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Startup Arcturus Pulls in \$1.3M in Seed Money amid Renewed Interest in RNAi

by Doug Macron

A NEW FIRM threw its hat into the RNAi therapeutics ring this week, with San Diego startup Arcturus Therapeutics announcing that it has raised \$1.3 million in seed financing to advance proprietary siRNA delivery and formulation technologies into *in vivo* proof-of-concept testing.

Arcturus represents the latest RNAi drug shop to secure investment dollars over the past 12 months to fund its research and development efforts, reflecting a renewal of interest in the gene-silencing technology following the departure of two key players in the space a few years ago.

The company was founded earlier this year by Joseph Payne, who serves as president and CEO, and Pad Chivukula, who is CSO and COO. Both most recently worked for Nitto Denko, helping shepherd that company's siRNA-based fibrosis drug toward a newly initiated phase I trial (see *related story*, this issue).

With backgrounds in both RNAi and nanoparticle delivery, the two set up Arcturus at Janssen Labs — an accelerator for startups housed at Johnson & Johnson's Janssen Research and Development — with a team of about 12 consultants and part-time employees.

The company's core technology is a biodegradable lipid-based nanoparticle that Chivukula says promises a therapeutic

index “much higher than what's already out there.

“The first generation of nanoparticles were non-biodegradable and have the potential to accumulate in the body,” he explained. “Keeping that in mind, we designed our nanoparticles to completely biodegrade.”

The nanoparticles also feature a targeting technology designed to carry them preferentially to target tissues, Payne noted. However, he added that Arcturus is not yet publicizing specific details about its technologies since the company is still in the process of hammering down relevant intellectual property.

Thus far, Arcturus has been working on demonstrating its approach to RNAi *in vitro*, and has managed to find private investors willing to put up seed funding that will enable the company to begin the *in vivo* studies. That work, Chivukula said, is expected to generate data that will raise interest in a bigger follow-on financing round.

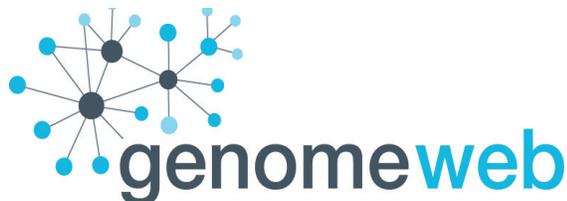
The \$1.3 million “only took a couple of weeks to raise,” Payne said. “We originally planned on raising a million dollars, and we oversubscribed. We pushed a lot of money away, quite frankly. Those people are still engaged, and we have a substantial list of private investors ... who are planning on supporting our next round.”

And Arcturus will need that additional money to support its plan to advance a lead drug candidate into at least phase II before looking for a partner.

Not looking to tip their hand at this early stage, the Arcturus officials did not comment in detail on the disease areas the company is pursuing, other than to say that they are rare diseases with unmet medical need, with a focus on the liver and the central nervous system.

“For a startup company, we have to make sure our flagship product succeeds,” Payne said, noting that the liver

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disease program would likely be the company's first. "So we're going with a high-probability target, for sure."

Despite its interest in moving into the clinic on its own, Arcturus is open to strategic alliances with bigger companies interested in its technology, he added.

And while such deals were hard to come by in recent years amid concerns that RNAi might not live up to its potential — as evidenced by the high-profile departures of Pfizer and Roche from the space — Payne said that he is encouraged by the renewed interest in the technology.

"Every single person we talk to now ... from big pharmaceutical companies, venture [capital firms], and the private investment community, they're now doing their due diligence ... [on] the industry and the technology, and they're becoming believers again," he said.

Indeed, the past 12 months has seen a resurgence of interest and investment in RNAi.

In January, for example, startup Solstice Biologics raked in commitments for \$18 million in a Series A financing round to help

advance its novel RNAi delivery technology (*GSN 1/10/2013*).

Also in January, Gradalis, which has a number of RNAi candidates in the clinic, closed a \$24 million Series B round (*GSN 1/3/2013*).

And in early 2012, AuraSense Therapeutics, which is developing proprietary nanoparticles for delivering a variety of therapeutic payloads including siRNAs (*GSN 7/12/2012*), raised \$5.4 million in a Series B round.

More established RNAi companies have also seen their stock rise — literally and figuratively.

Alnylam Pharmaceuticals has recently signed deal with two big pharmas including The Medicines Company, which picked up the rights to Alnylam's hypercholesterolemia program in February (*GSN 2/7/2013*), and Genzyme, which took the Asian rights to Alnylam's TTR-mediated amyloidosis program in late 2012 (*GSN 10/25/2012*).

Amid these deals and the recent announcements of positive clinical and preclinical data, Alnylam's stock is trading around a 52-week high of \$30.98.