



Genzyme Optes into Alnylam's ALN-AT3 Hemophilia Program for Development and Commercialization Outside of North America and Western Europe

- Marks First Product Opt-In since Formation of Landmark 2014 Alliance for Global Advancement of RNAi Therapeutics as Genetic Medicines -

Paris, France and Cambridge, Mass. - October 1st, 2015 - [Sanofi](#) and its subsidiary [Genzyme](#) announced today that Genzyme has elected to opt into Alnylam's investigational ALN-AT3 hemophilia program for development and potential future commercialization in territories outside of North America and Western Europe. This marks the first product from Alnylam's Genetic Medicines pipeline to which Genzyme has opted in since the formation of the companies' global alliance in January 2014, and the third product opt-in overall. Genzyme's opt-in decision was based on encouraging clinical data from the Phase 1 trial of ALN-AT3, including [positive interim data](#) that were presented at the International Society on Thrombosis and Haemostasis (ISTH) 2015 Congress in June 2015.

"Our collaboration with Genzyme is a key part of our strategy to advance RNAi therapeutics to global markets. Genzyme's proven track record in developing and commercializing therapies for rare diseases makes them an ideal partner to advance an innovative medicine for the treatment of hemophilia. Accordingly, we are very pleased that they have elected to opt into the ALN-AT3 program, the first product opt-in since formation of our landmark alliance," said John Maraganore, Ph.D., Chief Executive Officer of Alnylam. *"We believe that ALN-AT3 holds tremendous promise as a new investigational medicine for the management of hemostasis in hemophilia and rare bleeding disorders. We look forward to working with Genzyme to advance ALN-AT3 as a potential new treatment option for people with hemophilia around the world."*

"ALN-AT3 represents a unique and promising new approach for the potential treatment of hemophilia," said David P. Meeker, M.D., President and CEO of Genzyme, a Sanofi company. *"We are excited to expand our clinical stage pipeline of treatments for patients with rare diseases, and to broaden our relationship with Alnylam."*

In January 2014, Alnylam and Genzyme formed an alliance to accelerate and expand the development and commercialization of RNAi therapeutics across the world. The alliance is structured as a multi-product geographic alliance in the field of rare diseases, i.e., programs in Alnylam's Genetic Medicine Strategic Therapeutic Area. Alnylam retains product rights in North America and Western Europe, while Genzyme obtained the right to access certain programs in Alnylam's current and future Genetic Medicines pipeline in the rest of the world (ROW) through the end of 2019, together with certain broader co-development/co-commercialization rights and global rights for certain products. Alnylam maintains development and commercialization control for all programs in its territory.

ALN-AT3 is the third Alnylam product for which Genzyme has exercised its opt-in right, the first two occurring at the close of the deal in early 2014 for patisiran and revusiran, investigational RNAi therapeutics for the treatment of transthyretin-mediated amyloidosis. In the case of ALN-AT3, Genzyme has elected presently to opt into the program for its ROW rights. Genzyme retains its future opt-in right to co-develop and co-promote ALN-AT3 with Alnylam in North America and Western Europe. Specifically, Genzyme has the right to either co-develop and co-promote ALN-AT3 in Alnylam's territory - with Alnylam maintaining development and commercialization control - or to



maintain its ROW rights for ALN-AT3 and, if exercised by Genzyme, obtain a global license to ALN-AS1, Alnylam's investigational RNAi therapeutic for the treatment of acute hepatic porphyrias. Genzyme will exercise this selection right upon completion of human proof-of-concept for the ALN-AS1 program, which is expected to occur in 2016.

Per the 2014 agreement, Alnylam will receive R&D funding for programs where Genzyme has elected to opt in for development and commercialization. For "regional" programs where Genzyme will develop and commercialize in their ROW territory, such as patisiran and ALN-AT3 as currently structured, Genzyme pays 20% of global development costs. In the case of ALN-AT3, such cost sharing is expected to begin in January 2016. For "co-develop/co-promote" programs such as revusiran (and possibly ALN-AT3 in the future), Genzyme pays 50% of global development costs. For "global" programs (e.g., possibly ALN-AS1 if selected), Genzyme will pay 100% of global development costs. In addition, Alnylam is eligible to receive milestones totaling up to \$75 million per product for regional and co-develop/co-promote programs. In the case of global Genzyme programs, Alnylam is eligible to receive up to \$200 million in milestones per product. Finally, Alnylam is also eligible to receive tiered double-digit royalties up to 20% on net sales on all products commercialized by Genzyme in its territories. In the case of Genzyme's co-develop/co-promote products in the Alnylam territory, the parties will share profits equally and Alnylam will book net sales revenues.

About ALN-AT3 for Hemophilia

Hemophilias are hereditary disorders caused by genetic deficiencies of various blood clotting factors, resulting in recurrent bleeds into joints, muscles, and other major internal organs. Standard treatment for people with hemophilia involves replacement of the missing clotting factor either as prophylaxis or "on-demand" therapy. However, as many as one third of people with severe hemophilia A will develop an antibody to their replacement factor. These 'inhibitor' subjects become refractory to standard replacement factor therapy, and therefore are significantly more complicated to manage and have poorer clinical outcomes.

ALN-AT3 is an investigational, subcutaneously administered RNAi therapeutic for the treatment of hemophilia and other rare bleeding disorders. ALN-AT3 is aimed at correcting coagulation defects by knockdown of antithrombin (AT) - an important endogenous anticoagulant. AT acts as a "brake" on the production of thrombin, a protein essential for the formation of a blood clot. ALN-AT3 is being evaluated in a Phase 1 study in people with moderate-to-severe hemophilia. A pivotal Phase 3 clinical trial is planned to start in mid-2016.

About RNAi

RNAi (RNA interference) is a revolution in biology, representing a breakthrough in understanding how genes are turned on and off in cells, and a completely new approach to drug discovery and development. Its discovery has been heralded as "a major scientific breakthrough that happens once every decade or so," and represents one of the most promising and rapidly advancing frontiers in biology and drug discovery today which was awarded the 2006 Nobel Prize for Physiology or Medicine. RNAi is a natural process of gene silencing that occurs in organisms ranging from plants to mammals. By harnessing the natural biological process of RNAi occurring in our cells, the creation of 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, target the cause of diseases by potently silencing specific mRNAs, thereby preventing disease-causing proteins from being made. RNAi therapeutics have the potential to treat disease and help patients in a fundamentally new way.

About Alnylam Pharmaceuticals

Alnylam is a biopharmaceutical company developing novel therapeutics based on RNA interference, or RNAi. The company is leading the translation of RNAi as a new class of innovative medicines. Alnylam's pipeline of investigational RNAi therapeutics is focused in 3 Strategic Therapeutic Areas (STAr): Genetic Medicines, with a broad pipeline of RNAi therapeutics for the treatment of rare diseases; Cardio-Metabolic Disease, with a pipeline of RNAi therapeutics toward genetically validated, liver-expressed disease targets for unmet needs in cardiovascular and metabolic diseases; and



Hepatic Infectious Disease, with a pipeline of RNAi therapeutics that address the major global health challenges of hepatic infectious diseases. In early 2015, Alnylam launched its “Alnylam 2020” guidance for the advancement and commercialization of RNAi therapeutics as a whole new class of innovative medicines. Specifically, by the end of 2020, Alnylam expects to achieve a company profile with 3 marketed products, 10 RNAi therapeutic clinical programs - including 4 in late stages of development - across its 3 STArS. The company’s demonstrated commitment to RNAi therapeutics has enabled it to form major alliances with leading companies including Merck, Medtronic, Novartis, Biogen, Roche, Takeda, Kyowa Hakko Kirin, Cubist, GlaxoSmithKline, Ascleptis, Monsanto, The Medicines Company, and Genzyme, a Sanofi company. In addition, Alnylam holds an equity position in Regulus Therapeutics Inc., a company focused on discovery, development, and commercialization of microRNA therapeutics. Alnylam scientists and collaborators have published their research on RNAi therapeutics in over 200 peer-reviewed papers, including many in the world’s top scientific journals such as Nature, Nature Medicine, Nature Biotechnology, Cell, New England Journal of Medicine, and The Lancet. Founded in 2002, Alnylam maintains headquarters in Cambridge, Massachusetts. For more information about Alnylam’s pipeline of investigational RNAi therapeutics, please visit www.alnylam.com.

About Genzyme, a Sanofi company

Genzyme has pioneered the development and delivery of transformative therapies for patients affected by rare and debilitating diseases for over 30 years. We accomplish our goals through world-class research and with the compassion and commitment of our employees. With a focus on rare diseases and multiple sclerosis, we are dedicated to making a positive impact on the lives of the patients and families we serve. That goal guides and inspires us every day. Genzyme’s portfolio of transformative therapies, which are marketed in countries around the world, represents groundbreaking and life-saving advances in medicine. As a Sanofi company, Genzyme benefits from the reach and resources of one of the world’s largest pharmaceutical companies, with a shared commitment to improving the lives of patients. Learn more at www.genzyme.com.

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About Sanofi

Sanofi, a global healthcare leader, discovers, develops and distributes therapeutic solutions focused on patients’ needs. Sanofi has core strengths in diabetes solutions, human vaccines, innovative drugs, consumer healthcare, emerging markets, animal health and Genzyme. Sanofi is listed in Paris (EURONEXT: [SAN](#)) and in New York (NYSE: [SNY](#)).

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 ALN-AT3 for the treatment of hemophilia and rare bleeding disorders, expectations regarding the reporting of data from clinical studies, including completion of human proof-of-concept for ALN-AS1, its expectations regarding Genzyme's participation in the development and commercialization of RNAi therapeutics, its expectations regarding the receipt of potential R&D payments, development, regulatory and sales milestones and royalties from Genzyme, expectations regarding its STAr pipeline growth strategy, and its plans regarding commercialization of RNAi therapeutics, including ALN-AT3, constitute forward-looking statements for the purposes of the safe harbor provisions under The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important 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 drug 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, actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials, obtaining, maintaining and protecting intellectual property, Alnylam's ability to enforce its patents against infringers and defend its patent portfolio against challenges from third parties, obtaining regulatory approval for products, competition from others using technology similar to Alnylam's and others developing products for similar uses, Alnylam's ability to manage operating expenses, Alnylam's ability to 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, marketing, sales and distribution of products, the outcome of litigation, 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 to update any forward-looking statements.

Sanofi Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements are statements that are not historical facts. These statements include projections and estimates and their underlying assumptions, statements regarding plans, objectives, intentions and expectations with respect to future financial results, events, operations, services, product development and potential, and statements regarding future performance. Forward-looking statements are generally identified by the words "expects", "anticipates", "believes", "intends", "estimates", "plans" and similar expressions. Although Sanofi's management believes that the expectations reflected in such forward-looking statements are reasonable, investors are cautioned that forward-looking information and statements are subject to various risks and uncertainties, many of which are difficult to predict and generally beyond the control of Sanofi, that could cause actual results and developments to differ materially from those expressed in, or implied or projected by, the forward-looking information and statements. These risks and uncertainties include among other things, the uncertainties inherent in research and development, future clinical data and analysis, including post marketing, decisions by regulatory authorities, such as the FDA or the EMA, regarding whether and when to approve any drug, device or biological application that may be filed for any such product candidates as well as their decisions regarding labelling and other matters that could affect the availability or commercial potential of such product candidates, the absence of guarantee that the product candidates if approved will be commercially successful, the future approval and commercial success of therapeutic alternatives, the Group's ability to benefit from external growth opportunities, trends in exchange rates and prevailing interest rates, the impact of cost containment policies and subsequent changes thereto, the average number of shares outstanding as well as those discussed or identified in the public filings with the SEC and the AMF made by Sanofi, including those listed under "Risk Factors" and "Cautionary Statement Regarding Forward-Looking Statements" in Sanofi's annual report on Form 20-F for the year ended December 31, 2014. Other than as required by applicable law, Sanofi does not undertake any obligation to update or revise any forward-looking information or statements.

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