BIOWORLD™ TODAY
THE DAILY BIOPHARMACEUTICAL NEWS SOURCE
BIOTECH'S MOST RESPECTED NEWS SOURCE FOR MORE THAN 20 YEARS
FEBRUARY 28, 2017
VOLUME 28, NO. 39
SPECIAL REPRINT

Gilman-led Arrakis lands $38M series A round

By Michael Fitzhugh, Staff Writer

Arrakis Therapeutics Inc., a company seeking to develop a pipeline of RNA-targeted small molecules for neurology, oncology and rare genetic disorders, has completed a $38 million series A financing. The funds are expected to help it establish its RNA-focused drug discovery platform this year and then bring its most advanced program close to being ready for studies to back its first investigational new drug application.

It already has hits in its first screens and is pursuing medicinal chemistry work to move them forward, biotech veteran and Arrakis CEO Michael Gilman told *BioWorld Today*.

"There is a ton of biology represented by RNA that we've not yet been able to access with small-molecule drugs," Gilman said.

Canaan Partners led the round with participation by Advent Life Sciences, Pfizer Inc., Celgene Corp., Osage University Partners and Henri Termeer.

The types of proteins that are generally druggable by small molecules are either enzymes or receptors that already have binding pockets that can be targeted and occupied by small chemical compounds, Gilman explained.

RNAs, on the other hand, have a much more dynamic structure. While that hasn't precluded small molecules from binding to RNA, it has meant that finding such molecules hasn't been a focus for drug developers to date. When small-molecule medicines have been identified as acting via RNA binding, that insight has generally come in the wake of functional screening, not ahead of it.

Where Arrakis will be different is in its deployment of two proprietary platforms, TRYST and PEARL-seq, each of which fills in gaps in the traditional, but highly refined small-molecule drug discovery toolkit that need to be covered for RNA-focused work. TRYST is a high-throughput system of bioinformatics tools, assays and chemical libraries that help Arrakis' team identify new RNA targets. PEARL-seq, more of a lead optimization tool, focuses on evaluating candidates' binding functionality and selectivity inside cells.

Colleen Cuffaro, a principal on Canaan's health care team, said she finds Arrakis' approach compelling because it's "really addressing the limitations that exist with today's drug discovery toolkit." The small-molecule route the company has chosen also resonates, Cuffaro told *BioWorld Today*, because of its familiarity as a format in pharma. "When we think about complex therapeutic formats like cell therapies, gene therapies, gene editing and even RNA-targeted oligonucleotides, I think they're very interesting," she said. "But I have concerns about their commercial scalability."

Gilman is serving on Arrakis' executive team alongside its scientific founder and chief scientific officer, Russ Petter. Both men worked together at Biogen Inc. before moving on to other adventures, with Gilman going on to found and lead Padlock Therapeutics Inc. and Stromedix Inc. and Petter moving on to join Avila Therapeutics Inc., staying on through its acquisition by Celgene in 2012. (See *BioWorld Today*, Jan. 27, 2012.)

In "retirement," as Gilman tells it, Petter attended a chemistry conference where he was inspired by a couple of talks about the idea of drugging RNA with small molecules. While the idea wasn't new, Petter wondered how best one would go about executing it in an intentional, reproducible and efficient fashion.

Conversations between Petter and members of the team at Avila investor Advent Life Sciences uncovered an interest there in the same idea. Through Advent venture partner Alan Walts' connection, the circle grew to include former Genzyme chief Termeer, eventually leading Walts and Advent general partner Raj Parekh to invest in founding the company.

Gilman will dedicate half of his working time to fulfilling his executive role at Arrakis. He plans to spend the other half of his time helping to lead a soon-to-be financed Atlas venture company focused on gene and cell therapy.