Aceragen Launches with Acquisition of Enzyvant's Investigational Therapy for Farber Disease and $35 Million Product Financing with NovaQuest

-Acquires worldwide rights to RVT-801, a novel enzyme replacement therapy for treatment of Farber disease

-NovaQuest investment to fund product and clinical development

-Protocol for potential single registration study reviewed with FDA and EMA

-Program has been granted rare pediatric disease status, fast track and orphan designations, and is eligible for a priority review voucher once approved

Research Triangle Park, NC and Basel, CH - May 1, 2021 – Aceragen, Inc., a biopharmaceutical company focused on advancing transformational therapeutics for rare and ultra-rare diseases, today announced the acquisition of Enzyvant's RVT-801 (now ACG-801), an investigational enzyme replacement therapy (ERT) for acid ceramidase deficiency presenting as Farber disease, a lysosomal storage disease with a unique, severe inflammatory phenotype for which no disease-specific therapy exists. Enzyvant will receive an upfront payment and development and sales-based milestones up to $226 million, as well as tiered royalties on net sales. In addition, Aceragen entered into a $35 million product financing agreement with NovaQuest Capital Management to fund the development program into a potential registrational study.

Aceragen was recently founded by a team of industry and medical veterans with a long record of accomplishment in product development and commercialization in biopharmaceuticals, much of which has been in the rare disease field. Co-founder John Taylor will lead the company as President and Chief Executive Officer, having previously held the same roles with Spyryx Biosciences.

"The Aceragen team is delighted to announce the strategic transaction with Enzyvant and the relationship we have established with NovaQuest to continue the development of ACG-801," Taylor stated. "This program is based on the foundational work of Dr. Ed Schuchman at the Icahn School of Medicine at Mount Sinai, establishing the potential to address the underlying pathology of Farber disease, a genetic deficiency of acid ceramidase. In addition to the intellectual property and regulatory designations, Aceragen has also acquired from Enzyvant a robust preclinical package including several completed toxicology studies, a quantitative patient research study and the first ever natural history study in patients with Farber disease that documented and quantified the features, impact and progression of this devastating condition. Aceragen's relationship with NovaQuest, an experienced investor in the area of pharmaceutical development, will enable us to advance through the planned clinical study and associated regulatory submissions with the goal of delivering a disease-specific therapy to patients who are in desperate need."

ACG-801

ACG-801 is an investigational form of recombinant human acid ceramidase (rhAC) designed to address the genetic deficiency of the naturally occurring enzyme which is the cause of Farber

disease and Spinal Muscular Atrophy with Progressive Myoclonic Epilepsy (SMA-PME). Preclinical development for rhAC has been completed, including in vivo proof of concept in a mouse model of Farber disease, and IND-enabling GLP toxicology studies. Data in animal models has also been published supporting the potential of rhAC as a treatment for cystic fibrosis.

Patients with Farber disease have been involved in the development through a quantitative research study and the natural history study. Data from these studies support the clinical investigation of ACG-801 and significant progress has been made in harmonized regulatory discussions with the FDA and the EMA. ACG-801 has been granted Rare Pediatric Disease and Fast Track designations by the FDA, as well as Orphan Drug designations by the FDA and EMA.

“Breakthrough therapies for patients battling devastating rare diseases often result from extraordinary collaborations between academic discovery teams and dedicated developers like Enzyvant and Aceragen,” said Rachelle Jacques, CEO of Enzyvant. “We are delighted that Aceragen, with strong capabilities, a commitment equal to our own and a singular focus, will rapidly advance this important therapy to address the significant unmet needs of Farber disease patients and their families.”

Concurrent with the closing of the transaction, NovaQuest’s Managing Partner Ron Wooten and Vice President Stephen Lesser have joined Aceragen’s board of directors.

“We congratulate John and the Aceragen team for identifying this exceptional rare disease opportunity in Farber disease and for choosing NovaQuest’s Product Finance approach among the alternatives,” said Lesser. “It was a pleasure for all of us at NovaQuest to conclude that at-risk, non-dilutive financing is possible as a company-enabling resource for development funding within the realm of ultra-rare disease. NovaQuest believes that opportunities, like ACG-801, offer an attractive area for investment, combining a significant unmet medical need with a treatment that has the potential to deliver a clinically meaning benefit and improved quality of life for patients.”

“Our product financing agreement with Aceragen furthers NovaQuest’s core strategy and focus of providing innovative funding for a wide range of biopharma companies and products,” Wooten added. “We are pleased to join the Aceragen board and contribute to the team’s efforts to provide benefits to patients with Farber disease.”

Wedbush Securities acted as strategic financial advisor, Hutchison PLLC was corporate counsel, and Hogan Lovells provided IP counsel to Aceragen. Arnold & Porter was legal counsel to Enzyvant, and Wyrick Robbins Ponton & Yates LLP acted as legal counsel to NovaQuest.

About Farber disease and Acid Ceramidase Deficiency

Farber disease is caused by mutations in the *ASAH1* gene, resulting in a deficiency of acid ceramidase, a naturally occurring lysosomal enzyme. The enzyme normally acts to metabolize ceramide, a highly inflammatory and apoptotic lipid. Lack of adequate acid ceramidase function results in accumulation of ceramide and causes a severe inflammatory disease associated with macrophage-filled subcutaneous granulomas, joint damage and deformity, and life-threatening

respiratory complications among other severe symptoms. Patients with the most rapidly progressive form of Farber disease usually do not live beyond the first few years of life. A portion of the patient population also have progressive central nervous system involvement, leading to developmental delay and regression. Acid ceramidase deficiency also causes Spinal Muscular Atrophy with Progressive Myoclonic Epilepsy (SMA-PME) and dysregulation of ceramide metabolism has been implicated in a number of other diseases and conditions, including cystic fibrosis.

About Aceragen

Aceragen is a biopharmaceutical company focused on the development of innovative therapeutics for rare and ultra-rare diseases. The Company is advancing ACG-801 (rhAC) as an investigational enzyme replacement therapy for the treatment of patients with Farber disease and potentially other diseases associated with the dysregulation of ceramide metabolism.

For more information, please visit www.aceragen.com.

About NovaQuest Capital Management

Founded by a team of accomplished industry professionals who began working together in 2000, NovaQuest Capital Management is a premier biopharma and life sciences investment firm. NovaQuest pioneered a Product Finance solution for the industry, providing at-risk, nondilutive funding that enables partner companies to advance pivotal clinical trials, launch new brands, license products, and acquire accretive products or companies. NovaQuest has invested in scores of biopharmaceutical assets across therapeutic areas with a clinical success rate significantly higher than the industry average. Currently managing more than $2.2 billion in capital, NovaQuest is actively investing from the $1.2 billion Fund V, evaluating global opportunities with financing needs that range from $30-100 million.

For more information, please visit www.novaquest.com.

About Enzyvant

Enzyvant, a wholly owned subsidiary of Sumitovant Biopharma Ltd. (wholly owned by Sumitomo Dainippon Pharma Co., Ltd.), is a biotechnology company dedicated to developing novel, transformative regenerative therapies for people with devastating rare diseases. Enzyvant's lead asset is the investigational tissue-based regenerative therapy, RVT-802, for congenital athymia, an ultra-rare and life-threatening pediatric immunodeficiency. RVT-802 has been granted multiple regulatory designations, including the US Food and Drug Administration designation as a Regenerative Medicine Advanced Therapy (RMAT).

For more information about Enzyvant, visit www.Enzyvant.com. Follow @Enzyvant on Twitter, Facebook and LinkedIn.

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