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Dicerna, Archemix Team Up to Make Souped Up RNAi Combo Drugs

Luke Timmerman, 7/21/09

Dicerna Pharmaceuticals has set its sights on silencing problematic genes using what it calls the “second doorway” of RNA interference—an approach to the burgeoning field that other RNAi companies aren’t pursuing. But to get its drugs over the transom and where they need to go in the body, the Watertown, MA-based company will need a little help, and for that it has turned to Cambridge, MA-based Archemix.

The two firms have partnered up to develop combination treatments that will merge Archemix’s proprietary “aptamer” treatments with Dicerna’s gene-silencing drugs. Both companies hope the alliance will yield drugs that can precisely—and potentially—home in on the molecular roots of disease. Specific financial terms of the deal aren’t being disclosed, although Dicerna and Archemix will split the early development costs, and Dicerna has an option to get exclusive rights to take the new drugs through development, leaving the usual milestone payments and product royalties to Archemix if the collaboration bears fruit.

The idea behind RNAi-based drug

development is to create specially engineered RNA molecules that selectively turn off disease-causing genes. Entrants to the field typically have to pay some sort of toll to Cambridge, MA-based Alnylam Pharmaceuticals (NASDAQ: ALNY), which has an extensive RNAi IP estate, but Dicerna insists it has found a “second doorway.” Simply put, it’s designing drugs that are a little longer than so-called small interfering RNA molecules being developed by Alnylam and others—and which are therefore not covered by Alnylam’s patents. Dicerna says its drugs may have the added advantage of being more potent than other RNAi-based treatments.

Importantly, these slightly longer RNAi drugs can be made with a handle on them that allows them to be welded together with other drug compounds that could give them extra kick. In this case, they hope to combine the Dicerna molecules with Archemix’s “aptamers”—short synthetic molecules designed to bind very specifically and tightly to certain protein targets.

“This provides a double-punch with one molecule,” says Jim Jenson, Dicerna’s CEO. “This deal is impor-



tant to Dicerna and to the RNAi field. This will change the game.”

There isn’t any proof yet such drugs will work in people, so these treatments have a long way to go and a lot of high hurdles to clear. Dicerna is also hedging its bets with a number of partners who bring expertise with antibody fragments and peptides, which also might be used to soup up its RNAi drugs. Dicerna already has established a couple of research collaborations (which it hasn’t disclosed) over the past six months, and has “several more discussions underway” that it hopes will lead to a partnership with a Big Pharma company this year, Jenson says.

“This is an area of great interest in the Big Pharma world,” Jenson says.

Archemix, for its part, gets to align itself with a glamorous niche within biotech and keep itself busy as an independent company, a little more than six months after it got dumped

at the altar by Lexington, MA-based NitroMed, which was looking to merge. Two months ago, Archemix named Kenneth Bate, the former NitroMed CEO, as its new top executive. This is the first significant R&D deal at Archemix since he came on board.

“This collaboration showcases how our proprietary aptamer technology can be used in conjunction with other therapeutic modalities and we look forward to beginning this exciting work with Dicerna,” Bate said in a statement.

Dicerna wasn’t about to say when it will enter clinical trials with one of

these new RNAi-aptamer drugs, although it is most interested in treatments for cancer and metabolic diseases like diabetes, Jenson says. Dicerna, which closed the last bit of its \$21.4 million Series A venture round last July—right before the economy tanked—is still “doing well” with its cash balance, Jenson says. But even so, pushing new drugs through development will take more money. Dicerna plans to start raising more money before the end of this year, Jenson says. ■

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