



# Pachyonychia Congenita Project

15 March 2005

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We hope that making available the relevant information on Pachyonychia Congenita will be a means of furthering research to find effective therapies and a cure for PC.

# RNAi Therapeutics

rnaitherapeutics.blogspot.com follows the development of RNAi Therapeutics from bench to clinic. Comments on basic science issues as well as investment opportunities in this rapidly evolving field. From siRNAs to Dicer, from Alnylam to Silence Therapeutics- it's here.

THURSDAY, SEPTEMBER 23, 2010

## RXi Pharmaceuticals Continues String of Sensible RNAi Delivery Collaborations

After deciding to focus on the use of so-called 'self-delivering' siRNAs (sd-rxRNA) for dermatology and ophthalmology applications, RXi Pharmaceuticals has since entered into a number of relationships with companies to facilitate the uptake of sd-rxRNA in various organs.

The latest such technology [partnership with EyeGate Pharmaceuticals](#) for ocular direct RNAi applications follows another direct RNAi delivery [partnership in dermatology with TransDerm](#) and a [collaboration with Philips Electronics](#) for developing image-guided ultrasound-mediated siRNA delivery technologies. While the long-awaited and much-rumored deals that should bring in non-dilutive funding are still lacking, these steps promise the efficient development of RXi's RNAi Therapeutics platform by tapping into what appear to be companies with complementary know-how and technologies. If successful, the technologies developed as a result of these partnerships may well pave the way for these Big Pharma partnerships that RXi and its investors so crave.

A common theme of these collaborations is that the partner provides technologies that, through largely physical methods (electrical currents, ultrasound, microneedle injections etc), should allow for the co-localization of the sd-rxRNA with the target cells, after which RXi's chemistry kicks in to overcome the formidable cellular lipid membrane barrier and induce RNAi gene knockdown- all without the need for nanoparticle formulation.

The relationship with EyeGate also addresses a problem generally facing the development of ocular drugs, particularly those for back-of-the-eye disorders tied to severe vision loss: patient-friendly modes of drug administrations. While the rapidly increasing genetic insights into eye diseases has yielded a number of excellent gene targets, there is considerable doubt whether drugs such as the monoclonal antibody Lucentis for wet age-related macular degeneration are viable in the long run as they need to be injected into the eye by needle every 1-3 months with each administration carrying with it an incremental risk of serious adverse events such as retinal detachments.

One way of addressing this problem is through the development of sustained-release formulations where a depot of drug is placed in the eye which releases drug over time. Another promising approach are gene therapies which may need to be administered only once or twice in a life-time, and may even offer the prospect for real cures. A third approach, the one taken by EyeGate, is to use iontophoresis whereby low electrical currents are applied to generate ions that repulse like-charged drug molecules, appropriately formulated, and push them across the outer sclera to be distributed throughout the eye. Because this technique does not involve needle injections and instead works by placing electrodes on top of the eye, it should be relatively patient friendly. EyeGate is currently in late-stage development of a drug candidate delivered by its proprietary iontophoresis technology.

It is, of course, worth keeping in mind that such collaborations will take time before we will see resulting technologies move into the clinic. Nevertheless, one thing is clear: RXi Pharmaceuticals is highly competitive in attracting qualified partners in the area of conjugate/self-delivering siRNA Therapeutics development. Probably the result of focusing their efforts on one segment of RNAi Therapeutics technology instead of trying to dominate them all. It is now time, however, to leverage the sd-rxRNA platform for

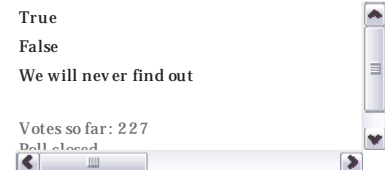
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[Avanir insider trading bear raid just before drug approval](#)

### NOVARTIS WILL MAKE TEKmira DELIVERY DEAL WITHIN 6 MONTHS



### NOVARTIS IS SILENCE THERAPEUTIS' MYSTERY SUITOR



### RNAI PORTFOLIO (START 1/6/08)

- Tekmira \$2790 +59.6%
- RXi Pharma \$438 +0.0%
- ISIS \$735 -41.3%
- Alnylam \$1371 -51.2%
- Silence Therapeutics \$602 -57.7%
- Value (8/25/10): \$5,936 -40.6%

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that non-dilutive funding.

Posted by Dirk Haussecker at Thursday, September 23, 2010 

Labels: [EyeGate Pharma](#), [iontophoresis](#), [ophthalmology](#), [RXi Pharmaceuticals](#), [sd-rxRNA](#)

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### WORLD OF RNAI THERAPEUTICS



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- [GSK to enter gene therapies as part of rare disease effort](#)
- [Invitrogen de-emphasizes shRNAs, Sigma not so](#)
- [Tekmira awarded major NIH Ebola/Marburg grant](#)
- [New phase 0/1 RNAi Candidate on ClinicalTrials](#)
- [Benitec re-issued fundamental Graham patent in US](#)

### GOOGLE NEWS: RNAI

- [RXi Pharmaceuticals Awarded \\$1 Million in IRS Grants to Advance RNAi Therapeutics - MarketWatch \(press release\)](#)
- [Dicerna Pharmaceuticals to Present at Upcoming Conferences in November - Benzinga](#)
- [Genetic switch that turns immune responses on and off discovered - Sify](#)
- [Marina Biotech Awarded \\$733000 in Grants for Qualifying Therapeutic ... -](#)

MarketWatch (press release)

- [Alnylam Scientists to Present New Data on RNAi Therapeutics at the 61st Annual ... - MarketWatch \(press release\)](#)

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