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Arrakis Therapeutics Announces $38M Series A Financing To Target RNA with Small-Molecule Medicines And Names Michael Gilman Chairman and CEO

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Veteran management team to re-architect small molecule drug discovery to pursue new RNA targets within vast human transcriptome

Waltham, Mass., February 27, 2017 – Arrakis Therapeutics, a pioneering biopharmaceutical company, has completed a $38 million Series A financing led by Canaan Partners with participation by Advent Life Sciences, Pfizer Inc., Celgene Corporation, Osage University Partners, and biotech industry leader Henri Termeer. The company has also named serial entrepreneur and biotech leader Michael Gilman, Ph.D., as Chairman and Chief Executive Officer.

The proceeds from the Series A financing will be used to advance Arrakis' proprietary discovery platforms, comprised of a high-throughput, comprehensive system of bioinformatics tools, assays and chemical libraries that can identify new ribonucleic acid (RNA) targets and create new small-molecule drugs. Using its TRYST™ and PEARL-seq™ platforms, Arrakis is developing an internal product pipeline of RNA-targeted small molecules focused on neurology, oncology, and rare genetic disorders. The basis of Arrakis' unique approach is a proprietary chemical biology technology that interrogates structures of folded RNAs in their native state, within cells, to enable discovery and rational design of potent, selective small-molecule inhibitors of RNA function.

"RNA is the locus of most human biology, yet our current pharmacopeia is largely limited to protein targets," said Dr. Gilman. "Arrakis intends to re-architect small molecule drug discovery by redirecting, modifying, and creating tools that enable medicinal chemistry to directly address therapeuticallyimportant RNA molecules. Our substantial intellectual property, financial resources, and the extensive experience of our team in discovering and developing novel drugs will enable us to build a pipeline of new small-molecule therapeutics for patients not helped by today's medicines."

Targeting RNA with Small Molecules

The transcriptome encompasses all of the RNA transcribed from our genetic blueprint, including coding RNAs, which are translated into proteins, and noncoding RNAs, which regulate the fate and function of both coding RNAs and proteins. There are over 200,000 RNA transcripts in the human transcriptome - a vast pool of potential therapeutic targets. Yet today's small-molecule medicines address approximately 500 protein targets in a small number of structural classes. Accessing the biology embodied by the rest of the transcriptome has required the development of new therapeutic modalities, such as oligonucleotide drugs and gene therapies, which are at the early stages of their clinical evolution. Arrakis intends to open RNA biology to medicines with the

attractive and predictable pharmaceutical properties of small-molecule drugs, including oral bioavailability, broad biodistribution, and efficient cell penetration.

“The importance of targeting RNA in disease is well recognized,” said Russell C. Petter, Ph.D., founder and CSO of Arrakis Therapeutics. “But until now, targeting RNA with small-molecule compounds has been thought to be too challenging. Nevertheless, there are a number of precedents for conventional small molecules that bind and modulate RNA, including several approved drugs. These molecules were all discovered in functional screens, and only later did we learn that they acted on RNA. Our goal is to intentionally discover RNA-modulating small-molecule medicines. We believe that new developments in informatics, structural biology, and biophysical tools now make that possible.”

Colleen Cuffaro, Ph.D., principal at Canaan, who is joining the Arrakis board said, “Arrakis is pursuing a fundamentally different approach to RNA targets. For decades, the pharmaceutical industry has invested billions of dollars to develop tools and techniques to create drugs for protein targets. Arrakis is building on this toolkit to create a platform to discover RNA-targeted drugs. In doing so, I believe Arrakis has the potential to create an entirely new class of medicines for patients with unmet need.”

Industry-leading Experts on Management Team and Board

Arrakis has assembled an executive team and board of directors comprised of industry veterans with established records of building successful biopharmaceutical companies.

The Arrakis senior management team is comprised of the following:

- Michael Gilman, Ph.D., CEO and chairman of Arrakis; former founding CEO of Padlock Therapeutics and Stromedix, as well as executive roles at Biogen and ARIAD Pharmaceuticals
- Russell C. Petter, Ph.D., founder and chief scientific officer of Arrakis; former executive roles at Celgene, Avila, Mersana, Biogen, and Sandoz/Novartis
- Daniel Koerwer, chief business officer of Arrakis; former executive roles at BIND and Biogen
- James Barsoum, Ph.D., senior vice president of biology of Arrakis; former executive roles at RANA, Synta, and Biogen

Raj Parekh, Ph.D., founder of Arrakis and general partner at Advent Life Sciences said, “Our investor syndicate is a collaborative team committed to supporting Arrakis’ pioneering approach, and providing the company the resources needed to bring transformative new drugs to patients.”

The Arrakis board of directors is comprised of the following:

- Michael Gilman, Ph.D., CEO and chairman of Arrakis Therapeutics
- Colleen Cuffaro, Ph.D., principal at Canaan Partners
- Carolyn Green, executive director of strategic investments, External R&D Innovation, Pfizer Worldwide Research & Development
- Raj Parekh, Ph.D., founder of Arrakis Therapeutics and general partner at Advent Life Sciences
- Alan Walts, Ph.D., founder of Arrakis Therapeutics and venture partner at Advent Life Sciences

Observers on the Arrakis board of directors are Tim Shannon, M.D., general partner at Canaan Partners; Elaine Jones, Ph.D., VP of Pfizer Ventures; Peter Worland, Ph.D., VP Integrative Research Development at Celgene; and Matthew Cohen, Ph.D., principal at Osage University Partners.

About Arrakis Therapeutics

Arrakis Therapeutics is a biopharmaceutical company pioneering the discovery of a new class of medicines that directly target RNA. The company's TRYST™ and PEARL-seq™ platforms identify new RNA targets and drug candidates to treat diseases unaddressed by today's medicines. Arrakis is developing a proprietary pipeline of RNA-targeted small molecules focused on neurologic diseases, cancer, and rare genetic diseases.

Arrakis was founded in 2015 by Russell Petter, Ph.D., Alan Walts, Ph.D., Henri Termeer and Raj Parekh, Ph.D. with a vision to create a proprietary, transformational discovery platform that identifies small-molecule drugs that act directly on disease-causing RNA. The company was established with seed funding provided by Advent Life Sciences and Henri Termeer. The company brings together scientific leaders in RNA structure, chemistry and biology, along with a highly experienced management team and the backing of leading life sciences investors. The company is located in Waltham, Mass. Please visit www.arrakistx.com.

About Advent Life Sciences

Advent Life Sciences founds and invests in early- and mid-stage life sciences companies that have a firstor best-in-class approach to unmet medical needs. The investing team consists of experienced professionals, each with extensive scientific, medical and operational experience, a long-standing record of entrepreneurial and investment success in the US and Europe, and is particularly focused on supporting entrepreneurs and founders to take innovative new medical entities from concept to approval. The firm invests in a range of sectors within life sciences, principally drug discovery, enabling technologies and med tech, always with an emphasis on innovative, paradigm-changing approaches. Advent Life Sciences has a presence in the UK, US and France. For more information, please visit AdventLS.com.

About Canaan Partners

Canaan Partners is an early stage venture capital firm that invests in entrepreneurs with visionary ideas. With $4.2 billion under management, a diversified fund and 190 exits to date, Canaan has invested in some of the world's leading technology and health care companies over the past 30 years. Canaan's focus areas include fin tech, enterprise/SaaS, marketplace, e-commerce, biopharma, digital health and medtech. To learn more about our people and our portfolio, please visit canaan.com.

About Pfizer Inc.

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products. Our global portfolio includes medicines and vaccines as well as many of the world's best-known consumer health care products. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, Pfizer has worked to make a difference for all who rely on us. For more information, please visit us at www.pfizer.com.

About Celgene Corporation

Celgene Corporation, headquartered in Summit, New Jersey, is an integrated global biopharmaceutical company engaged primarily in the discovery, development and commercialization of innovative therapies for the treatment of cancer and inflammatory diseases through next-generation solutions in protein homeostasis, immuno-oncology, epigenetics, immunology and neuro-inflammation. For more information, please visit www.celgene.com.

About Osage University Partners

Osage University Partners is a venture capital firm focused on investing in startups that are commercializing pioneering university technologies. Osage partners with top research universities to invest in their most innovative startups, and Osage shares its investment profit with its partner institutions. The firm invests in software, hardware, and life science companies at all stages of company development. Osage has partnered with over 80 universities, including 36 of the top 50 U.S. institutions by research expenditures, and has invested in over 60 of their spinouts. Osage University Partners is part of a family of investment funds within Osage Partners, which is based in Philadelphia, PA and manages in excess of $500 million. For more information, visit www.osageuniversitypartners.com.

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