

October 23, 2009

Louisville's Potentia Pharmaceuticals sells drug to eye-care giant Alcon

By Patrick Howington
phowington@courier-journal.com

A small Louisville-based biotech company has struck a multi-million dollar deal to sell rights to a potentially ground-breaking drug it is developing to Alcon, the world's largest eye-care company.

Terms of the deal are confidential, but Potentia Pharmaceuticals said it expects to receive hundreds of millions of dollars if the drug for age-related macular degeneration — the leading cause of blindness — is approved for widespread use.

The deal, formally announced Friday, gives Alcon licensing rights to the drug and an option to buy the Louisville company. Potentia will receive an undisclosed payment now, and is promised a series of payments if it achieves certain milestones — for example, successfully completing a clinical trial phase and receiving FDA clearance.

The deal is potentially the largest ever signed by a Louisville biotech company — and the largest coup to date for the city and University of Louisville's efforts to establish a life-sciences industry here.

It would dwarf the city's previous biggest life-sciences success story— the \$21.4 million acquisition of Aptamera, a Louisville company that developed cancer drugs, by British company Antisoma.

"I feel fantastic," said Cedric Francois, CEO of Potentia. "I think it's really neat, in the current economic environment, to be able to get a deal done like the one that we did — and to have a company, like Alcon, which is absolutely incredible in ophthalmology, take over this program."

But most of all, "we're hopeful that this will become a new treatment for macular degeneration," he said. "As happy as I am today, what I really wish and hope is that this drug ... becomes available to patients worldwide."

Potentia's deal with Switzerland-based Alcon, which had \$6.3 billion in U.S. sales last year, is "another example of how Louisville is becoming a city of medical innovations and discoveries," Metro Louisville Mayor Abramson said through a spokesman on Thursday. "Our decision to focus on growing the medical and biomedical industry ... is paying dividends for our city."

University of Louisville President James Ramsey said Potentia's success shows Louisville's strategy of developing U of L researchers' technologies and moving them "from the mind to the marketplace" is working.

He said U of L hopes Potentia's scientists will expand their work "and grow even more at the University of Louisville," perhaps becoming tenants of the life-sciences complex the university plans to build on the old Haymarket property.

Francois, 37, said he expects the company's scientists will stay in Louisville and start new companies.

Potentia was founded in 2001 in the Boston area and moved in 2003 to Louisville, where its growth was aided by U of L and two related organizations that help life-sciences companies grow — Nucleus and MetaCyte Business Lab.

Potentia's drug compound is aimed at blocking the deterioration of the central retina that causes macular degeneration. More than 10 million Americans have the incurable disease.

The company has been working for four years on the drug, which has been found safe in an initial human clinical trial. Potentia said the trial also found that once injected in the eye, the compound keeps working longer than some other AMD drugs that require monthly injections.

If approved by the FDA after remaining trials, it might be used initially on the less common but more serious form of the disease, the so-called "wet" form in which leaking blood vessels block vision.

But its great promise may be against the "dry" stage, which makes up about 90 percent of cases.

Potentia's drug is in a race to become the first treatment for that form. Other companies have drugs in clinical trials, but they use a different approach than Potentia's.

Approval of any treatment for the dry form "is going to be very exciting, because when you're diagnosed ... and then they tell you that there's nothing that they can do for you, and to go get an eye vitamin — that's very discouraging," said Liz Trauernicht, president of the Macular Degeneration Foundation.

She said she doesn't know enough about the treatments being tested to evaluate their promise.

The deal with Alcon is expected to help Potentia's compound reach the marketplace, a process that could take five to 10 years.

Besides funding, the company has expertise in drug formulation and clearing regulatory hurdles to bring a product to market, said Doug MacHatton, vice president and spokesman.

He said Alcon was attracted to Potentia because of the mechanism the company's compound, called POT-4, uses to attack macular degeneration. The compound inhibits the activation of an immune-system protein linked to the disease.

Alcon has long had an interest in that mechanism and its potential to treat a variety of retinal diseases, MacHatton said. He said Potentia's compound was the first in clinical trials to take that approach.

MacHatton said he can't predict the drug's chances of being approved by the Food and Drug Administration for use by doctors.

But Alcon is "the best partner we could hope for," said Pascal Deschatelets, 39, chief operating officer of Potentia and one of its founding scientists.

Potentia's scientists were trained at Harvard, and the company has a cosmopolitan flavor: its CEO, Francois, is Belgian, and Deschatelets is from Canada.

The company moved to Louisville after Francois, a medical doctor, came to work with U of L's hand-transplant team while earning a doctorate in physiology.

Dr. Donald Miller, who has aggressively recruited scientists to U of L's James Graham Brown Cancer Center since becoming its director in 1999, helped arrange faculty posts for Potentia's two top scientists so they would have a salary and lab space, while letting Potentia keep the rights to its

scientific discoveries.

The fledgling company set up offices in Louisville's downtown life-sciences research park.

Through state and federal grants, angel investors, and venture funds, Potentia raised about \$25 million to pay for its continuing research and clinical trials.

A key discovery by company scientists involved scavenger cells called macrophages, which the human body sends out to eat and get rid of its dead cells.

Potentia researchers found a way to alter living disease cells to make the scavenger cells go after them.

The discovery seemingly had wide applications. But in 2006 it led Potentia to a deal with the University of Pennsylvania aimed at one disease in particular — macular degeneration.

The deal gave Potentia the rights to develop and market a compound discovered at Pennsylvania. The peptide will bind tightly to an immune-system protein linked to macular degeneration. The binding blocks the disease process.

"I'm just so thrilled for these guys," said Steve Gailar, CEO of MetaCyte Business Lab, which is helping 16 young life-sciences companies to grow. "Because it proves the point. ... We have a number of companies here. ... If we just stick with them, and if they can raise money, these kinds of things can happen."

Reporter Patrick Howington can be reached at (502) 582-4229.
