PRESS RELEASE

Stockholm, Sweden, 27 October 2020

Sobi and Apellis enter collaboration for global co-development and ex-US commercialisation of systemic pegcetacoplan in rare diseases with urgent need for new treatments

- Sobi obtains global co-development and exclusive ex-US commercialisation rights for systemic pegcetacoplan, a targeted C3 therapy
- Apellis retains US commercialisation rights for systemic pegcetacoplan and worldwide commercialisation rights for ophthalmological pegcetacoplan (geographic atrophy program in phase 3)
- The companies will jointly advance systemic pegcetacoplan in five parallel registrational programmes including two new haematological studies planned to start in 2021 (CAD and HSCT-TMA). These join ongoing registrational programmes in haematology (PNH), nephrology (IC-MPGN/C3G) and neurology (ALS)
- Sobi will make an upfront payment of USD 250 million to Apellis and USD 80 million in committed development reimbursements over four years, and up to USD 915 million in regulatory and commercial milestones plus tiered double-digit royalties
- Sobi will arrange a webcast today at 15:00 CET

Stockholm, Sweden and Waltham MA, USA - Swedish Orphan Biovitrum AB (publ) (Sobi™) (STO:SOBI) and Apellis Pharmaceuticals Inc. (Nasdaq: APLS) today announced a strategic collaboration to accelerate the advancement of systemic pegcetacoplan, a targeted C3 therapy, for the treatment of multiple rare diseases with high unmet need that impact more than 275,000 patients globally.

Sobi will receive global co-development and exclusive ex-US commercialisation rights for systemic pegcetacoplan. Apellis retains US commercialisation rights for systemic pegcetacoplan and worldwide commercial rights for ophthalmological pegcetacoplan, which is being evaluated by Apellis in two fully enrolled phase 3 studies in geographic atrophy (GA). Pegcetacoplan targets excessive activation of C3 in the complement cascade, part of the body's immune system, which can lead to the onset and progression of many serious diseases.

Apellis and Sobi plan to jointly advance the clinical development of systemic pegcetacoplan in five parallel registrational programmes across haematology, nephrology and neurology. These include new registrational programmes in cold agglutinin disease (CAD) and haematopoietic stem cell transplantation-associated thrombotic microangiopathy (HSCT-TMA), both of which are expected to start in 2021. By controlling complement activation centrally, pegcetacoplan offers the potential to

become a transformative new therapy in several rare diseases where patients have few or no treatment options today.

“We are excited to collaborate with Apellis, a leader in targeted C3 therapies. The collaboration will significantly strengthen and broaden our late-stage R&D portfolio and be a catalyst for further internationalisation. The products have an excellent fit with our strategic focus on haematology and immunology,” said Guido Oelkers, CEO and President of Sobi. “Given the central role of C3 in the complement cascade, pegcetacoplan has the potential to become the foundation for a broader platform in rare diseases. With positive phase 3 data in PNH, pegcetacoplan can elevate the standard of care for this debilitating blood disorder.”

“This collaboration enables us to further expand on the broad platform potential of targeting C3 for serious rare diseases that impact hundreds of thousands of patients around the world,” said Cedric Francois, MD, PhD, co-founder and Chief Executive Officer of Apellis. “We evaluated numerous companies, medium and large, and chose Sobi because of their global leadership in haematology and rare diseases, track record of successful product launches, and deep commitment to patients. Together, we will quickly advance systemic pegcetacoplan in multiple registrational programmes across haematology, nephrology and neurology, while also preparing for our first potential US launch in PNH. Financially, this transaction also strengthens our position, with our cash runway expected to extend into the second half of 2022.”

As part of the collaboration, Apellis and Sobi will co-develop systemic pegcetacoplan in the following rare diseases:

Haematology – Paroxysmal nocturnal haemoglobinuria (PNH), CAD, and HSCT-TMA
PNH represents the first potential indication to market for systemic pegcetacoplan. Marketing applications for pegcetacoplan for the treatment of PNH were submitted to the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA) based on positive results from the Phase 3 PEGASUS study. Top-line results from the phase 3 PRINCE study, which is evaluating pegcetacoplan in treatment-naïve patients with PNH, are expected in the first half of 2021.

Sobi will lead development activities for the phase 3 study in CAD and a potential registrational phase 2 study in HSCT-TMA, both planned to start in 2021.

Nephrology – Immune complex membranoproliferative glomerulonephritis (IC-MPGN) and C3 glomerulopathy (C3G)
Apellis has initiated and will continue to lead a registrational programme in IC-MPGN and C3G which includes phase 2 and phase 3 studies.

Neurology – Amyotrophic lateral sclerosis (ALS)
Apellis has initiated and will continue to lead a potential registrational phase 2 study in ALS. Multiple other neurological conditions are under consideration for future clinical development.

About the transaction

Sobi will make an upfront payment of USD 250 million to Apellis and up to USD 915 million in other regulatory and commercial milestone payments, and will contribute USD 80 million in reimbursement payments over a four-year period for research and development to support the initial development plan, which includes ongoing studies in PNH, IC-MPGN/C3G, and ALS and new studies in CAD and HSCT-TMA. Apellis will also be eligible for tiered double-digit royalties on sales ranging from high teens to high twenties. Sobi intends to finance these payments with available funds. Sobi will receive reimbursement payments for the costs incurred by Sobi in connection with the CAD and HSCT-TMA trials that Sobi will conduct. The parties have agreed to split costs 50/50 for any future global studies beyond the initial development plan.

Per the terms of the agreement, Apellis will be responsible for all regulatory and commercial activities in the United States and the ongoing Marketing Authorisation Application (MAA) review for PNH in the European Union, which will be subsequently transferred to Sobi. Sobi will be responsible for regulatory and commercial activities for systemic pegcetacoplan in ex-US markets. The co-development of systemic pegcetacoplan will be overseen by a joint development committee, and the commercial strategy will be overseen by a joint commercial committee.

Latham & Watkins LLP are acting as legal advisors to Sobi in connection with the transaction, and Morgan Stanley are acting as financial advisors to Sobi.

About pegcetacoplan (APL-2)

Pegcetacoplan is an investigational, targeted C3 therapy designed to regulate excessive complement activation, which can lead to the onset and progression of many serious diseases. Pegcetacoplan is a synthetic cyclic peptide conjugated to a polyethylene glycol polymer that binds specifically to C3 and C3b. Apellis is evaluating pegcetacoplan in several clinical studies across haematology, ophthalmology, nephrology, and neurology. Pegcetacoplan was granted Fast Track designation by the US Food and Drug Administration (FDA) for the treatment of PNH and the treatment of geographic atrophy and received orphan drug designation for the treatment of C3G by the FDA and European Medicines Agency.

About pegcetacoplan for paroxysmal nocturnal haemoglobinuria (PNH)

In October, the European Medicines Agency validated the Marketing Authorisation Application (MAA) for pegcetacoplan in PNH, and an opinion from the Committee for Medicinal Products for Human Use is expected in 2021. A decision by the FDA regarding the acceptance of the New Drug Application (NDA) and a Prescription Drug User Fee Act (PDUFA) target action date is expected in the fourth quarter of 2020. Top-line results from the phase 3 PRINCE study, which is evaluating pegcetacoplan in treatment-naïve patients with PNH, are expected in the first half of 2021.

The NDA and MAA submissions for pegcetacoplan for the treatment of PNH are based on positive results from the phase 3 PEGASUS study (APL2-302, NCT03500549), a multicentre, randomised, active-comparator controlled phase 3 study in 80 adults with PNH. The primary objective of PEGASUS was to establish the efficacy and safety of pegcetacoplan compared to eculizumab. Pegcetacoplan is also being evaluated in the phase 3 PRINCE study (APL2-308, NCT04085601), a randomised, multicentre, controlled study evaluating pegcetacoplan in 53 patients with PNH who had not received a complement inhibitor within three months before entering the study.

About PNH

PNH is a rare, chronic, life-threatening blood disorder characterised by the destruction of oxygen-carrying red blood cells through extravascular and intravascular haemolysis. Persistently low haemoglobin can result in frequent transfusions and debilitating symptoms such as severe fatigue, haemoglobinuria, and difficulty breathing (dyspnoea). A retrospective analysis shows that, even on eculizumab, approximately 72 per cent of people with PNH have anaemia, a key indicator of ongoing

haemolysis.[1] The analysis also finds that 36 per cent of patients require one or more transfusions a year and 16 per cent require three or more.[1]

About cold agglutinin disease (CAD)
CAD is a severe, chronic, rare blood disorder[2] that currently has no approved therapies and impacts around 10,500 people across the United States and Europe.[3] People living with CAD may suffer from chronic anaemia, transfusion requirements, and an increased risk of life-threatening thrombotic events such as stroke.[4] In people with CAD, immunoglobin M (IgM) autoantibodies cause red blood cells to agglutinate, or clump together, at temperatures below 30°C or as a result of a compromised immune system or infection.[5] This activates the complement cascade to destroy healthy red blood cells through extravascular and intravascular hemolysis.[6,7]

About haematopoietic stem cell transplantation thrombotic microangiopathy (HSCT-TMA)
HSCT-TMA is rare blood disease that can be a fatal complication of a bone marrow transplant or HSCT.[8] In HSCT-TMA, microscopic blood clots form in small blood vessels, leading to organ damage. The kidneys are commonly affected, although any organ may be involved.[8] HSCT-TMA occurs in up to 40 per cent of HSCT recipients;[9] every year, there are around 9,000 allogeneic transplants in United States and around 18,000 in the EU+.[10,11] Excessive complement activation is a high-risk feature in patients with HSCT-TMA,[12] and C3 is believed to play a critical role in TMA based on proinflammatory and procoagulant properties of C3a and C3b.[13]

About immune complex membranoproliferative glomerulonephritis (IC-MPGN) and **C3 glomerulopathy (C3G)**
IC-MPGN and C3G are rare, debilitating kidney diseases that affect around 18,000 people in the United States and Europe.[14] There are no approved therapies for the diseases, and symptoms include blood in the urine, dark foamy urine due to the presence of protein, swelling, and high blood pressure.[15] Approximately 50 per cent of people living with IC-MPGN and C3G ultimately suffer kidney failure within five to 10 years of diagnosis.[16] Although IC-MPGN is considered a distinct disease from C3G, the underlying cause and progression of the two diseases are remarkably similar and include overactivation of the complement cascade, with excessive accumulation of C3 breakdown products in the kidney causing inflammation and damage to the organ. [17,18]

About amyotrophic lateral sclerosis (ALS)
ALS is a devastating neurodegenerative disease that results in progressive muscle weakness and paralysis due to the death of nerve cells, called motor neurons, in the brain and spinal cord.[19, 20] The death of motor neurons leads to the progressive loss of voluntary muscle movement required for speaking, walking, swallowing and breathing.[19,20] In individuals with ALS, high levels of C3 are present at the neuromuscular junction[21] where motor neurons communicate directly to muscle cells. Numerous studies suggest that elevated levels of C3 present throughout the motor system of ALS patients are likely to contribute to chronic neuroinflammation and the death of motor neurons.[21,22,23] There are no treatments that stop or reverse the progression of ALS, which impacts ~225,000 patients worldwide.[24]

About Sobi
Sobi is a specialised international biopharmaceutical company transforming the lives of people with rare diseases. Sobi is providing sustainable access to innovative therapies in the areas of haematology, immunology and specialty indications. Today, Sobi employs approximately 1,500 people across Europe, North America, the Middle East, Russia and North Africa. In 2019, Sobi's revenue amounted to SEK 14.2 billion. Sobi's share (STO:SOBI) is listed on Nasdaq Stockholm. You can find more information about Sobi at www.sobi.com.

About Apellis
Apellis Pharmaceuticals, Inc. is a global biopharmaceutical company that is committed to leveraging courageous science, creativity, and compassion to deliver life-changing therapies. Leaders in targeted C3 therapies, we aim to develop transformative therapies for a broad range of debilitating diseases that are driven by excessive activation of the complement cascade, including those within haematology, ophthalmology, nephrology, and neurology. For more information, please visit www.apellis.com.

Investor and telephone conference
Financial analysts and media are invited to participate in a telephone conference to discuss the transaction at 15:00 CET. The event will be hosted by Sobi's CEO and President, Guido Oelkers, and the presentation will be held in English.

The presentation can be followed live, or afterwards on www.sobi.com.

To participate in the telephone conference, please call:

SE: +46 8 505 58356
UK: +44 333 300 9263
US +1 646 722 4957

Click here to go to the live webcast.

After the live event the webcast will be available on-demand via the same URL.

This information is information that Swedish Orphan Biovitrum AB (publ) is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of Linda Holmström, Corporate Communication and Investor Relations, at 12:00 CET on 27 October 2020.

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[1] McKinley C. Extravascular Hemolysis Due to C3-Loading in Patients with PNH Treated with Eculizumab: Defining the Clinical Syndrome. Blood. 2017;130:3471.
[2] Sokol RJ, Hewitt S, Stamps BK. Autoimmune haemolysis: an 18-year study of 865 cases referred to a regional transfusion centre. *Br Med J (Clin Res Ed).* 1981;282(6281):2023-2027.National Institute of Health (NIH), Genetic and Rare Diseases Information Center (GARD)
[3] Catenion using physician and literature consensus
[4] Website https://rarediseases.info.nih.gov/diseases/6130/cold-agglutinin-disease. Accessed November 21, 2019.
[5] Berentsen S, Ulvestad E, Langholm R, et al. Primary chronic cold agglutinin disease: a population based clinical study of 86 patients. *Haematologica.* 2006;91(4):460-466.
[6] Cold agglutinin disease. Genetic and Rare Diseases Information Center Web site. https://rarediseases.info.nih.gov/diseases/6130/cold-agglutinin-disease. Accessed November 21, 2019.
[7] Reynaud Q, Durieu I, Dutertre M, et al. Efficacy and safety of rituximab in auto-immune hemolytic anemia: A meta-analysis of 21 studies. *Autoimmun Rev.* 2015;14(4):304-313.
[8] Dvorak C, et al. Transplant-Associated Thrombotic Microangiopathy in Pediatric Hematopoietic Cell Transplant Recipients: A Practical Approach to Diagnosis and Management. Frontiers in Pediatrics. Vol 7, article 133. (2019)
[9] Jodele S, et al. Diagnostic and risk criteria for HSCT-associated thrombotic microangiopathy: a study in children and young adults. Blood. 124(4): 645–653 (2014)
[10] Current Uses and Outcomes of Hematopoietic Cell Transplantation (HCT): CIBMTR Summary Slides
[11] Passweg et al, BMT. 2019, 38: 1575–1585
[12] Jodele S, et al. Complement blockade for TA-TMA: lessons learned from a large pediatric cohort treated with eculizumab. Blood. 135 (13): 1049–1057. (2020)
[13] Noris M, et al. STEC-HUS, atypical HUS and TTP are all diseases of complement activation. Nature Reviews Nephrology. 8, 622–633 (2012)
[14] ClearView Analysis using physician and literature consensus.
[15] Complement 3 Glomerulopathy (C3G). National Kidney Foundation Website. https://www.kidney.org/atoz/content/complement-3-glomerulopathy-c3g.Accessed November 21, 2019.
[16] C3 glomerulopathy. National Institute of Health, Genetics Home Reference. https://ghr.nlm.nih.gov/condition/c3-glomerulopathy#resources. Accessed November 21, 2019.
[17] Noris M, Donadelli R, Remuzzi G. Autoimmune abnormalities of the alternative complement pathway in membranoproliferative glomerulonephritis and C3 glomerulopathy. Pediatr Nephrol. 2019 Aug;34(8):1311-1323.
[18] Cook HT. Evolving complexity of complement-related diseases: C3 glomerulopathy and atypical haemolytic uremic syndrome. Curr Opin Nephrol Hypertens. 2018 May;27(3):165-170.
[19] Genetics Home Reference. Available at: https://ghr.nlm.nih.gov/condition/c3-glomerulopathy. Accessed November 27, 2019.
[20] National Institute of Neurological Disorders and Stroke. (2020). Amyotrophic Lateral Sclerosis Fact Sheet. Retrieved from https://www.ninds.nih.gov/Disorders/Patient-Caregiver-Education/Fact-Sheets/Amyotrophic-lateral-Sclerosis-ALS-Fact-Sheet
[21] ALS Association. What is ALS? Retrieved June 2020 from https://www.als.org/understanding-als/what-is-als
[22] Bahia El Idrissi N, et al. *J Neuroinflammation.* 2016;13(1):72.[4] Sta M, et al. *Neurobiol Dis*. 2011;42(3):211-220.
[23] Woodruff, et al., *PNAS* January 7, 2014 111 (1) E3-E4
[24] Lee, et al *Journal of Neuroinflammation* volume 15: 171 (2018)[25] Arthur K et al. Nat Commun, 2016, Vol 7, article 12408
[25] Arthur K et al. Nat Commun, 2016, Vol 7, article 12408