ARA 290 Obtains European Union Orphan Drug Designation for Prevention of Graft Loss in Pancreatic Islet Transplantation

TARRYTOWN, NY, November 28, 2016. **Araim Pharmaceuticals**, a clinical stage drug development company with a unique platform technology for activating post-injury tissue repair and recovery, today announced that the European Commission (EC) has granted Orphan Medicinal Product designation in the European Union (EU) to the Company's lead product candidate, Innate Repair Receptor activator ARA 290, for prevention of graft loss in pancreatic islet transplantation. The EC's approval follows a positive opinion in July from the European Medicine Agency's (EMA) Committee for Orphan Medicinal Products (COMP). The U.S. Food and Drug Administration (FDA) has previously granted Orphan Drug Designation to ARA 290 for treatment to increase survival and improve functioning of pancreatic islets following transplantation.

Type 1 diabetes (T1D) is an autoimmune disease in which the body attacks and destroys the insulin producing beta cells in the pancreas. Pancreatic islet cell transplantation (PITx) is a promising experimental treatment option for patients with severe T1D characterized by significant metabolic instability and frequent life-threatening hypoglycemic episodes. However, currently islet cell transplantation requires that each recipient receive a large number of islets, as the procedure causes an inflammatory reaction which destroys a significant proportion of the administered islets.

"In a rodent model of islet transplantation, ARA 290 markedly reduced transplantation-induced inflammation, resulting in a marked increase in islet survival and function, such that 85% of ARA 290 animals achieved the target blood glucose concentration, compared to none of the control animals[1]," said Dr. Michael Brines, Co-Founder and Chief Scientific Officer of Araim Pharmaceuticals. "These effects of ARA 290 on islet preservation, if translated successfully for human disease, could result in the need for far fewer islets per transplant, permitting a substantial increase in the number of individuals with T1D who can undergo the potentially curative procedure."

There are no products authorized in the EU for prevention of islet cell graft loss, signifying an important unmet medical need. "This EC decision highlights the significant need for a drug that could transform an important treatment option for patients with T1D", stated Dr. Anthony Cerami, the Founder of Araim Pharmaceuticals. "As the developer of the HbA1c diagnostic test, I have had a very strong interest in improving outcomes for patients with diabetes. I am pleased that yet another discovery I have worked on may have a significant role in improving the lives of patients with T1D."

About Orphan Drug Designation

The European Commission grants orphan drug status for medicinal products intended to diagnose, prevent or treat a life-threatening or chronically debilitating condition that affects not more than five in 10,000 people in the EU. The designation provides certain

---

[1] Watanabe M, Lundgren T, Saito Y et al. A nonhematopoietic erythropoietin analogue, ARA 290, inhibits macrophage activation and prevents damage to transplanted islets. Transplantation 2016;100(3):554–562.

benefits and incentives in the EU, including protocol assistance, fee reductions, and ten years of market exclusivity upon regulatory approval.

About Pancreatic Islet Cell Transplantation

Pancreatic islet cell transplantation is rare, with North America comprising 60% of recipients, and Europe/Australia comprising 40% of recipients; for the latter, average yearly incidence based on 2008-2013 data is estimated at 0.0012/10,000 individuals per year[2]. Significant obstacles to more widespread use of PITx are shortage of suitable islets from donors and process-related islet damage or destruction. The immediate inflammatory response associated with islet transplantation has been recognized as the primary cause of early damage to islets and graft loss[3,4]. Multiple different regimen with immune suppressive therapies that help prevent the long-term rejection of allogenic transplanted cells are under active study. However, since a significant mass of islets is lost immediately, effective control of pre- and peri-transplant islet inflammation could improve post-transplant islet survival and in turn increase the benefits of islet cell transplantation for a greater number of patients.

About Araim Pharmaceuticals, Inc.

Araim Pharmaceuticals, Inc. is a clinical stage drug development company with a novel platform technology designed to address devastating injuries and chronic diseases underserved by current therapies. With their discovery of the Innate Repair Receptor (IRR), Araim has identified the target for activating tissue repair and recovery from inflammatory and other injuries. They have a novel peptide library of IRR specific ligands that activate tissue protective, reparative and anti-inflammatory signaling pathways. Araim has an ongoing, active and promising preclinical program in a wide array of conditions involving tissue injury and repair, including neuropathy, cardiovascular injury, diabetes complications, wound healing and aging. Their lead compound, ARA 290, a novel 11 amino acid peptide, has been granted US and EU orphan drug designations for the treatment of sarcoidosis, and US orphan drug and Fast Track designations for treatment of neuropathic pain in patients with sarcoidosis. The most advanced clinical program with ARA 290 is in sarcoidosis-related small fiber neuropathy, with the recent completion of a Phase 2b dose-ranging trial. A pilot study evaluating the safety and efficacy of ARA 290 in diabetic macular edema is currently ongoing at Queen's University Belfast. www.araimpharma.com

!

##

Media Contact:

Rita Kirk

Araim Pharmaceuticals, Inc.
rkirk@araimpharma.com

---

[2] Collaborative Islet Cell Registry newsletter, 2015.

[3] Kanak MA, Takita M, Kunnathodi F, et al. Inflammatory response in islet transplantation. Int J Endocrinol 2014;vol 2014: Article ID 451035.

[4] Piemonti L, Leone BE, Nano R, et al. Human pancreatic islets produce and secrete MCP-1/CCL2: relevance in human islet transplantation. Diabetes. 2002;51(1):55-65.