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Roche Backs New Method for Drug Delivery to Cells

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The Swiss pharmaceutical giant [Roche](#) is throwing its weight behind an experimental technology that could be used to treat a number of diseases.

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Jodi Hilton for The New York Times

Joseph A. Yanchik III, chief executive of Aileron Therapeutics, which is developing a new drug technology called stapled peptides.

The company has agreed to pay \$25 million now and up to \$1.1 billion later to Aileron Therapeutics of Cambridge, Mass., for developing a new type of drug technology called “stapled peptides.” They are expected to announce the agreement on Tuesday.

Aileron, which holds patent rights from Harvard and the Dana-Farber Cancer Institute, hopes to start clinical trials next year. It is testing a stabilized form of peptides, a small protein, to deliver medicine inside cells for a variety of medical conditions, including Roche’s priorities like treatments for [cancer](#) and inflammation.

“This is a significant commitment by a very smart pharmaceutical company betting they’re finally going to unlock the power of peptides as a superclass of drugs,” Joseph A. Yanchik III, the chief executive of Aileron, said in a phone interview on Monday.

The synthetic peptides, developed by a Harvard chemical biologist, have been described as a type of magic bullet that can deliver particularly potent doses of drugs at the cellular level. They are stabilized in a helical shape that stays active longer in the body. Mr. Yanchik said they have been successfully tested in animals.

He said the company started preliminary talks with the [Food and Drug Administration](#) in June. The F.D.A. must approve an investigatory new drug application before any human testing.

Dr. Jean-Jacques Garaud, Roche's global head of pharmaceutical research and early development, said stapled peptides could open new horizons in medicines.

"One of the challenges the industry is facing is not to identify new targets, but to be able to reach the target that we would like to reach with the right therapeutic benefit, particularly inside the cell," he said in a phone interview.

He emphasized, however, that they are years from proving it will work in patients. "Obviously no one knows yet, but it is worth exploring as a tool," Dr. Garaud said.

Richard D. DiMarchi, a professor and former chairman of the chemistry department at [Indiana University](#), who is not involved with Aileron, said the deal shows that big pharmaceutical companies are embracing the novel peptide and protein technologies.

"Peptide molecular medicine is a very hot area, and this concept of stapled peptides is a new area of great promise," he said.

Professor DiMarchi co-founded Marcadia Biotech in 2005 and signed a licensing deal with [Merck](#) in 2008 to pursue other forms of stabilized peptides to treat [diabetes](#) and [obesity](#). He said those materials, which work outside cells, are now in clinical trials.

Roche and Aileron said they would focus on five target diseases. Citing proprietary reasons, they would not identify the diseases but said they fall among Roche's priorities of cancer, viruses, inflammation, metabolism and the central nervous system. Aileron's lead research aims at cancer.

Dr. Garaud said Roche's chief executive has approved the deal with Aileron. Mr. Yanchik said the companies signed the deal last week.

Officials with both companies said Aileron would receive the full \$1.1 billion only if its products succeeded in all five therapeutic areas. Most of the money would be paid after reaching certain milestones and in royalty rights.

Such new technologies are fraught with possible setbacks, however, on the path from laboratory bench to bedside. Monoclonal [antibodies](#), for instance, have yet to live up to expectations.

In another example of innovative biology that companies hoped would provide new therapeutics, Roche has invested heavily in technology called RNA interference. Roche has made two big deals to advance

the RNA technology. It agreed in 2007 to pay up to \$1 billion to [Anylam Pharmaceuticals](#) of Cambridge and in 2009 to pay up to \$50 million to Tekmira Pharmaceuticals of Canada.

The company, however, is not meeting its goal of starting a clinical trial this year, a spokesman said.

Existing peptide products, without the stapling, include Forteo from [Eli Lilly](#) for [osteoporosis](#) and Byetta from Amylin and Lilly for [Type 2 diabetes](#). So far the peptides must be injected; they do not come in pill form.

Roche, based in Basel, is the world's largest biotech company and bought [Genentech](#) last year. Roche recently reported a \$5.35 billion profit for the first six months of the year.

Aileron, founded in 2005, has about 40 employees.

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