BIOTECHNOLOGY

Cellceutix Corp.

Developing a still-secret prodrug for psoriasis, and a heralded cancer candidate

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Contact: Leo Ehrlich, CEO
Business: Treatments for proliferative disorders
Founded: June 2007
Founders: Krishna Menon, PhD, DVM; Leo Ehrlich
Employees: 2
Financing To Date: Public
Ticker Symbol: OTCBB CTIX
Board Of Directors: Krishna Menon; Leo Ehrlich
Advisors: Emil Frei III, MD (Dana-Farber Cancer Institute); Paul Marks, MD (Memorial Sloan-Kettering Cancer Center); Samuel Danishefsky, PhD (Columbia University); Paul Ginsburg (formerly Pfizer Inc.); Jim Boeheim (Syracuse University)

These days, most start-up companies intent on developing a novel treatment for nearly any disease make a point of talking about their drug candidate's presumed mechanism of action. It is pretty much de rigueur that a would-be drugmaker will discuss molecular *something*: if not the candidate itself, then certainly its target.

But **Cellceutix Corp.** is not a typical start-up. Founded in June 2007, it became a publicly traded company through a reverse merger in December of that year. The company already has in its portfolio eight compounds for a variety of diseases, and it also boasts an indisputably impressive roster of advisors: there's Emil Frei III, a pioneer of cancer chemotherapy whose lofty positions have included chief of medicine at the National Cancer Institute and physician-in-chief at the Dana-Farber Cancer Institute. Cell biologist Paul Marks, for 19 years the president and CEO of Memorial Sloan-Kettering Cancer Center, is on call for this company, as is famed chemist Samuel Danishefsky. Power patent attorney Paul Ginsburg, who built the intellectual property to protect mega-products like *Viagra* (sildenafil) and *Claritin* (loratadine), is on the Cellceutix support team. The firm gets business advice from legendary college basketball coach Jim Boeheim, who is also a great philanthropist for cancer research and treatment.

This all-star advisory team has likely been attracted mostly by Cellceutix's anticancer drug candidate, *Kevetrin* (thioureidobutyronitrile), which is beginning Phase I trials in October 2012 at **Dana-Farber Cancer Institute** and **Beth Israel Deaconess Medical Center**. Already, the molecule is generating huge buzz as a first-in-class compound that activates p53, a gene whose protein product is known to induce apoptosis, alias programmed cell death, in cancer cells. Kevetrin has been shown, in preclinical studies, to kill lines of cancer cells resistant to all contemporary treatments.

While Kevetrin will undoubtedly continue attracting attention appreciated by Cellceutix co-founders Krishna Menon and Leo Ehrlich, the men are deliberately saying next to nothing of substance about *Prurisol* (KM-133), their novel drug candidate for psoriasis. A patent application covering Prurisol was filed in January 2012, so the company has a year from the filing date before the application is published and available for one and all to read. "It's not heavy technology, so it's important to keep it closer to the vest," Ehrlich declares, adding, "I'm sure that many, many companies will want to read that patent. For now, the secrecy gives us a competitive advantage."

The duo will say this much: Prurisol is a prodrug formulation of an existing drug, one they believe few people would ever consider using as a treatment for psoriasis. The molecule was brought to Menon's attention some years ago, when he met with the chancellor of a university in India. At the time he was running a lab in India in conjunction with his lab in Massachusetts. Cellceutix licensed the compound upon the company's formation, and Menon says he has since worked to "improve the structure/activity relationship between the primary target and each moiety present in the compound, to optimize the effects on proteins present in psoriasis."

Menon, originally trained as a veterinary surgeon, has occupied diverse roles in academia and the pharmaceutical industry, from running the animal lab at Harvard's Dana Farber Cancer Institute to serving as group leader, cancer in vivo research and clinical development, for Eli Lilly & Co. Inc. from 1995 to 2001. Menon has already proven his ability to sniff out good drugs when they are still at the earliest stages of discovery, Ehrlich points out. In 1999, Lilly honored Menon with the President's Recognition Award, because he helped identify from Lilly's libraries the basic compounds that eventually became the hugely successful cancer drugs *Gemzar* (gemcitabine) and *Alimta* (pemetrexib).

Menon modestly credits his success at identifying winning drug candidates to "looking with an open mind" at both physical binding assays and the animals given experimental compounds. He describes Prurisol as "an anti-inflammatory drug, but not a typical one, although it has some of the same characteristics." It is an immune modulator, he declares, explaining, "When people get distressed, their immunity is going down. Some proteins released at that time will have an impact on disease, such as psoriasis, recurring in those people." He believes this drug can influence the balance of factors that stimulate hyperproliferation of cells, and so help people with psoriasis.

Because Prurisol is a prodrug formulation of a medicine already known to be safe, the FDA has advised the company that a 505(b)(2) would be an acceptable regulatory approach. A 505(b)(2) designation would allow Cellceutix to forgo early-stage trials and advance directly into Phase II clinical trials. The company expects to start the 15-patient trial in the first quarter of 2013, and plans to follow individuals with psoriasis of varying severity for two months, to assess where Prurisol works best. Once Cellceutix determines an ideal patient population, the company expects to run a larger Phase II/III trial in the US and the EU simultaneously. When the data from that trial are in, the firm will seek a marketing partner for Prurisol so that it can focus all its attention on developing Kevetrin.

But first things first. Cellceutix won't start any trials of Prurisol until it knows it has sufficient quantities of the molecule to pro-

ceed. To that end, the company announced at the end of August that it had hired **Dr. Reddy's Laboratories Ltd.** to manufacture the compound. The decision came about in part because Dr. Reddy's, the second-largest drug manufacturer in India, with a $5 billion market capitalization on the New York Stock Exchange, is one of the world's few manufacturers of the parent compound.

But that's not the only reason Cellceutix picked this partner. Ehrlich says that Cellceutix originally tried to formulate Kevetrin with a Massachusetts company called Formatech, after consciously choosing to support local business and the state. "During the project they didn't tell us they had a warning letter from the FDA and were going to go out of business," Ehrlich moans. Sure enough, when Cellceutix first applied to FDA for an IND for Kevetrin, the agency turned it down. The firm had to reformulate the compound from scratch, wasting hundreds of thousands of dollars and losing a year. In advancing Prurisol this time, Ehrlich had no patience for dealing with smaller vendors.

While Dr. Reddy's sets about synthesizing Prurisol and making it into a pill, Cellceutix is gearing up for clinical trials and a patent publication that both Ehrlich and Menon expect will bring positive attention to their anything but ordinary start-up. [A#2012900202]

— Deborah Erickson