

EMERGING COMPANY PROFILE | REPRINT FROM MAR. 2, 2022

NextRNA: small molecules take on lncRNA-protein interactions

BY KAREN TKACH TUZMAN, SENIOR EDITOR



BIOCENTURY & GETTY IMAGES

Armed with platforms for lncRNA-based target discovery and small molecule design, NextRNA aims to drug interactions between the non-coding RNAs and the proteins through which they drive disease pathology.

“I really think this novel class of targets is so untapped,” co-founder, CSO and interim CEO Dominique Verhelle told BioCentury. Before starting NextRNA Therapeutics Inc., Verhelle was head of strategic academic alliances in the External Innovation Center at Takeda Pharmaceutical Co. Ltd. (Tokyo:4502; NYSE:TAK).

The company emerged from stealth Tuesday with \$9.3 million in seed financing and a \$46.8 million series A round led by Cobro Ventures and Lightchain Capital, with participation from Circle Alternative Investments, Evans Capital, Jefferies, Rivas Capital and Willett Advisors.

NextRNA is based on technology from the Dana-Farber Cancer Institute lab of co-founder Carl Novina. “Our lab has developed a suite of platform technologies to understand RNA function by looking at what proteins are binding. It’s a guilt-by-association approach,” Novina told BioCentury.

The company is focused on long non-coding RNAs (lncRNAs) — transcripts more than 200 nucleotides long that are not translated into proteins. “There’s clear evidence that when they’re dysregulated, they lead to disease,” Verhelle said.

NextRNA’s target discovery platform incorporates patient data, computational biology algorithms and high-throughput screens to identify disease-driving lncRNA-protein interactions.

“A lot of proteins bind lncRNAs. We have a way to figure out which are the most important for driving disease processes, with physiologically relevant functional readouts,” Novina said, adding that the platform also allows determination of the RNA motifs and protein domains critical for the interactions.

The company then uses its small molecule discovery platform to develop compounds that disrupt target lncRNA-protein interactions by binding either species.

“We can develop a small molecule that interacts with the RNA, but also one that interacts with the protein,” she said. “We’re

developing the capability for both, and feel we're unique in that sense."

Verhelle said recent improvements in biophysical and biochemical approaches have enabled better understanding of RNA structure, and RNA-targeted structure-based drug design. "We have developed expertise in this technology."

The funds will support development of two programs, one for oncology and another for immunology, plus initiation of two additional projects and validation of lncRNA targets newly discovered through the platform. NextRNA anticipates it will bring a project to lead optimization by 2H22.

Verhelle thinks there will be opportunities for lncRNA targets in a wide range of diseases. "We may go there on our own, or leverage additional therapeutic areas with partners."

At least one other company, Haya Therapeutics S.A., has disclosed a lncRNA-targeting platform. The Swiss biotech raised a CHF 18 million (\$20 million) seed round led by Broadview Ventures to develop therapeutics targeting lncRNAs, including an antisense oligonucleotide targeting the lncRNA WISPER to treat cardiac fibrosis.

Arrakis Therapeutics Inc., Expansion Therapeutics Inc. and Ribometrix Inc. each have preclinical RNA-targeting small molecule platforms, but have not disclosed a specific focus on lncRNA.

Companies delivering therapeutic versions of lncRNA, rather than using other modalities to target endogenous lncRNA, include Laronde Inc., whose platform is based on circularized forms of lncRNA, and Transine Therapeutics Ltd., which is developing therapeutic versions of SINEUPs, a type of lncRNA that increases translation of target mRNAs.

COMPANY PROFILE NEXTRNA THERAPEUTICS INC.

Cambridge, Mass.

Technology: Small molecules that disrupt the interaction between long non-coding RNAs and proteins

Origin of technology: Dana-Farber Cancer Institute

Disease focus: Cancer, autoimmune

Clinical status: Preclinical

Founded: 2021 by Carl Novina, Marc Cohen, Leon Wert-Lamas, Dominique Verhelle, Kenneth Anderson, Robert Langer

Academic collaborators: N/A

Corporate partners: N/A

Number of employees: 26

Funds raised: \$56 million

Investors: Cobro Ventures, Lightchain Capital, Circle Alternative Investments, Evans Capital, Jefferies, Rivas Capital, Willett Advisors

CEO: Dominique Verhelle

Patents: Undisclosed

BIOCENTURY

BIOCENTURY INC.

BioCentury's mission is to provide value-added business intelligence & analysis for life science companies, investors, academia and government on the strategic issues essential to the formation, development and sustainability of life science ventures.

NEWSROOM

news@biocentury.com

SAN CARLOS, CA

+1 650-595-5333; Fax: +1 650-595-5589

CHICAGO

+1 312-755-0798; Fax: +1 650-595-5589

WASHINGTON, DC

+1 202-462-9582; Fax: +1 202-667-2922

UNITED KINGDOM

+44 (0)1865-512184; Fax: +1 650-595-5589

BioCentury®; Because Real Intelligence is Hard to Find™; BCIQ™; The BioCentury 100™; and The Clear Route to ROI™ are trademarks of BIOCENTURY INC. All contents Copyright © 2021, BIOCENTURY INC. ALL RIGHTS RESERVED. No part of BioCentury's Publications or Website may be copied, reproduced, retransmitted, disseminated, sold, distributed, published, broadcast, circulated, commercially exploited in any form or used to create derivative works without the written consent of BioCentury. Information provided by BioCentury's Publications and Website is gathered from sources that BioCentury believes are reliable; however, BioCentury does not guarantee the accuracy, completeness, or timeliness of the information, nor does BioCentury make any warranties of any kind regarding the information. The contents of BioCentury's Publications and Website are not intended as investment, business, tax or legal advice, and BioCentury is not responsible for any investment, business, tax or legal opinions cited therein or for any decision made or action taken in reliance upon such information.

All use of BioCentury and its contents by current subscribers is governed by the BioCentury User Agreement and by all

others is governed by the BioCentury Terms of Use, unless a written agreement to the contrary has been executed by BioCentury Inc.

USE OF IMAGES: Certain Images used in BioCentury Inc.'s Publications, Video Content, Websites, Services, Notices and/or Marketing Materials are licensed from Getty Images (US), Inc. Any such image of a person or object so displayed is being used for illustrative purposes only and any such person or object depicted, if any, is merely a model. For more information see "Use of Images" found under the "Legal" section on the footer of the homepage at www.biocentury.com.

PRIVACY & ADVERTISING: In accordance with its Privacy Policy, BioCentury does NOT sell its customer information or usage data to third parties. BioCentury sells advertising in its BioCentury product emails and on its website. BioCentury is pleased to acknowledge its conference and events partners and sponsors through promotional announcements, on our web site, at our events and in our product emails. BioCentury also provides advertising opportunities to partners, sponsors, and advertisers through stand-alone emails to our community.