

Pervasis case study

Education



Pervasis Receives Orphan Drug Designation in Europe for Vascugel® to Prevent Arteriovenous Access Failure in Patients Undergoing Hemodialysis Cell-Based Therapy Aims to Regulate the Body's Healing Response, Promoting Vascular Repair, Reducing the Need for Repeat Surgical Procedures and Improving Patient Outcomes Cambridge, Mass. , March 1, 2011 — Pervasis Therapeutics, Inc. announced today that the European Commission (EC) has granted Orphan Drug Designation for Vascugel® for the prevention of hemodialysis vascular access failure in patients with end stage renal disease (ESRD).

The designation follows a positive opinion from The Committee for Orphan Medicinal Products (COMP) within the European Medicines Agency (EMA) earlier this year. Vascugel is a novel endothelial cell-based therapy that aims to regulate the body's healing response following surgical interventions to create vascular access which are necessary for ESRD patients undergoing hemodialysis.

By promoting and enhancing vascular repair, Vascugel reduces the need for repeat surgical interventions and improves overall patient outcomes. ESRD is an advanced and irreversible condition treated mainly by hemodialysis or kidney transplantation. It is estimated that more than 250, 000 ESRD patients in the EU receive hemodialysis, a blood purification therapy designed to replace critical kidney functions – such as filtering waste.

Orphan Drug Designation by the EC provides regulatory and financial incentives for companies to develop and market therapies that treat a life-threatening or chronically debilitating condition affecting no more than five in 10, 000 persons in the European Union (EU). In addition to a 10 year period

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of marketing exclusivity in the EU after product approval, Orphan Drug Designation provides companies with scientific advice and regulatory assistance from the EMA during the product development phase, direct access to centralized marketing authorization, as well as reductions in certain fees. We are committed to bringing Vascugel to patients with end stage renal disease who currently must endure serious complications and repeat surgical procedures so they can continue to receive life-saving hemodialysis treatment,” Frederic Chereau, president and chief executive officer of Pervasis. “ Receiving Orphan Drug Designation in the EU is an important step, as it will help to advance the development process, and enhance our ability to deliver our novel cellbased therapeutic approach to address this significant unmet medical need. Vascugel has demonstrated proof of concept in two Phase 2 clinical trials involving patients with ESRD who require a permanent arteriovenous (AV) access in order to undergo hemodialysis. In these trials, Vascugel exhibited an excellent safety profile, and encouraging efficacy trends were observed, including improved duration of patency (or unimpeded blood flow) and a delay in time to first intervention as compared to placebo. In 2009, Pervasis received Orphan Drug Designation from the U.

S. Foodand Drug Administration (FDA) for Vascugel in patients with ESRD. Pervasis announced last month that the FDA had granted Fast Track review status for Vascugel. Last year, Pervasis reached an agreement with the FDA for a Phase 3 clinical trial of Vascugel under the Agency’s Special Protocol Assessment (SPA) procedure, whereby FDA formalized its agreement that

the design of the Phase 3 trial was acceptable to support a regulatory submission seeking new drug approval.

Failure of Hemodialysis Access Points Leads to Poor Outcomes During hemodialysis, blood is removed from the body, filtered through a dialyzer, or artificial kidney, and then returned to the body. Patients must undergo a surgical intervention to create a vascular access that enables blood to flow from the body to the dialyzer and back to the body. AV fistulae (created by directly joining an artery and vein) and AV grafts (created using a synthetic tube to join an artery and vein) are the two primary types of hemodialysis access.

Due to an inflammatory cascade triggered by surgical intervention, the vascular access often has difficulty healing, and quickly become unusable or clot rapidly, prompting the need for additional, recurring surgeries to create a new access which can lead to multiple complications. Up to 60 percent of all arteriovenous (AV) grafts require re-intervention after one year. 1, 2 AV access failure is the most common reason for hospitalization among hemodialysis patients and can lead to anemia, infection, weight loss, jaundice, prolonged bleeding, and other serious complications. Vascugel® - Combating Inflammation and Promoting Healing Pervasis' novel approach to cell therapy uses adult-differentiated allogeneic endothelial cells (donor endothelial cells with a highly targeted biologic function) embedded in a polymer matrix to enhance the body's natural healing response. The endothelium is the thin layer of cells that lines the interior surface of blood vessels in the body. Endothelial cells are critical to tissue repair and health,

and have a well-understood role in regulating many of the body's healing processes, including those associated with vascular repair.

Vascugel, which utilizes Pervasis' patented endothelial cell-based platform, is placed on the outside of the blood vessel at the AV access site during the surgical intervention to create the access. The endothelial formulation in Vascugel secrete several factors that combat inflammation and promote proper vascular healing, reducing thrombosis (or clotting) and the formation of intimal hyperplasia, or a thickening of the blood vessel wall in response to injury. After approximately four to eight weeks, Vascugel is safely resorbed by the body.

Pervasis' other areas of clinical investigation include improving outcomes in patients with peripheral artery disease (PAD) following surgical procedures such as percutaneous transluminal angioplasties (PTAs) with stenting, the failures of which result in serious complications and a significant increase in medical costs. Earlier this year, Pervasis announced it has also embarked on an oncology development program using its proprietary endothelial cellbased platform to prevent solid tumor growth, cancer recurrence and metastatic disease. About Pervasis Dixon et al.

DAC Study Group. Effect of dipyridamole plus aspirin on hemodialysis graft patency. *N Engl J Med.* 2009; 360: 2191-2201. 2 Hayashi et al. Vascular access for hemodialysis. *Nat Clin Pract Nephrol* 2006; 2: 504-513 3 Castner D. Recommendations for tracking arteriovenous access complications using a charting-by-exception model. *Anna Journal*, 1998; 25(4): 393-396. 1 Pervasis Inc. is a clinical stage company that is developing groundbreaking

endothelial cell-based therapies designed to regulate the body's natural healing and repair processes in various critical therapeutic areas.

The company has initially focused on developing therapies to improve the outcomes of common vascular interventions, such as arteriovenous access, angioplasties, stent placements, and peripheral and coronary bypass grafts – the failure of which result in serious complications and a significant increase in medical costs. The company's lead program, Vascugel®, has demonstrated proof of concept and safety in two Phase 2 clinical trials.

Pervasis is also applying its endothelial cell-based platform technology to develop an oncology therapy focused on preventing solid tumor growth, cancer recurrence and metastatic disease, as well as products for inflammatory disease and orthopedic injury. Pervasis is a privately held company with funding from Flagship Ventures, Polaris Venture Partners, Highland Capital Partners and the Richter Family Fund. For more information, please visit www.pervasistx.com. This news release contains certain forward-looking statements that involve risks and uncertainties.

Such statements are only predictions and the company's actual results may differ materially from those anticipated in these forward-looking statements. Factors that may cause such differences include the timing of clinical trials, the risk that products that appeared promising in early research and clinical trials do not demonstrate safety or efficacy in clinical trials and the risk that the company will not obtain approval to market its products. Company

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