *SciBX* 7(33); doi:10.1038/scibx.2014.973
Published online Aug. 28, 2014

REFERENCES

1. Cain, C. *SciBX* **4**(5); doi:10.1038/scibx.2011.123
2. Haas, M.J. *SciBX* **7**(6); doi:10.1038/scibx.2014.160
3. Haas, M.J. *SciBX* **7**(22); doi:10.1038/scibx.2014.633
4. Haas, M.J. *SciBX* **4**(16); doi:10.1038/scibx.2011.446
5. Haas, M.J. *SciBX* **6**(33); doi:10.1038/scibx.2013.880
6. Cain, C. *SciBX* **3**(46); doi:10.1038/scibx.2010.1371

COMPANIES AND INSTITUTIONS MENTIONED

Daiichi Sankyo Co. Ltd. (Tokyo:4568), Tokyo, Japan
Epizyme Inc. (NASDAQ:EPZM), Cambridge, Mass.
GlaxoSmithKline plc (LSE:GSK; NYSE:GSK), London, U.K.
Johnson & Johnson (NYSE:JNJ), New Brunswick, N.J.
Pfizer Inc. (NYSE:PFE), New York, N.Y.
Roche (SIX:ROG; OTCQX:RHHBY), Basel, Switzerland
Sanford-Burnham Medical Research Institute, La Jolla, Calif.
Takeda Pharmaceutical Co. Ltd. (Tokyo:4502), Osaka, Japan