BY CHRIS TACHIBANA

A New Year's wish at Santaris: more of the same

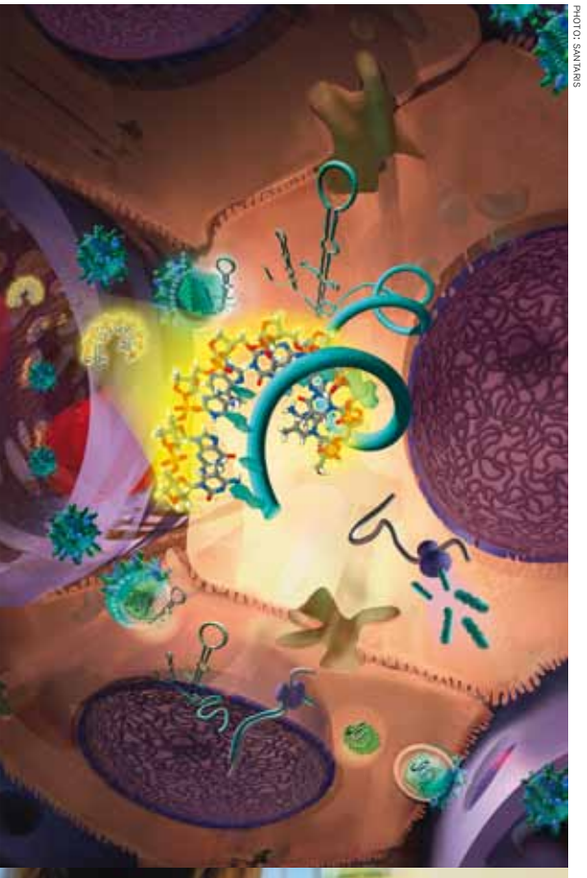

PHOTO: SANTARIS

High hopes for 2012 after business and clinical wins in 2011

It was a good year at Santaris. In January 2011, the biopharmaceutical company based in Hørsholm, Denmark announced a $14 million drug development deal with Pfizer. This continues an agreement made with Wyeth, which was acquired by Pfizer in 2009. Milestone payments to Santaris could total $600 million.

Søren Tulstrup, Santaris president and CEO says, "2011 was a phenomenal year for us, with three programs in the clinical phase and proof-of-concept for our platform overall." The proof—the second piece of good news at Santaris in 2011—came from a phase 2a clinical trial of miravirsen, an anti-hepatitis C drug. The hepatitis C virus infects an estimated 170 million people worldwide and can lead to cirrhosis and increased risk of liver cancer. No vaccine is available and current treatments have severe side effects. However, in 2011, Vertex Pharmaceuticals won US Food and Drug Administration approval for a protease inhibitor that boosts the previous 30-40 percent cure rates to 80 percent. Gilead, Merck, Roche and others are also exploring anti-hepatitis C therapies.

In November 2011, Santaris announced results from a randomized controlled clinical trial of miravirsen. Cohorts of nine hepatitis C patients treated with miravirsen and three receiving placebo were tested at three dose levels. At the highest level, all nine treated patients had reduced viral levels after four weeks; in four, the virus was undetectable. Michael Hodges, Santaris vice president and chief medical officer, says the drug is well tolerated, with no signs of viral resistance so far. Miravirsen has a long half-life in the body, so once-monthly subcutaneous injections should be possible. The next trials, planned for early 2012, will extend the treatment to 12 weeks.

Hepatitis C is treated with a combination, or cocktail of drugs. Treatment used to be up to 18 months of a regimen of interferon and the antiviral drug ribavirin, which can cause serious side effects, such as anemia. Future therapies may use more well-tolerated agents and take 12-24 weeks. Miravirsen could make that even better, says Hodges. "Miravirsen in a future cocktail could result in a non-interferon-containing regimen that is well tolerated and could lead to a cure in as little as 12 weeks."

Tulstrup says it will be "a few years" before miravirsen might reach the market. "We are in dialog with potential partners," he says, "or we're prepared to take this compound further ourselves." The really exciting news, says Hodges, "is this is the first microRNA in clinical testing. In pharma, we first saw small molecule drugs, then came monoclonal antibodies. The third wave in drugs might come from oligonucleotide therapies."

Miravirsen, like the other drugs in development at Santaris, is a synthetic oligonucleotide. These short nucleic acids are "locked" or chemically modified, to increase compound stability and target affinity. The locked oligonucleotides bind to a specific nucleic acid in cells, controlling cell properties such as production of a particular protein, or in the case of miravirsen, production of hepatitis C virus. This strategy is known as antisense therapy.

The third piece of good news at Santaris in 2011 was about the locked nucleic acid technology. In November, the United States Patent and Trademark Office (USPTO) ruled in favor of Santaris in a challenge brought by Isis Pharmaceuticals of California. Isis claimed that the locked nucleic acid products infringed on Isis patents for modification of oligonucleotides for antisense therapy. The USPTO, however, upheld the Santaris patents.

For 2012, Santaris is focusing on oligonucleotide treatments for infectious and metabolic diseases and working with partner Enzon Pharmaceuticals on cancer therapies, hoping to keep the momentum going. "We're wired up to go in 2012, hoping for another exciting year," says Tulstrup.