【演題５】

A Structure-based Approach to RNA-targeted Small Molecules.

Arrakis Therapeutics
Founder & Chief Innovation Officer
Jennifer C. Petter

勤務先： 828 Winter Street Waltham, MA 02451

学歴・職歴：

2015-now Founder & CIO, Arrakis Therapeutics (Employee 1)

Founder and CSO of Arrakis Therapeutics, a new start-up pursuing the discovery and development of drugs targeting the structure and function of folded RNAs. Built the original management and core scientific teams. Defined and articulated the vision and strategy for the company. Secured the seed funding ($3M) and worked with the CEO and CBO to secure the A ($38M) and B ($75M) rounds of venture funding as well as a multi-target collaboration w/ Roche ($190M upfront).

2012-2015 Vice President of Chemistry, Celgene

Oversaw all aspects of drug discovery chemistry at the San Diego, CA and Bedford, MA (now Cambridge) sites. In addition, oversaw the biochemistry, biology, and pharmacology groups in the Bedford, MA site.

2007-2012 Vice President of Drug Discovery, Avila Therapeutics, Inc. (Employee 10)

Oversaw all aspects of chemistry and drug discovery, including drug design, medicinal chemistry, process research, and CMC. Avila's platform technology focused on the structure-based design of covalent inhibitors with anti-cancer, anti-viral, and autoimmune applications.

2006-2007 Vice President of Research, Mersana Therapeutics, Inc. (Employee 10)

Oversaw all aspects of research and early drug development. Mersana focused on water-soluble, polymeric pro-drugs of anti-tumor agents designed to widen therapeutic window and improve efficacy.

2004-2005 Director of Small Molecule Drug Discovery, Biogen Idec, Inc.

Oversaw 50+ scientists engaged in medicinal chemistry, automated parallel synthesis, total chemical synthesis of proteins, process chemistry, cellular and biochemical assay development, medium-throughput screening, and the cloning, expression, and

purification of protein reagents for assays.

2000-2004 Director of Medicinal Chemistry, Biogen Idec, Inc.
Research Topics: integrins, GPCRs, kinases, and other CNS, oncology & immunology targets.

1996-2000 Section Head, Medicinal Chemistry
Dept. of Drug Discovery and Evaluation, Biogen, Inc.
Research Topics: cell adhesion molecules; adenosine receptors.

1995-1996 Fellow, Head of Chemistry, Oncology/Adhesion Biology Dept.
Sandoz Research Institute (now Novartis)
Research Topics: cell adhesion molecules, angiogenesis.

1993-1994 Senior Associate Fellow
Head of Chemistry, Receptor Mechanisms Department
Sandoz Research Institute (now Novartis)
Research Topics: cell adhesion molecules, drugs for treating sepsis.

1991-1993 Associate Fellow
Atherosclerosis and Cardiovascular Diseases
Sandoz Research Institute (now Novartis)
Research Topics: inhibition of squalene synthase and later Section Head for development of new antisense conjugates.

1984-1991 Assistant Professor
Department of Chemistry, University of Pittsburgh

Bio Sketch：

Dr. Jennifer Petter is the Founder and Chief Innovation Officer of Arrakis Therapeutics. Previously she was Vice President of Chemistry at Celgene, Vice President of Drug Discovery at Avila Therapeutics, Vice President of Research at Mersana Therapeutics, Director of Small Molecule Drug Discovery at Biogen, Section Head in Oncology Chemistry at Sandoz/Novartis, and Assistant Professor of Chemistry at the University of Pittsburgh. Dr. Petter graduated from Dartmouth College with an AB in chemistry, earned her PhD in organic chemistry at Duke University with Ned Porter, and was a post-doctoral fellow in Ron Breslow's group at Columbia University. She has ushered multiple compounds into the clinic for the treatment of cancer, cardiovascular disease, autoimmune disorders, and sepsis.

Arrakis Therapeutics is a venture-backed start-up in the Boston area devoted to the discovery and development of small-molecule drugs that bind to and modulate the functions of RNAs.

A Structure-based Approach to RNA-targeted Small Molecules

Jennifer C Petter

Arrakis Therapeutics, 828 Winter St, Waltham, MA 01775 USA

RNA offers a broad array of folded, three-dimensional structures that mediate or regulate the functional roles played by those RNAs. Our drug discovery platform at Arrakis Therapeutics is directed at the intervention of those functions to therapeutic benefit using drug-like small molecules that bind folded, RNA structures. Specifically, we have focused on binding to and modulating the function of structures in pre-mRNA and mRNA that govern splicing and translation, respectively.

The construction of this broad discovery platform presents many unique challenges: characterization of endogenous RNA structures, sub-target selection and validation, high-throughput screening, assessment of target engagement, new cellular assays, and demonstration of on-target mechanism. This presentation will touch on some of these challenges, describing novel methods and provide early data on specific RNA targets. Particular attention will be given to methods for the demonstration of target engagement.[1]

REFERENCES

1. Mukherjee, H., Blain, J. C., Vandivier, L. E., Chin, D. N., Friedman, J. E., Liu, F., Maillet, A., Fang, C., Kaplan, J. B., Li, J., Chenoweth, D. M., Christensen, A. B., Petersen, L. K., Vest Hansen, N. J., Barrera, L., Kubica, N., Kumaravel, G., and Petter, J. C.* *ACS Chem. Biol.* 2020, *15*, 2374-2381.