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Nobel Discovery Already Sparked Hunt for Drugs

By DAVID P. HAMILTON and RACHEL ZIMMERMAN
October 3, 2006; Page A17

Two U.S. biologists who discovered a powerful method for "silencing" the activity of individual genes won the Nobel prize in medicine for their work, an award that highlights a young but mushrooming research field that is one of the hottest areas in drug development.

MEET THE WINNERS

Learn more about Messrs. Fire and Mello, see which Nobels will be awarded next and catch up on previous years' winners.

Andrew Fire, now a biomedical researcher at Stanford University, and Craig Mello, a scientist at the University of Massachusetts, shared the prize for research done in 1998 that outlined a previously unknown mechanism by which many cells suppress errant natural genes or genes carried by invading viruses. Known as RNA interference, or RNAi, the mechanism is now also seen as a possible way to create powerful new therapies that could target the activity of specific disease-related genes.

That promise has kicked off an almost unprecedented flurry of commercial activity over the past several years.

More than a half-dozen biotechnology start-ups are now in hot pursuit of new RNAi therapies, and three of them have already completed early-stage human tests. Investors and major pharmaceutical companies ranging from Merck & Co. to GlaxoSmithKline PLC have handed these biotechs roughly half-a-billion dollars, and the big drug makers have pledged nearly $2 billion more if the drugs work as planned.

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Even the federal government is climbing aboard the bandwagon, having recently awarded Alnylam Pharmaceuticals Inc., an RNAi biotechnology company in Cambridge, Mass., a $23 million contract to develop drugs as a defense against bioterrorism.

"This is a poster child for how the most seemingly obscure basic science can have just an enormous impact," said Gregory Hannon, a researcher at Cold Spring Harbor Laboratory on Long Island, who has made key contributions to understanding how the interference process works. "It opened the door to a whole world of biology that we didn't think even existed."

The seminal work by Drs. Fire and Mello described machinery inside human cells that can disrupt the production of proteins by a particular gene. In effect, this RNA interference process silences the gene, blocking any harmful effects its proteins might cause.

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AP/Wide World Photo

Prof. Gran Hansson of the Nobel Committee announced the recipients of this year's Nobel Prize in Medicine.

As researchers expanded their understanding of the process, it gradually became clear that RNAi might be an extremely useful tool for treating disease. Because the RNAi gene-silencing machinery already existed in cells, it needed only a trigger to kick it into action. That meant an RNAi drug might work at relatively low doses, unlike earlier and largely unsuccessful attempts to silence genes directly, which required high doses to overwhelm the protein-production process.

In 2002, Phillip Sharp, a Nobel laureate biologist at the Massachusetts Institute of Technology, co-founded Alnylam in order to turn RNAi discoveries into medical treatments for a variety of conditions. The same year, a team from the University of Pennsylvania founded Acuity Pharmaceuticals in Philadelphia, which focused on eye diseases such as age-related macular degeneration, a leading cause of blindness in the elderly.

They were soon joined by several other start-ups, one of which was previously an unsuccessful biotechnology company called Ribozyme Pharmaceuticals. In 2003, the company shifted its focus to RNAi, changed its name to Sirna Therapeutics and adopted

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"RNAI" as its stock symbol to capitalize on its association with the burgeoning field.

Several major pharmaceutical companies have recently agreed to put their financial muscle behind the new technology. Last year, for instance, Novartis AG agreed to pay Alnylam as much as $700 million to develop drugs for a number of diseases based on Alnylam's technology. In April, Sirna struck a similar deal with GlaxoSmithKline to develop RNAi drugs for respiratory disease.

Genetic Missiles

Biotechnology companies creating drugs based on RNA interference

COMPANY	MAJOR PARTNERS	TOTAL FUNDS RAISED	DISEASE TARGETED	PROGRESS
Acuity Pharmaceuticals (Philadelphia, Pa.)	None	$24 million	Macular degeneration	Completed mid-stage human tests
Alnylam Pharmaceuticals (Cambridge, Mass.)	Merck, Novartis	$232 million plus commitments of more than $950 million	Respiratory syncytial virus	Completed early-stage test
Sirna Therapeutics (San Francisco, Calif.)	GlaxoSmith-Kline, Allergan	$188 million plus commitments of more than $900 million	Macular degeneration	Completed early-stage test

Note: Commitments are future milestone payments and, in some case, royalties that will generally materialize if the drugs seem to work.
Source: the companies

Despite the technology's promise, the field of RNAi therapeutics remains in its infancy. Companies like Alnylam, Sirna and Acuity aim to make drugs from short stretches of RNA that can target the cellular gene-silencing machinery against a disease-related gene. In their natural form, however, such RNA molecules are cleared quickly from the blood, meaning that they can't easily be delivered via pills or even as shots. As a result, the first RNAi treatments tested in people target diseases that can be treated with a local application of a drug. Acuity's macular-degeneration drug, which just completed a midstage human trial, requires direct injections to the eye, the same as existing treatments for the condition.

Similarly, Alnylam recently tested a new RNAi drug against respiratory syncytial virus, a common childhood infection, by delivering it directly to the lungs via a nasal spray. Alnylam and Sirna both say they've made important progress in chemically modifying

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COMPANIES

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Merck & Co. Inc. (MRK)

PRICE	41.82
CHANGE	-0.04
	4:01p.m.

GlaxoSmithKline PLC ADS (GSK)

PRICE	54.12
CHANGE	-0.21
	4:02p.m.

Sirna Therapeutics Inc. (RNAI)

PRICE	6.65
CHANGE	0.34
	4:00p.m.

Novartis AG ADS (NVS)

PRICE	57.74
CHANGE	-0.27
	4:02p.m.

Alnylam Pharmaceuticals Inc. (ALNY)

PRICE	17.09
CHANGE	0.30
	3:59p.m.

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The discovery of RNAi has been a windfall for the Carnegie Institution, where Dr. Fire used to work, and the University of Massachusetts, which share rights to a basic patent on the work. The two institutions have already taken in more than $5 million since around 2000, and some experts believe the patent could be among the biggest intellectual-property home runs ever for a university, depending on what products are developed.

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Some observers grouse that the U.S. Patent Office allowed the Nobelists too powerful a patent, one that undergirds all future work. However, the schools have eased tensions by licensing rights to all comers. Companies wanting to use RNA interference pay an initial $85,000 fee, followed by $35,000 annual payments and certain milestones ($50,000 on FDA approval of a trial, $50,000 upon marketing). Royalties on product sales are negotiated separately, said Paul Kokulis, an attorney with Morgan, Lewis & Bockius of Washington, D.C., which has handled the patent. More than 50 companies have licensed rights so far.

--Antonio Regalado contributed to this article.

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