LigamiR Therapeutics

Company Overview

LigamiR Therapeutics is a newly formed small privately held biotech start-up company working to advance microRNAs as anti-cancer agents, with a major focus on delivery and stability of the RNAs. It is co-founded by Dr. Andrea Kasinski, Associate Professor at Purdue University and Deputy Director of the Purdue Institute for Cancer Research and Dr. Frank Slack, Professor of Pathology and Director of the Institute for RNA Medicine at Beth Israel Deaconess Medical Center (BIDMC). Drs. Kasinski and Slack have been collaborating in the microRNA therapeutics field for over 10 years. To support the effort of the company, the company plans to license intellectual property generated by Dr. Kasinski in her laboratory at Purdue. These patents include a full patent, PCT/US2017/061997 titled "Ligand Ionophore conjugates," as well as a pending international application under application number PCT/US24/21171 titled "Fully modified miR-34a and related conjugates, compositions and methods of use".

Problem or Market Opportunity

The projected market size for patients that would be candidates for FM-FolamiR-34a therapy would be ~115,840 – 168,129 patients per year. Our initial indications are NSCLC and TNBC, but we have the ability to easily expand into ovarian cancer once data are acquired. Our commercialization strategy includes i) R&D, which is mainly focused on de-risking our lead agent with seed funding form Purdue, the NIH (STTR submitted), or investors, ii) drug development which includes identifying a CMO-GMP facility, conducing pre-clinical toxicity, ADME, large animal GLP-compliant toxicity analysis, and setting up a pre-IND meeting with the FDA, and iii) corporate tasks which include licensing patents from Purdue, identifying a CEO/CSO/CTO, hiring a scientific team, leasing laboratory space, and securing series A funding.

Technical & Competitive Advantage

Cancer therapeutics are either pleiotropic leading to unmanageable toxicity or are directed to a single target that leads to resistance. To eradicate cancer requires an agent that can target multiple relevant pathways and to do so in the absence of unwanted toxic side effects. Our agent, FM-FolamiR-34a is the first agent of its kind, an agent that overcomes these challenges. The technical advantage includes our IP on the first fully modified microRNA that targets multiple relevant genes. Full modification leads to sustained activity in the absence of toxicity. The RNA is targeted to cancer cells using a high affinity ligand that binds specifically and robustly to a receptor that is over expressed on tumor cells, while avoiding non-tumorigenic tissues. RNA therapeutics is on the same trajectory as the antibody therapeutics field, a multi-billion dollar/year industry. LigamiR Therapeutics is a foundational company in this novel sector.

Regulatory Strategy & Intellectual Property

Our planned regulatory strategy includes early engagement with the FDA to be certain we are in compliance and are navigating our risk management appropriately. We have benchmarked this meeting for the last quarter of 2024 or first quarter of 2025, which we feel is appropriate based on our current R&D plan. We also have plans to interview/meet with GMO-GMP manufacturing facilities. Based on our timeline, we our optimistic about IND filing in the last quarter for 2025. Dr. Kasinski currently has IP at Purdue University that fully covers the lead compound. The most recent full application, submitted in March of 2024 covers the modification pattern of the microRNA and ligands used for delivery. LigamiR Therapeutics intends to license both patents from Purdue, likely in the 2nd or 3rd quarter of 2024.

Key Milestones

Q/YYYY	Objective	Milestone Description
Q3 2023	Proof of Concept	Demonstrated feasibility and efficacy of FM-FolamiR-34a, our lead agent. This work was published in 2023 in Oncogene. Efficacy was shown in a breast cancer xenograft model.
Q1 2024	IP Protection	Tech. developed is protected by a patent issued in 2017 to Dr. Kasinski/Purdue and a newly submitted application. The new application is the most relevant and was filed March 23, 2024.
Q1 2025	Pre-Clinical Dev.	Conduct extensive comparison studies to the original formulation of miR-34a (MRX34) which failed in clinical trial in 2013. We will also conduct pre-clinical toxicology and ADME analysis.

Capitalization History

Year	Grant or Equity Type	Description	Amount
2023		Purdue Institute for Drug Discovery, for conducting PDX studies at Charles River	$40K
2024		Purdue Trask Innovation Fund, for conducing efficacy and immune response studies in comparison to MRX34, the initial formulation of miR-34a that failed in clinical trial	$45K
2024	Grant	NIH STTR Phase I (pending review, July 2024)	$400K
2024	Seed Funding	Actively pitching our technology & new company, LigamiR Therapeutics. (Intend to raise)	$~1M

Current Round, Terms, and Use of Proceeds

To conduct proof-of-concept efficacy studies in NSCLC PDX models and pre-clinical toxicity analysis of FM-FolamiR-34a.

Key Team Members and Advisors

Andrea Kasinski, PhD | Co-Founder and CEO

Dr. Kasinski is an Associate Professor at Purdue University and Deputy Director of the Purdue Institute for Cancer Research (PICR). She has worked in the microRNA therapeutics field for 15 years and is recognized as one of the leading experts on the role of microRNAs in tumorigenesis and using microRNAs as a novel strategy for targeting cancer. She is also an expert in using ligands for delivery of tumor suppressive microRNAs. Her work was instrumental in transitioning the first microRNA, miR-34a into the clinic using NSCLC cell lines and multiple transgenic mouse models.

Frank J. Slack, PhD | Co-Founder and Chief Commercial Officer

Dr. Slack is the Director of the Harvard Medical School Initiative for RNA Medicine. He discovered the first human-encoded microRNA, let-7 and has been instrumental in uncovering the biological roles for many microRNAs, including miR-34a. Dr. Slack has founded multiple successful companies including MiraDx, 28/7 Therapeutics, Impilo Therapeutics, Redona Therapeutics, and others.