Pharmaceutical Executive

Seven top therapies and technologies vying to deliver the next decade's breakthroughs and blockbusters. They want to become...

TOMORROW'S DRUGS

Have you heard the rumors that all the drugs have already been discovered? Well, forget it. Maybe some of the low-hanging fruit is gone—but in the last few years, science has opened up thousands of new targets, while new technologies have made it possible to treat disease in unprecedented ways.

In the next few pages, the *Pharm Exec* staff profiles some of the hottest therapies and smartest technologies that have caught our eye—from RNAi therapies to genetic editing to therapies that stop disease before it starts. Taken together, they form a vision for the next generation of drugs, one that will transform the industry (and the face of healthcare) in the decades to come.

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HEALTHY **HEARTS**

A cardiovascular drug company builds stronger hearts with new blood vessels

The idea came as Thomas Stegmann walked down a hospital corridor in Fulda, Germany, after performing a triple-bypass on a patient with severe coronary artery disease. It was surgery he had performed countless times before, but this time Stegmann was struck by a thought, inspired by an article by Harvard's Judah Folkman on the use of anti-angiogenesis to halt tumor growth.

He liked the approach as a solution to an oncologist's problem. But he was a cardiovascular surgeon, and saw the concepts in the article as a potential application to heart disease as well. "What the tumor researchers do not want [the growth of the new blood cells] would be very much desired by *our* patients who suffer from narrowed, calcified, occluded vessels," he wrote later, describing the "a-ha" moment.

"Stegmann developed this treatment with his own money to prove that he could grow blood vessels—and he did it," says Dan Montano, chief executive officer of CardioVascular BioTherapeutics, the company that is now developing Stegmann's discovery.

The treatment uses fibroblast growth factor 1 (FGFR1), a protein that the body naturally creates. CardioVascular BioTherapeutics concentrates the protein and delivers FGFR1 directly into the heart, setting off an angiogenic effect.

In Phase I, the treatment was delivered surgically through a small incision in the chest. Going forward, the drug will be administered with a catheter, much like an angioplasty. The treatment consists of one injection per area. A person with, say, four blockages might need four injections, each in a different area. Using the catheter, patients can get the treatment and be out of the hospital in one day. The angiogenesis process takes approximately 12 weeks.

"The majority of the patients had miraculous recoveries," says Dave Balekdjian of the Bruckner Group, which has consulted with CardioVascular BioTherapeutics. "It's one thing to get a treatment and live longer, but it's another to return them to the quality of life they were used to before they became ill."

To date, there have been no known side effects or safety issues, probably due to the fact that the protein is injected in a relatively small quantity and is injected directly into the blockage. According to research, the body clears the protein within six hours.

"Normally, people wouldn't be getting excited about a drug that is just hitting Phase II, but the difference is that the drug has been dosed in more than 70 patients, and there are long-term outcomes data," Balekdjian says. The company can image the growth of new arteries and see the physical evidence of angiogenesis with before-and-after pictures.

CardioVascular BioTherapeutics seems to be in the lead in bringing this treatment to market, but it's not alone. *Business Week* recently reported that close to 700 angiogenesis-based drugs were in development. Many are for cardiac care, but other areas under consideration include wound healing, peripheral arterial disease, chronic back pain, and stroke. Stegmann also hopes that FGFR1 will lead to medical treatments for neurological diseases such as smyotrophic lateral sclerosis, multiple sclerosis, Parkinson's disease, and spinal cord injury.

–GEORGE KORONEOS

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