Alnylam Announces 2018 Corporate Goals

January 7, 2018

Patisiran Expected to be First Commercially Available RNAi Therapeutic in Mid-2018

RestructuredSanofi Alliance Provides Alnylam with Global Commercial Footprint for ATTR Amyloidosis

Givosiran Interim Phase 3 Data Expected in Mid-2018 and U.S. New Drug Application Filing Anticipated in Late 2018

Company Plans to Advance Three Additional Phase 3 Programs: Fitusiran, Inclisiran, and ALN-TTRsc02

Company Updates Guidance to End 2017 with Greater Than$1.7 Billion in Cash

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Jan. 7, 2018-- Alnylam Pharmaceuticals, Inc. (Nasdaq:ALNY), the leading RNAi therapeutics company, today announced its corporate goals for 2018. The goals focus on clinical and regulatory progress against its five mid- to late-stage programs, including patisiran, which is advancing toward potential global regulatory approvals and commercialization, paving the way for the Company to achieve its *Alnylam 2020* goals.

"In the year ahead, we look forward to the potential global commercial launch of patisiran, the first-ever RNAi therapeutic to enter the market, marking the birth of a whole new class of medicines with the potential to transform the treatment of diseases with high unmet need. Together with the advancements made in our other late-stage programs, we now look forward to our transition from a development-stage company toward a multi-product, global commercial-stage biopharmaceutical company with a deep and sustainable pipeline and research engine," said John Maraganore, Ph.D., Chief Executive Officer of Alnylam. “With continued diligence and investment in our pipeline and commercial execution we believe we are well positioned to realize our *Alnylam 2020* goals, making RNAi therapeutics broadly available to patients in need. Further, with our recently completed strategic restructuring of our Sanofi alliance, we are now poised to optimize our ATTR amyloidosis programs, patisiran and ALN-TTRsc02, for patients around the world.”

“The recently restructured alliance with Sanofi provides strategic and operational clarity and a global launching pad for patisiran and our entire ATTR amyloidosis business. Given the transformational potential of patisiran based on our recently completed APOLLO Phase 3 study, we’re excited to accelerate our build of capabilities to bring this investigational RNAi therapeutic to patients around the world,” said Barry Greene, President of Alnylam. “As part of a staged global effort, we are preparing for the patisiran launch in mid-2018 in the U.S. and late 2018 in Europe, and we will then focus on Japan and other rest-of-world markets.”

2018 Corporate Goals

Patisiran, an investigational RNAi therapeutic currently under regulatory review for the treatment of hATTR amyloidosis. Alnylam plans to:

- Present additional data from the APOLLO Phase 3 study in early, mid-, and late 2018;
- File for regulatory approval in Japan and other global markets, including a Japanese NDA filling with the Pharmaceuticals and Medical Device Agency (PMDA) in mid-2018;
- File for regulatory approvals in additional rest-of-world countries in late 2018;
- Gain regulatory approvals from the U.S. Food and Drug Administration and the European Medicines Agency in mid- and late 2018, respectively; and
- Assuming regulatory approval, launch patisiran in the U.S. and EU in mid- and late 2018, respectively.

Givosiran, an investigational RNAi therapeutic in development for the treatment of acute hepatic porphyrias (AHPs). Alnylam plans to:

- Present Phase 1/2 open-label extension (OLE) data in early 2018;
- Report topline interim results from the ENVISION Phase 3 trial in mid-2018;
- Assuming a positive interim readout, file for accelerated regulatory approval with submission of a New Drug Application (NDA) in the U.S. in late 2018; and
- Complete enrollment in the ENVISION Phase 3 study in late 2018.

ALN-TTRsc02, an investigational, subcutaneously administered RNAi therapeutic in development for the treatment of ATTR amyloidosis. Alnylam plans to:

- Initiate a Phase 3 pivotal study program in late 2018.

Alnylam also intends to support Sanofi’s efforts in advancing fitusiran, an investigational RNAi therapeutic in development for the treatment of hemophilia and rare bleeding disorders. Throughout 2018, Sanofi expects to enroll patients in the ATLAS Phase 3 program of fitusiran in patients with hemophilia A or B with and without inhibitors. If successful, fitusiran will be commercialized globally by Sanofi Genzyme, the specialty care global business unit of Sanofi. Alnylam is eligible to receive 15 to 30 percent royalties on net sales.

Alnylam also plans to support The Medicines Company's continued efforts with respect to the ORION Phase 3 studies of inclisiran – an investigational RNAi therapeutic targeting PCSK9 in development for the treatment of hypercholesterolemia – throughout 2018. Specifically, The Medicines Company

has guided to complete enrollment in the ORION 9, 10, and 11 LDL-C pivotal studies in early 2018 and to initiate enrollment in the ORION 4 cardiovascular outcomes (CVOT) study in mid-2018. Alnylam is eligible to receive milestones and royalties of up to 20 percent from The Medicines Company based on the successful development and commercialization of inclisiran.

In addition, the Company plans to continue advancement of its earlier-stage clinical pipeline programs with multiple data read-outs expected throughout 2018. Alnylam also plans to file one or more new clinical trial applications (CTA) in 2018, and advance its infectious disease collaboration with Vir Biotechnology.

The Company now expects to end 2017 with greater than $1.7 billion in cash, cash equivalents, marketable securities, and restricted cash and intends to provide financial guidance for 2018 in connection with its year-end 2017 financial results in February.

Alnylam management will present a company overview detailing these goals and guidance at the 36th Annual J.P. Morgan Healthcare Conference tomorrow, Monday, January 8, 2018 at 8:00 a.m. PT (11:00 a.m. ET) at the Westin St. Francis Hotel in San Francisco, California. In addition, the Company plans to webcast its Q&A breakout session immediately following the presentation at 8:30 a.m. PT (11:30 a.m. ET). A live audio webcast of the presentation and breakout session will be available on the Investors section of the Company's website, www.alnylam.com. A replay will be available on the Alnylam website within 48 hours after the event.

About RNAi

RNAi (RNA interference) is a natural cellular process of gene silencing that represents one of the most promising and rapidly advancing frontiers in biology and drug development today. Its discovery has been heralded as “a major scientific breakthrough that happens once every decade or so,” and was recognized with the award of the 2006 Nobel Prize for Physiology or Medicine. By harnessing the natural biological process of RNAi occurring in our cells, a major new class of medicines, known as RNAi therapeutics, is on the horizon. Small interfering RNA (siRNA), the molecules that mediate RNAi and comprise Alnylam's RNAi therapeutic platform, function upstream of today’s medicines by potently silencing messenger RNA (mRNA) – the genetic precursors – that encode for disease-causing proteins, thus preventing them from being made. This is a revolutionary approach with the potential to transform the care of patients with genetic and other diseases.

About Alnylam Pharmaceuticals, Inc.

Alnylam (Nasdaq:ALNY) is leading the translation of RNA interference (RNAi) into a whole new class of innovative medicines with the potential to transform the lives of people afflicted with rare genetic, cardio-metabolic, and hepatic infectious diseases. Based on Nobel Prize-winning science, RNAi therapeutics represent a powerful, clinically validated approach for the treatment of a wide range of severe and debilitating diseases. Founded in 2002, Alnylam is delivering on a bold vision to turn scientific possibility into reality, with a robust discovery platform and deep pipeline of investigational medicines, including four product candidates that are in late-stage development. Looking forward, Alnylam will continue to execute on its "Alnylam 2020" strategy of building a multi-product, commercial-stage biopharmaceutical company with a sustainable pipeline of RNAi-based medicines to address the needs of patients who have limited or inadequate treatment options. Alnylam employs over 700 people in the U.S. and Europe and is headquartered in Cambridge, MA. For more information about our people, science and pipeline, please visit www.alnylam.com and engage with us on Twitter at @Alnylam or on LinkedIn.

Alnylam Forward-Looking Statements

Various statements in this release concerning Alnylam's future expectations, plans and prospects, including, without limitation, Alnylam's views with respect to the potential for RNAi therapeutics, including patisiran, ALN-TTRsc02, givosiran, fitusiran, and inclisran, its expectations regarding the potential regulatory approval and commercial launch of patisiran in the United States and Europe and the expected timing of regulatory filings seeking approval for patisiran in Japan and other global markets, its expectations regarding the timing of clinical studies for givosiran, ALN-TTRsc02, fitusiran, and inclisiran and expected regulatory filings for givosiran, its expectations regarding the further development of fitusiran by its collaborator, Sanofi Genzyme, its plans for the development and commercialization of RNAi therapeutics for infectious diseases with Vir Biotechnology, its expected cash, cash equivalents, fixed income, marketable securities, and restricted investments position as of December 31, 2017, and expectations regarding its "Alnylam 2020" guidance for the advancement and commercialization of RNAi therapeutics, constitute forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995. Actual results and future plans may differ materially from those indicated by these forward-looking statements as a result of various important risks, uncertainties and other factors, including, without limitation, Alnylam's ability to discover and develop novel drug candidates and delivery approaches, successfully demonstrate the efficacy and safety of its product candidates, the pre-clinical and clinical results for its product candidates, which may not be replicated or continue to occur in other subjects or in additional studies or otherwise support further development of product candidates for a specified indication or at all, actions or advice of regulatory agencies, which may affect the design, initiation, timing, continuation and/or progress of clinical trials or result in the need for additional pre-clinical and/or clinical testing, delays, interruptions or failures in the manufacture and supply of its product candidates, obtaining, maintaining and protecting intellectual property, Alnylam's ability to enforce its intellectual property rights against third parties and defend its patent portfolio against challenges from third parties, obtaining and maintaining regulatory approval, pricing and reimbursement for products, progress in establishing a commercial and ex-United States infrastructure, competition from others using technology similar to Alnylam's and others developing products for similar uses, Alnylam's ability to manage its growth and operating expenses, obtain additional funding to support its business activities, and establish and maintain strategic business alliances and new business initiatives, Alnylam's dependence on third parties for development, manufacture and distribution of products, the outcome of litigation, the risk of government investigations, and unexpected expenditures, as well as those risks more fully discussed in the "Risk Factors" filed with Alnylam's most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) and in other filings that Alnylam makes with the SEC. In addition, any forward-looking statements represent Alnylam's views only as of today and should not be relied upon as representing its views as of any subsequent date. Alnylam explicitly disclaims any obligation, except to the extent required by law, to update any forward-looking statements.

None of Alnylam’s investigational therapeutics have been approved by the U.S. Food and Drug Administration, European Medicines Agency, or any other regulatory authority and no conclusions can or should be drawn regarding the safety or effectiveness of such investigational therapeutics.

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Source: Alnylam Pharmaceuticals, Inc.

Alnylam Pharmaceuticals, Inc.
Investors and Media
Christine Regan Lindenboom, 617-682-4340
or
Investors
Josh Brodsky, 617-551-8276