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ST. LOUIS--(BUSINESS WIRE)--APT Therapeutics, Inc. is developing an investigational new drug, APT102, for heart attack and stroke patients. According to a recent study published in Science Translational Medicine, APT102 maintained optimal blood flow and reduced damage to the heart muscle by 80% in animal model studies of heart attack. Drugs commonly used to prevent blood clots for heart attack and stroke patients carry a risk of serious bleeding events. In contrast, APT102 attenuated bleeding in the study. Two of APT Therapeutics' lead scientists were co-authors of the study, Soon Seog Jeong, PhD, and Ridong Chen, PhD. Dr. Chen is also founder and CEO of APT Therapeutics.

"The results in the Science paper reflect 12 years of tremendous effort by leading scientists at Cornell, Harvard and Washington University," stated Brian Clevinger, Ph.D., Chairman of the company and Managing Director of Prolog Ventures. "This represents a significant step toward developing a safer and more effective therapy for heart attacks, the leading cause of death in the industrialized world."

The evidence for this innovative therapeutic approach began accumulating approximately 20 years ago with the pioneering work on CD39, the first discovered human apyrase, by the research teams of Dr. Aaron J. Marcus at Weill Cornell Medical College and Dr. Simon C. Robson at Harvard University, co-authors on the recent study. ADP and ATP are molecules released at sites of clotting and vascular injury during a heart attack, thereby enhancing the formation of blood clots and exacerbating damage to cardiac tissue. Apyrase is an enzyme expressed on the outside of cells, which metabolizes ADP and ATP and in return reduces their ability to 1) stimulate clotting by blood cells known as platelets and 2) enhance inflammation of cardiac and vascular tissue. Furthermore, apyrase-induced metabolism of ATP and ADP ultimately generates the cardio-protective molecule adenosine. APT102 is an optimized, drug-able version of human apyrase that has demonstrated anti-clotting and cardio-protective effects in experimental models of heart attack and stroke.

"Human apyrase does not cause bleeding, as the enzyme has no effect on platelets per se, but deletes the released molecule ADP that causes platelets to promote a clot. In contrast, current drugs used to prevent clotting damage or modify platelets and as such can cause dose-limiting hemorrhage," explained Aaron J. Marcus, MD, Professor of Medicine, and Pathology and Laboratory Medicine at Weill Cornell Medical College in New York, and an International Aspirin Award winner.

"The complete lack of bleeding side effects with APT102 fits well with our CD39 data," said M. Johan ("Han") Broekman, PhD, retired Associate Professor at Weill Cornell Medical College. "When we

opened the chest to obtain blood samples, there was much more bleeding from the small vessels of aspirin-treated mice than in controls. To our great surprise, no "oozing" whatsoever was observed in CD39-treated mice. Therefore, human apyrase differs radically from conventional antithrombotics."

"The therapeutic approach with human apyrase is highly innovative and practical, as the drug converts the pro-clotting and pro-inflammatory site of vascular injury into a largely anti-clotting and anti-inflammatory environment," noted Simon C. Robson, MD, Charlotte F. and Irving W. Rabb Professor of Medicine at Harvard Medical School in Boston. He further commented that APT102 might also have potential to limit tissue damage that occurs during organ transplantation.

Dana Abendschein, PhD, Associate Professor of Medicine at Washington University believes the efficacy and safety data of APT102 is truely promising, "We have tested many of the novel adjunctive agents in large animals developed over the last 20 years and have not seen any that approach the preclinical efficacy obtained with APT102."

"I believe there remains a tremendous unmet need for a safe and efficacious antithrombotic agent," said Steve Steinhubl, MD, Professor at Scripps Translational Science Institute, who previously led a number of major clinical trials designed to evaluate novel therapies that inhibit the production of blood clots. Dr. Steinhubl emphasized that even after a blocked blood vessel is opened substantial damage can still occur due to the blockade of small branch blood vessels and inflammatory injury. APT102 can prevent both blood clots and inflammation to potentially minimize serious long-term consequences in heart attack survivors.

"These findings strongly suggest that APT102 is a viable therapeutic candidate. Given that the majority of adverse cardiovascular events occur in the first hours and days after reperfusion therapy, the optimal treatment regimen may be acute treatment with APT102, a safe, yet highly efficacious agent," said Robert Leadley, Ph.D., who has led numerous antithrombotic drug discovery efforts at leading pharmaceutical companies. "Following this high-risk period, patients could be safely bridged to chronic treatment with orally active drugs such as clopidogrel, prasugrel, or ticagrelor."

"The company is thankful for the amazing teamwork that led to the completion of the highly challenging pharmacological validation in animal models," noted Dr. Ridong Chen. "We look forward to working together with partners to complete preclinical safety studies and progress to clinical trials so that this unique therapy will eventually make a difference for patients."

About APT Therapeutics, Inc.: APT Therapeutics™ is developing optimized human apyrases as safe and effective therapy for acute myocardial infarction, stroke and pulmonary arterial hypertension. The business strategy is to out-license or co-develop the drug candidates with pharma partners. The Company is headquartered in St Louis, Missouri. For more information about the company please visit www.apt-therapeutics.com.

Article: "Optimizing human apyrase to treat arterial thrombosis and limit reperfusion injury without increasing bleeding risk," by D. Moeckel; A. Nguyen; D. Abendschein at Washington University School of Medicine in St. Louis, MO; S.S. Jeong; R. Chen at APT Therapeutics Inc. in St. Louis, MO; X. Sun; S.C. Robson at Beth Israel Deaconess Medical Center in Boston, MA; X. Sun; S.C. Robson at Harvard Medical School in Boston, MA; M.J. Broekman; J.H.F. Drosopoulos; A.J. Marcus at Veterans Affairs New York Harbor Healthcare System in New York, NY; M.J. Broekman; J.H.F. Drosopoulos; A.J. Marcus at Weill Cornell Medical College in New York, NY.

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