



## Newark company targets 'orphan drugs'

### UD scientists research diseases overlooked by big drug makers

By GARY HABER, The News Journal

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NEWARK -- With a new management team in place and a focus on a market largely ignored by big pharmaceutical companies, a tiny biotech company started by two researchers from the University of Delaware is ramping up operations.

Orphagenix Inc. doesn't plan on slugging it out with behemoths like AstraZeneca PLC and Merck & Co. in developing drugs for widespread conditions, such as diabetes or high cholesterol.

Instead, this two-year-old startup plans to use a novel gene-repair therapy developed by its founders, Eric Kmiec, a UD professor, and Hetal Parekh-Olmedo, a UD senior research associate, to develop what are known as orphan drugs.

Orphan drugs target diseases that affect 200,000 patients or fewer. The phrase comes from "The Orphan Drug Act," the law Congress passed in 1983 to encourage drug makers to develop treatments for relatively rare, but sometimes deadly, diseases the big drug makers were overlooking.

Two of the diseases Orphagenix is pursuing are sickle cell anemia and spinal muscular atrophy, says Michael Herr, who joined the company this year as president and chief executive officer.

Herr said the company is in discussions with Dr. Kwaku Ohene-Frempong, director of the Comprehensive Sickle Cell Center at Children's Hospital of Philadelphia, and Dr. Betty Pace, chief medical officer for the Sickle Cell Disease Association of America, to collaborate on developing a treatment for sickle cell anemia.

Sickle cell anemia and spinal muscular atrophy are genetic diseases that are serious, but relatively uncommon.

Sickle cell anemia is an inherited blood disorder that affects about 70,000 people in the United States, mostly blacks. Spinal muscular atrophy is a motor-neuron disorder that affects about 1 in 6,000 newborns. It's the biggest genetic cause of death among children younger than age 2, according to Families of Spinal Muscular Atrophy, an advocacy group.

Herr, who came to Orphagenix from the University City Science Center, a Philadelphia incubator for early-stage life science and technology companies, says both diseases are well-suited to the gene-replacement process Kmiec and Parekh-Olmedo developed.

The two researchers found a way to introduce a minuscule fragment of DNA into a cell where a portion of the DNA is damaged.

The introduced DNA binds to the damaged portion of the DNA, triggering the cell to heal itself. What is introduced into the cell is eventually released out of the cell.

#### **A potential big payoff**

The promising process is what attracted Herr to join the company from the Science Center, where he helped biotech startups develop business plans and find funding.

"I was blown away by the elegance, the simplicity, the thoroughness of the work they were doing here," he says.

Orphagenix licenses the technology it uses from the University of Delaware, which holds the patents. The university will share in the profits from any drug developed using the process.

The payoff could be huge.

Merck & Co. recently paid \$1.1 billion to acquire Sirna Technologies, a California biotech that is developing drugs using a gene technology called RNAi, or RNA interference, that can switch off genes that cause various diseases, such as cancer.

Kmiec, one of Orphagenix's two founders, said he realized that if he wanted the company to grow, he needed someone with business expertise to turn the research into a commercially viable product.

"You've got to be willing to hand the ball off," says Kmiec, 51, who, along with Parekh-Olmedo, remains as a consultant to the company. "The science is done. It's now the applications side."

Herr, already a biotech veteran at the age of 30, is helping Orphagenix decide which diseases it should target.

He also helped line up about 20 backers to invest in the company, raising an amount Herr declined to specify, but called "enough to begin our commercialization process."

"We've got the tool box full," he says. "Now we can actually build something with it."

Herr has also helped Orphagenix craft its strategy to focus on orphan drugs.

### **Big money for niche drugs**

The Orphan Drug Act, to help spur research into rare diseases that the drug companies would otherwise neglect because of the small patient base, provides a host of incentives, from a streamlined review process to a tax credit that allows companies to recoup as much as 50 percent of their research-and-development costs.

Perhaps the biggest carrot the law provides is seven years of market exclusivity, meaning that if the Food and Drug Administration grants a company's drug an "orphan drug status," it would prohibit other companies from selling a competing drug during that period.

The law has resulted in what Maria Hardin, vice president of patient services for the National Organization for Rare Diseases, calls "a paradigm shift" in the drug industry, causing biotech companies to pursue orphan drugs with a vigor.

The success of companies such as Genentech Inc. and Genzyme Corp. "has served as a model for other up-and-coming biotech companies," Hardin says.

Genentech, which had revenue of more than \$9 billion last year, started out making a human growth hormone for dwarfism, a market with a small number of patients. It has since branched into other areas, including drugs for treating cancer.

Genzyme developed a highly profitable drug for treating Gaucher's disease, a rare condition in which patients suffer extreme fatigue due to the buildup of a fatty substance in the spleen, liver and lungs.

While Big Pharma has left orphan drugs largely untouched, it doesn't mean Orphagenix won't face plenty of competition.

At least two drug companies are working on treatments for spinal muscular atrophy, as are researchers at a host of academic institutions, said Lenna Scott, communications director for Families of Spinal Muscular Atrophy.

But there is room for researchers to work on all sorts of different approaches to the disease, says Scott, whose group funds research into the disease.

"Any researcher looking at novel approaches to this disease should be commended," she said.

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