

Startup Dicerna Will Use \$13M in Venture Capital to Enter RNAi Therapeutics Field

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By Doug Macron

Dicerna Pharmaceuticals, a Boston-based startup developing RNAi therapeutics based on City of Hope's Dicer-substrate technology, will close a \$13 million financing round next week that it expects will give it enough resources to operate through the filing of its first investigational new drug application, *RNAi News* has learned.

According to co-founder and CEO James Jenson, Dicerna has thus far been operating on an undisclosed amount of seed financing from venture capital firm Oxford Bioscience Partners, where chairman and co-founder Douglas Fambrough is a general partner.

But next week the company expects to close the Series A financing round, being led by Oxford, which will generate enough capital to fund the company for at least two years, during which time it anticipates filing its first IND, Jenson said.

Dicerna is still in the process of fleshing out its pipeline, so details of the planned IND remain unclear, but Jenson said that a decision on the company's first indications will be made shortly.

"We're doing a broad-ranging assessment of target opportunities," weighing factors such as target feasibility and validation, commercial opportunities, competition, and the potential for successful delivery, he said. "We're in the midst of that program now and expect to have our initial targets selected within a month."

Once it closes its \$13 million Series A, which Jenson noted will likely include some follow-on financing of a lesser amount, Dicerna will also begin ramping up its operations in earnest.

This includes the hiring of a senior vice president of research to round out its management team, which includes former Sirna Therapeutics CMO Roberto Gueriolini as a co-founder and senior vice president of pharmaceutical development, and about 20 scientific staffers.

"We'll have initially two dozen people in the company focused significantly on the biology and on the *in vivo* pharmacology" of Dicer-substrates, Jenson explained. "That's the core expertise we have to have in-house."

How Dicerna Discerned Dicer's Dominance Over 21-mer siRNAs

According to Dicerna CEO and co-founder James Jenson, the company grew out of discussions he had two years ago with Oxford Bioscience Partners official Douglas Fambrough, who is Dicerna's chairman, about starting an oncology drug company.

A few months later, Jenson met Mark Behlke, vice president of molecular genetics at Integrated DNA Technologies and co-inventor of the Dicer-substrates, with whom he began discussing RNAi and the therapeutic possibilities of the RNAi technology.

Dicer-substrates are 27-nucleotide long RNA duplexes that have been shown to be up to 100 times more effective at silencing genes than conventional 21 nucleotide-long siRNAs without inducing an interferon response or activating protein kinase R in cells.

Fambrough and Jenson "began thinking of this as an additional approach in our [planned] new company and gradually over time ... it occurred to [us] that this is a separate and equally powerful approach [as traditional siRNA] for entry into the RNAi field," Jenson said.

"So about a year ago, Doug and I decided [to] do a Dicer-substrate

Assisting Dicerna in establishing that core expertise will be scientific co-founders John Rossi, a researcher at COH, and Mark Behlke, vice president of molecular genetics at Integrated DNA Technologies, both of whom co-invented the Dicer-substrate technology (see *sidebar for further details on the company's background*).

company, and here we are," he said.

Dicerna also expects to conduct a limited amount of chemistry and formulation work internally, but expects that the bulk of this will be done through collaborations.

Specifically, Dicerna has contracted IDT to handle Dicer-substrate synthesis activities — a natural choice given the association with Behlke and IDT's growing interest in RNA manufacturing (see [RNAi News, 1/11/2007](#)).

As for drug formulation, Jenson said Dicerna is currently in talks with five "smaller," unnamed firms about possible collaborations.

"Our approach is to do feasibility studies ... to determine which [delivery technologies] work the best ... with a number of companies that are already out there doing formulation, and then to form collaborations with one or more of these companies on a target-by-target basis," he said. "We don't intend to build ourselves into a formulation company, but rather to work with those that are already up and running."

As for clinical development, Jensen said that he believes Dicerna is capable of carrying its first drug candidates up to phase II studies, at which point it would require a bigger partner to go further.

The Dicer-substrate technology is "a second doorway into the RNAi space."

"We think there is a lot of opportunity to do ... non-dilutive financing via partnerships with pharmaceutical companies," he said, given the opportunity in Dicerna's access to the Dicer-substrate technology.

"This is an intellectual property-constrained field," Jenson said. "There are a lot of companies that would like access [to the RNAi drugs market] that currently do not have access." A deal with Dicerna, however, would offer a way in.

Many industry-watchers view Dicer-substrates as a possible means to operate within the RNAi drugs field without having to license intellectual property related to standard siRNA, which is primarily held by RNAi heavyweights Merck and Alnylam Pharmaceuticals.

"It's a second doorway into the RNAi space" that allows Dicerna and its future partners to go after "almost any target," Jenson said.

The technology is far from a secret in the RNAi field: IDT has been selling the 27-mers for research applications for some time. And, as first reported by *RNAi News*, Natestch Pharmaceutical this time last year acquired the exclusive therapeutic rights to the technology for five undisclosed targets and non-exclusive rights for all other mammalian targets (see [RNAi News, 11/9/2006](#)).

Dicerna's license gives it exclusive, worldwide rights to the Dicer-substrate technology for all targets not exclusively licensed to Natestch. Jenson noted that his firm's license does not affect Natestch's non-exclusive access to the technology.

As a result, "there will be no other licenses of this technology" for therapeutic applications, he said.

Jenson expects that Dicerna will approach alliances on a target-by-target basis, even though a broader arrangement might be more valuable in the near term.

"We want to build a great company here," he said. "But at the same time, we don't want to deals that would be acquisition-threatening."