



# ProNAi Licenses Cancer Drug Candidate Targeting CDC7 from Carna Biosciences

- Small molecule kinase inhibitor AS-141 positioned for clinical trials in 2017 - CDC7 is an important regulator of DNA replication and DNA damage response -

**Vancouver, BC – May 26, 2016.** ProNAi Therapeutics, Inc. (NASDAQ: DNAI), a clinical-stage oncology company advancing novel targeted therapeutics for patients with cancer, today announced it has obtained an exclusive license from Carna Biosciences, Inc., Kobe, Japan (JASDAQ: 4572), for worldwide rights to develop and commercialize AS-141, a small molecule kinase inhibitor targeting CDC7.

"Our exclusive license with Carna gives us access to a highly promising asset that was created leveraging Carna's world-class kinase drug discovery expertise. Our team has extensive experience developing oncology drugs and will be focused on the rapid and efficient advancement of this drug candidate," said Dr. Nick Glover, President and CEO of ProNAi. "This agreement, and our focused efforts to identify additional high-quality assets to acquire, reflect our strategy of building a broad and diverse pipeline of targeted oncology drugs that will change people's lives."

Under the terms of the agreement, ProNAi will pay Carna Biosciences an initial upfront payment of \$0.9 million and aggregate additional potential payments upon achievement of certain developmental, regulatory and commercial milestones of up to \$270 million. ProNAi will also pay Carna single-digit tiered royalties on the net sales of any product successfully developed.

"ProNAi and Carna Biosciences are fully aligned in our vision of making a meaningful difference for patients with cancer," said Kohichiro Yoshino, PhD, Founder, President and CEO of Carna Biosciences. "We are confident the further development of AS-141 will be well-managed under the stewardship of the ProNAi team."

"We are very excited to work with the highly experienced team at ProNAi to progress AS-141 to the clinic," added Dr. Masaaki Sawa, Chief Scientific Officer of CarnaBio. "AS-141 has demonstrated compelling anti-tumor activity against multiple tumor types in preclinical studies and represents an opportunity for promising clinical development."

"CDC7's role as a key regulator of both DNA replication and DNA damage response make it a compelling emerging target for the treatment of a broad range of tumor types, providing significant commercial potential for the agent," said Dr. Angie You, Chief Business & Strategy Officer and Head of Commercial for ProNAi. "While there is growing interest in targets of this class, we believe we have an opportunity with this potent and selective kinase inhibitor to be first-in-class and highly differentiated."

"Preclinical data and published literature suggest a variety of oncology indications with potential for response to CDC7 inhibitors," added Dr. Barbara Klencke, ProNAi's Chief Development Officer. "Continued preclinical assessment of AS-141 will further inform our clinical development plans and patient selection strategies, with the objective of advancing this drug into the clinic in H2 2017.

#### About CDC7

CDC7 (cell division cycle 7) is a serine-threonine kinase positioned as an essential regulator of both DNA replication and DNA damage response, two critical functions required for tumor cell survival. Seminal discoveries related to DNA damage response have been recognized in the award of the 2015 Nobel Prize in Chemistry and the 2015 Albert Lasker Basic Medical Research Award, and have led to the discovery of potential new treatments for cancer.

Overexpression and activity of CDC7 is correlated with poor clinical outcomes and poor survival in a broad range of hematological malignancies and solid tumors. CDC7 inhibitors have been shown to trigger apoptosis (cancer cell death) in a p53-independent manner and to induce tumor stasis or regression in a variety of in vivo animal models.

### About Carna Biosciences

Carna Biosciences is a biopharmaceutical company focused on the discovery and development of kinase inhibitor drugs to treat serious unmet medical needs in oncology, autoimmune and inflammatory diseases, and neurological diseases by inhibiting kinases that are important drivers for those diseases. Carna Biosciences was founded in Kobe, Japan, in 2003 as a spinoff of Japan Organon (Nippon Organon KK). Carna's initial focus was to develop an extensive number of stateof-the-art, highest quality reagents for kinase drug discovery, and has since established a leading drug discovery program with a significant collection of proprietary chemical libraries. To date, the company has discovered a portfolio of preclinical stage compounds in relevant, multiple disease areas. Carna is a publicly traded company in the JASDAQ of the Tokyo Stock Exchange with securities code, 4572. For more information, please visit <u>www.carnabio.com</u>.

#### **About ProNAi Therapeutics**

ProNAi Therapeutics is a clinical-stage oncology company advancing novel targeted therapeutics for patients with cancer. ProNAi's lead product candidate, PNT2258, is designed to target cancers that overexpress BCL2, an important and validated oncogene known to be dysregulated in many types of cancer. ProNAi is evaluating PNT2258 in two Phase 2 trials: "Wolverine", a Phase 2 trial evaluating PNT2258 for the treatment of relapsed or refractory diffuse large B-cell lymphoma (DLBCL); and "Brighton", a Phase 2 trial evaluating PNT2258 for the treatment of Richter's transformation. For more information, please visit www.pronai.com.

#### **Cautionary Note on Forward-Looking Statements**

This press release contains forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding ProNAi's anticipated clinical development, expected benefits of its product candidates and business development strategies. All statements other than statements of historical fact are statements that could be deemed forward-looking statements. These statements are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and assumptions that could cause actual results to differ materially from those described in the forward-looking statements. Such forward-looking statements are subject to risks and uncertainties, including, among others, the risk that ProNAi may be unable to successfully develop and commercialize PNT2258, AS-141 or any future product candidates, PNT2258 and AS-141 may fail to demonstrate safety and efficacy or may not otherwise produce positive results, ProNAi may experience delays in clinical trials, including due to difficulties enrolling patients, ProNAi's third-party manufacturers may cause its supply of materials to become limited or interrupted or fail to be of satisfactory quantity or quality. ProNAi's cash resources may be insufficient to fund its current operating plans and it may be unable to raise additional capital when needed, ProNAi may be unable to obtain and enforce intellectual property protection for its technologies and product candidates and the other factors described under the heading "Risk Factors" set forth in ProNAi's filings with the Securities and Exchange Commission from time to time. ProNAi undertakes no obligation to update the forward-looking statements contained herein or to reflect events or circumstances occurring after the date hereof, other than as may be required by applicable law.

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