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Niiki Pharma developing new weapons to battle cancer

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[Hooshmand Sheshbaradaran](#) had a specific model in mind when he co-founded Niiki Pharma, a Philadelphia biopharmaceutical company whose focus is to develop new strategies for fighting cancer.

He didn't want the cumbersome layers of management found in Big Pharma companies, nor the limited scope of expertise that hampers some smaller biotech firms.

What he created was a semi-virtual company led by a small team of industry veterans. The seven-member staff has experience in all facets of drug development, having previously worked within the oncology divisions of global pharmaceutical companies such as Roche, Johnson & Johnson, Pharmacia, Pfizer and Bristol-Meyers Squibb.

"You need a broad base of experience to develop a drug," Sheshbaradaran said. "There's product licensing, research and development, manufacturing, marketing. Our team is not people who have thought about [getting a product to market], they've actually done it.... I like to say we are doing pharmaceutical company quality work with biotech agility and adding our own Niiki brand of cost-effectiveness."

With a staff of seven augmented by 14 consultants, the company maintains a low overhead and is able to allocate 80 cents of every dollar raised for drug-development expenses.

Sheshbaradaran, an Iranian-American raised in the United Kingdom, Sweden and United States, was inspired to spend his career in oncology drug development after he lost his mother to cancer when he was 24. He began his career at Pharmacia and worked at Roche then a small biotech firm before starting Niiki.

His company's name, Niiki, means "goodness" in Persian. Sheshbaradaran said the company's goal is to bring "Niiki" to cancer patients in need.

Since Niiki Pharma was incorporated in 2007, the company has raised \$15 million.

Co-founder [Ali Ardakani](#), Niiki's vice president of operations and corporate development, said the company raised \$1 million in seed funding from Philadelphia-based biotech greenhouse operator BioAdvance and another \$750,000 from Ben Franklin Technology Partners of Southeastern Pennsylvania. The remainder of the funds have come from angel investors, state and federal grants and the company's founders.

Niiki, which also has offices in Hoboken, N.J., received a \$488,000 grant under last year's Qualifying Therapeutic Discovery Project program included in the health-care reform bill. "For a company like us," Ardakani said, "it made a big difference."

"Their team had done an exceptional job raising a significant amount of angel funding in what we all know is a terrible capital environment," said [Barbara Schilberg](#), CEO of BioAdvance. "They also have done a great job managing outside consultants to get high-quality skills on an as-needed basis. The combination of the two shows the power of the semi-virtual model."

[Howard Brooks](#), an Ernst & Young partner in the firm's life science practice, said in this difficult funding environment it's not uncommon for early-stage biopharmaceutical companies to operate with small staffs and outsource activities such as research, manufacturing and even sales. "It's easier to get started and leverage efficiencies that are in place for companies that specialize in those areas," Brooks said.

Niiki Pharma recently formed a scientific advisory board led by Dr. [Daniel G. Haller](#), a University of Pennsylvania professor of gastrointestinal oncology and former editor-in-chief of the "Journal of Clinical Oncology."

The four-year-old company has two new product candidates in development. Both were invented by Dr. [Bernard Keppler](#), an internationally known metalorganic chemist, and licensed from a German incubator company that held the rights to the experimental compounds.

The first product, NKP-1339, is an intravenous injection designed to target the GRP78 pathway. In normal cells, GRP78 proteins fix or get rid of malfunctioning proteins.

In cancer cells, Sheshbaradaran explained, GRP78 goes into overdrive and high jacks proteins cancer cells need to survive. The proteins fix the damage to cancer cells caused by chemotherapy. NKP-1339 is designed to reduce production levels of GRP78 proteins. That, in turn, causes tumor cells dependent on the proteins to start dying.

Dr. [Angela K. Ogden](#), an oncologist and the chief medical officer at Niiki Pharma, said a growing body of medical literature is identifying GRP78 as a target for cancer therapies. "Nobody [other than Niiki] had developed a drug targeting GRP78," Ogden said. "Now companies are starting to look at developing molecules to block it, but those are years away from getting into patients."

Ogden said Niiki phase-I clinical trial tested NKP-1339 in patients with a variety of advanced cancers. They found the treatment produced limited and manageable side effects, such as moderate nausea and chills, but no skin rashes, hair loss or liver or heart problems — all side effects associated with existing cancer therapies.

Among their findings was positive response in several patients with neuroendocrine tumors associated with such cancers as pancreas and liver. Sheshbaradaran said the company expects to decide by mid-year what specific type of cancers it will target for phase-II testing.

Its second new drug candidate, NKP-2235, is a tablet that works, Sheshbaradaran said, by increasing the amount of stress in tumor cells so they “shut down their engines” and die.

In preclinical testing, the company said the therapy had shown the most promise in breast, cancer, lung and multiple myeloma cancers. The testing also demonstrated that NKP-2235 at low doses can prevent bone breakdown.

John George covers health care, biotech/pharmaceuticals and sports business.