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Business focus: Biotech

## New kids on the block

By Penni Crabtree, SPECIAL TO THE UNION-TRIBUNE

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A decade after the Human Genome Project, which provided new insights into genes and novel tools to explore them, biotechnology companies are using that knowledge to develop cutting-edge therapies and technologies.

Here are some of the newest and most innovative leaders on San Diego's biotech block:

### REGULUS THERAPEUTICS

Founded: 2007

Employees: 45

Product: Potential treatments for inflammatory disease, hepatitis C

What happens when two venerable biotech players in the field of RNA research come together to create something new?

You get Regulus Therapeutics, the decidedly precocious offspring of Carlsbad's Isis Pharmaceuticals and joint-venture partner Alnylam Pharmaceuticals of Cambridge, Mass.

RNA, the genetic material crucial in the production of proteins, has spawned several biotech companies over the years.

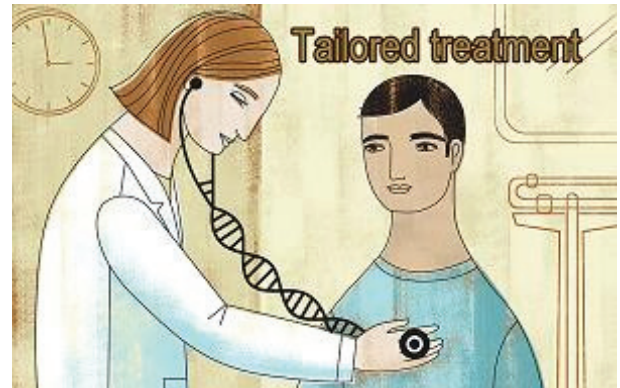
But Regulus is cutting its baby teeth on a relatively new discovery — microRNA — and has already signed major deals to develop drugs to treat hepatitis C and inflammatory diseases.

Kleanthis Xanthopoulos is CEO of Regulus Therapeutics, which is developing drugs that act on microRNA, believed to regulate about one-third of all human genes.

MicroRNA, tiny strands of RNA that regulate gene expression, weren't discovered in humans until 2001. Nearly 700 microRNAs have been identified in the human genome, and more than one-third of all human genes are believed to be regulated by them, said Regulus Chief Executive Kleanthis Xanthopoulos.

MicroRNAs can affect one gene or protein, or entire networks of genes. That makes them the "master maestros" of the human genome, holding sway like a conductor over an orchestra, Xanthopoulos said.

But in a disease, the maestro microRNA — like any sensitive conductor — can get into a huff, erratically blocking some musicians or occasionally disemboweling them with the genetic baton.



"When the maestro gets out of whack and overdoes the job, there are severe consequences," Xanthopoulos said with a laugh. "We want to develop drugs to get the maestro to return to normal so the musicians can get back on key."

At least one major pharmaceutical company is already tapping its feet to the beat. In 2008, Regulus signed a deal with GlaxoSmithKline for \$20 million, and as much as \$600 million in future milestone and development fees, to create microRNA drugs for inflammatory diseases.

And GlaxoSmithKline came to the table again in February, this time paying as much as \$150 million for a collaboration to develop drugs for hepatitis C.

Xanthopoulos says drug companies are jumping at microRNA because it has the same potential for blockbuster therapies that monoclonal antibodies showed 20 years ago.

"MicroRNA is one of the most innovative discoveries of the decade," Xanthopoulos said. "The science is exploding right now."



Photo by John Gastaldo - Union-Tribune