

US Patent Office affirms 'Zamore Design Rule' patents

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The United States Patent and Trademark Office has reaffirmed the validity of four important patents in the field of RNA therapeutics.

The affirmation validates the inventions known as the "Zamore Design Rules," compositions and methods of designing double stranded RNAi agents having decreased off-target silencing activity through certain structural modifications. The discoveries underlying these inventions were made by Phillip D. Zamore, PhD, a Howard Hughes Medical Institute Investigator, co-director of the RNA Therapeutics Institute and the Gretchen Stone Cook Professor of Biomedical Sciences and professor of biochemistry and molecular pharmacology at the University of Massachusetts Medical School in Worcester. This portfolio is currently licensed to Silence Therapeutics. The four patents are: US 7,459,547, US 7,732,593, US 7,772,203 and US 7,750,144.

Silence holds exclusive licenses to the "Zamore Design Rule" patent families in the human health care field from the University of Massachusetts Medical School. These patent families disclose various efficacy-enhancing methods and structural elements for RNAi therapeutics.

The ability to minimize the off-target effects of RNAi therapeutics is critical for limiting unwanted <u>cellular activity</u> and/or potential safety concerns. The decisively reaffirmed patents cover reducing off-target gene expression silencing using short interfering RNA (siRNA) and specific claims directed to microRNA (miRNA).



"We are very pleased that the USPTO has reissued these four patents," said Thomas Christély, Chief Executive Officer of Silence Therapeutics. "This outcome sends a clear message regarding the strength of Silence's intellectual property. We believe that there is significant value in the Zamore technology as a fundamental tool for the development and commercialization of RNAi therapeutics with enhanced efficacy and will continue to work to translate this value into our own RNAi therapeutic pipeline."

The "Zamore Design Rules" describe methods of enhancing the ability of an antisense strand of an RNAi agent to act as a guide strand; codify RNAi and siRNA agents for enhancing silencing of a target mRNA in an organism; and cover the composition of siRNA duplexes, pre-miRNA and shRNA in the process of RNA interference. The rules allow scientists to determine how effective and specific the RNA interference process will be in a given application, and are fundamental to the creation of RNA –based therapeutics.

"The initial granting of these patents was a significant milestone in the translation of the basic science of RNA interference into the real world of RNA therapeutics," said Dr. Zamore. "The unequivocal affirmation of the intellectual basis of these discoveries by the US Patent Office clears up any misapprehension about the origins of these discoveries, and we are excited that laboratories and research organizations around the world have had the benefit of using this science to work toward therapeutics to treat human disease."

Provided by University of Massachusetts Medical School

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