



Alnylam and Regeneron Announce Broad Collaboration to Discover, Develop and Commercialize RNAi Therapeutics Focused on Ocular and Central Nervous System (CNS) Diseases

April 8, 2019

– Companies to Also Jointly Advance Select Number of Preclinical Disease Programs with Targets Expressed in the Liver and Treatments for C5 Complement-Mediated Diseases –

– Regeneron to Invest \$800 million Through Upfront Cash and Equity Investment in Alnylam, with up to Additional \$200 Million in Potential Near-Term Milestones –

– Alnylam to Host Conference Call Today, Monday, April 8th at 8:30 am ET –

CAMBRIDGE, Mass. & TARRYTOWN, N.Y.--(BUSINESS WIRE)--Apr. 8, 2019-- [Alnylam Pharmaceuticals, Inc.](#) (Nasdaq:ALNY), the leading RNAi therapeutics company, and Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN), announced today a collaboration to discover, develop and commercialize new RNA interference (RNAi) therapeutics for a broad range of diseases by addressing disease targets expressed in the eye and central nervous system (CNS), in addition to a select number of targets expressed in the liver. The collaboration will leverage both companies' scientific and technological expertise and will build on Alnylam's recent preclinical data showing potent and highly durable delivery of RNAi therapeutics to achieve target gene silencing in the eye and CNS. The collaboration will also benefit from Regeneron's industry-leading *VelociSuite*[®] technologies and capabilities from the Regeneron Genetics Center (RGC).

Under the terms of the alliance, Alnylam will work exclusively with Regeneron to discover RNAi therapeutics for eye and CNS diseases. Regeneron will lead development and commercialization for all programs targeting eye diseases, with Alnylam entitled to potential milestone and royalty payments. The companies will jointly advance and alternate leadership on CNS programs, with the lead party retaining global development and commercial responsibility. For CNS programs, both companies will have the option at candidate selection to participate equally in potential future profits of programs led by the other party.

The collaboration also includes a select number of RNAi therapeutic programs designed to target genes expressed in the liver, which can influence a wide variety of diseases throughout the body. These programs include a planned joint effort evaluating the combination of Regeneron's pozelimab (REGN3918), currently in Phase 1 development, with Alnylam's cemdisiran, currently in Phase 2 development. Alnylam will retain control of cemdisiran monotherapy development, and Regeneron will lead combination development. The parties will equally share investment and potential future profits on the monotherapy program, and Alnylam will receive royalties on any potential combination product sales. For all other alliance liver programs, the parties will alternate leadership and participate equally in potential profits. The companies will continue their [previously-announced](#) collaboration to identify RNAi therapeutics for the chronic liver disease nonalcoholic steatohepatitis (NASH) based on novel RGC findings. Alnylam retains broad global rights to all of its other unpartnered liver-directed clinical and preclinical pipeline programs.

"At Regeneron we believe the best use of our resources is to invest in potentially game-changing science that will yield innovative medicines for patients with serious diseases. This collaboration couples proven and emerging RNAi technology, which holds important promise in many diseases, with Regeneron's world-leading genetics research and target discovery engine," said George D. Yancopoulos, M.D., Ph.D., President and Chief Scientific Officer of Regeneron. "This collaboration enables us to reach targets inside the cell complementing our expertise in antibodies, which are ideal for extracellular targets and those on the cell surface. Through the RGC and our other research groups, we are already identifying additional targets that may be well-suited for RNAi-based drug development, particularly in the eye and CNS."

"This new industry-leading alliance is aimed at realizing what we believe to be a significant opportunity for RNAi therapeutics as potentially transformative medicines for ocular and CNS diseases. We are thrilled to collaborate with Regeneron, a like-minded science-based organization, to significantly accelerate our efforts to bring RNAi therapeutics to patients," said John Maraganore, Ph.D., Chief Executive Officer of Alnylam. "Importantly, the alliance structure enables Alnylam to continue to build its industry-leading pipeline of RNAi therapeutics while retaining significant product rights. In addition, the near-term payments under this new agreement will strengthen Alnylam's balance sheet with over \$2 billion in *pro forma* cash upon closing of the transaction, supporting our global efforts to develop and commercialize multiple products as potentially breakthrough medicines and advance our profile toward sustainable profitability."

Regeneron has agreed to make a \$400 million upfront payment to Alnylam and to purchase \$400 million of Alnylam equity at a price per share of \$90.00 (4.44 million common shares), based on the volume-weighted average price over the last fifteen-trading-day period. Alnylam is eligible to receive up to an additional \$200 million in milestone payments upon achievement of certain criteria during early clinical development for the eye and CNS programs. The companies plan to advance programs directed to 30 targets and introduce many into clinical development during the initial five-year discovery period, which includes an option to extend. For each program, Regeneron will provide Alnylam with \$2.5 million in funding at program initiation and an additional \$2.5 million at lead candidate identification, translating to the potential for approximately \$30 million in annual discovery funding to Alnylam as the alliance reaches steady state. The alliance and equity-related agreements are subject to customary closing conditions and clearances, including clearance under the Hart-Scott Rodino Antitrust Improvements Act.

Alnylam, alongside multiple other leading life sciences companies, is also a member of Regeneron's pre-competitive consortium to sequence the DNA of 500,000 individuals in the UK Biobank health resource and subsequently make the data publicly available to the global research community.

Alnylam Conference Call Information

Alnylam Management will discuss this collaboration via conference call on Monday, April 8, 2019 at 8:30 am ET. A webcast presentation will also be available on the Investors page of the Company's website, www.alnylam.com. To access the call, please dial 800-289-0438 (domestic) or

323-794-2423 (international) five minutes prior to the start time and refer to conference ID 2197882. A replay of the call will be available beginning at 11:30 am ET on the day of the call. To access the replay, please dial 888-203-1112 (domestic) or 719-457-0820 (international) and refer to conference ID 2197882.

About Alnylam Pharmaceuticals

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, hepatic infectious, and central nervous system (CNS)/ocular 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. Alnylam's first U.S. FDA-approved RNAi therapeutic is ONPATTRO[®] (patisiran) lipid complex injection available in the U.S. for the treatment of the polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis in adults. In the EU, ONPATTRO is approved for the treatment of hATTR amyloidosis in adults with stage 1 or stage 2 polyneuropathy. Alnylam has a deep pipeline of investigational medicines, including five 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 1,000 people worldwide 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](https://twitter.com/Alnylam) or on [LinkedIn](https://www.linkedin.com/company/alnylam).

About Regeneron Pharmaceuticals, Inc.

Regeneron (NASDAQ: REGN) is a leading biotechnology company that invents life-transforming medicines for people with serious diseases. Founded and led for 30 years by physician-scientists, our unique ability to repeatedly and consistently translate science into medicine has led to seven FDA-approved treatments and numerous product candidates in development, all of which were homegrown in our laboratories. Our medicines and pipeline are designed to help patients with eye disease, heart disease, allergic and inflammatory diseases, pain, cancer, infectious diseases and rare diseases.

Regeneron is accelerating and improving the traditional drug development process through our proprietary *VelociSuite*[®] technologies, such as *VelocImmune*[®] which produces optimized fully-human antibodies, and ambitious research initiatives such as the Regeneron Genetics Center, which is conducting one of the largest genetics sequencing efforts in the world.

For additional information about the company, please visit www.regeneron.com or follow [@Regeneron](https://twitter.com/Regeneron) on Twitter.

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 of RNAi therapeutics to achieve target gene silencing in the CNS and eye, Regeneron's participation in the development and commercialization of RNAi therapeutics directed to CNS, eye and a select number of liver targets, as well as the planned joint effort evaluating an anti-C5 antibody-siRNA combination as well as a monotherapy approach, the parties plans to advance 30 targets and file multiple Investigational New Drug Applications during the discovery period, Alnylam's expectations regarding funding for each program under the collaboration at various stages of development, its expectations regarding the receipt of upfront cash and an equity investment, as well as potential development, regulatory and sales milestones and royalties from Regeneron, its expectations regarding available cash for its operations through multiple product launches, 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; Regeneron's ability to successfully advance and develop programs targeting eye diseases, resulting in the potential payment of milestones and royalties to Alnylam; the parties ability to successfully develop and commercialize CNS programs; 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, successfully launching, marketing and selling its approved products globally; Alnylam's ability to successfully expand the indication for ONPATTRO in the future; 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 Annual Report on Form 10-K 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.

Regeneron Forward-Looking Statements and Use of Digital Media

This press release includes forward-looking statements that involve risks and uncertainties relating to future events and the future performance of Regeneron Pharmaceuticals, Inc. ("Regeneron" or the "Company"), and actual events or results may differ materially from these forward-looking statements. Words such as "anticipate," "expect," "intend," "plan," "believe," "seek," "estimate," variations of such words, and similar expressions are intended to identify such forward-looking statements, although not all forward-looking statements contain these identifying words. These statements concern, and these risks and uncertainties include, among others, the nature, timing, and possible success and therapeutic applications of Regeneron's or its collaborators' products, product candidates, and research and clinical programs now underway or planned, such as the RNA interference programs discussed in this press release (including programs evaluating anti-C5 antibody-siRNA combinations for C5 complement-mediated diseases and RNAi therapeutics for the chronic liver disease nonalcoholic steatohepatitis); the extent to which the results from the research

and development programs conducted by Regeneron or its collaborators (including based on the collaboration discussed in this press release) may be replicated in other studies and lead to therapeutic applications; the potential for any license or collaboration agreement, including Regeneron's agreements with Sanofi, Bayer, and Teva Pharmaceutical Industries Ltd. (or their respective affiliated companies, as applicable), as well as Regeneron's collaborations with Alnylam Pharmaceuticals, Inc. discussed in this news release, to be cancelled or terminated without any product success; the likelihood, timing, and scope of possible regulatory approval and commercial launch of Regeneron's late-stage product candidates and new indications for marketed products; unforeseen safety issues resulting from the administration of products and product candidates in patients, including serious complications or side effects in connection with the use of Regeneron's or its collaborators' product candidates in clinical trials; ongoing regulatory obligations and oversight impacting Regeneron's marketed products, research and clinical programs, and business, including those relating to patient privacy; determinations by regulatory and administrative governmental authorities which may delay or restrict Regeneron's or its collaborators' ability to continue to develop or commercialize products and product candidates; competing drugs and product candidates that may be superior to Regeneron's or its collaborators' products and product candidates; uncertainty of market acceptance and commercial success of Regeneron's or its collaborators' products and product candidates and the impact of studies (whether conducted by Regeneron or others and whether mandated or voluntary) on the commercial success of Regeneron's or its collaborators' products and product candidates; the ability of Regeneron to manufacture and manage supply chains for multiple products and product candidates; the ability of Regeneron's collaborators, suppliers, or other third parties (as applicable) to perform manufacturing, filling, finishing, packaging, labeling, distribution, and other steps related to Regeneron's products and product candidates; the availability and extent of reimbursement of the Company's products from third-party payers, including private payer healthcare and insurance programs, health maintenance organizations, pharmacy benefit management companies, and government programs such as Medicare and Medicaid; coverage and reimbursement determinations by such payers and new policies and procedures adopted by such payers; unanticipated expenses; the costs of developing, producing, and selling products; the ability of Regeneron to meet any of its financial projections or guidance and changes to the assumptions underlying those projections or guidance; and risks associated with intellectual property of other parties and pending or future litigation relating thereto, including without limitation the patent litigation and other related proceedings relating to EYLEA® (afibercept) Injection, and Dupixent® (dupilumab) Injection, and Praluent® (alirocumab) Injection, the ultimate outcome of any such proceedings, and the impact any of the foregoing may have on Regeneron's business, prospects, operating results, and financial condition. A more complete description of these and other material risks can be found in Regeneron's filings with the U.S. Securities and Exchange Commission, including its Form 10-K for the year ended December 31, 2018. Any forward-looking statements are made based on management's current beliefs and judgment, and the reader is cautioned not to rely on any forward-looking statements made by Regeneron. Regeneron does not undertake any obligation to update publicly any forward-looking statement, including without limitation any financial projection or guidance, whether as a result of new information, future events, or otherwise.

Regeneron uses its media and investor relations website and social media outlets to publish important information about the Company, including information that may be deemed material to investors. Financial and other information about Regeneron is routinely posted and is accessible on Regeneron's media and investor relations website (<http://newsroom.regeneron.com>) and its Twitter feed (<http://twitter.com/regeneron>).

View source version on businesswire.com: <https://www.businesswire.com/news/home/20190408005387/en/>

Source: Alnylam Pharmaceuticals, Inc.

Alnylam Pharmaceuticals, Inc.

Christine Regan Lindenboom
(Investors and Media)
617-682-4340

Josh Brodsky
(Investors)
617-551-8276

Regeneron Investor Relations

Mark Hudson
Tel: +1 (914) 847-3482
Mark.hudson@regeneron.com

Regeneron Media Relations

Hala Mirza
Tel: +1 (914) 847-3422
Hala.mirza@regeneron.com