Staff Writer
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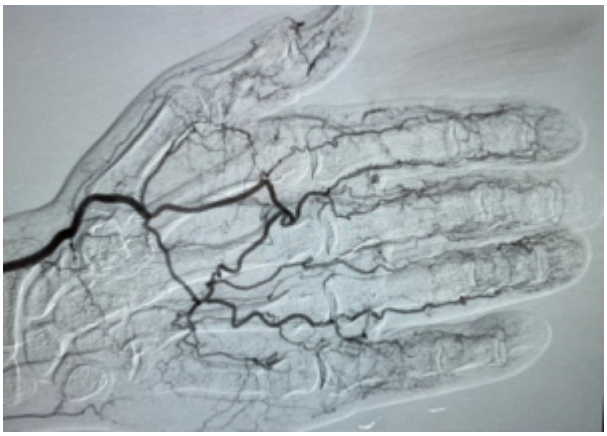
UM Spinoff Achieves FDA Orphan Drug Status for Buerger's Disease

A biotech company that emanated from the Miller School's Omaida Velazquez, M.D., professor and chair of the Dewitt Daughtry Family Department of Surgery, and Zhao-Jun Liu, M.D., Ph.D., associate professor of the Dewitt Daughtry Family Department of Surgery, has achieved a major milestone. The company, Ambulero, Inc., is developing new cell and gene therapy treatments for patients suffering from severe vascular disease. The FDA's Office for Orphan Products Development has granted the company's request for orphan drug status for its gene therapy candidate, AMB-301, to treat Buerger's disease.



Angiographic example wherein the blood flow shows in black contrast. In Buerger's Disease, the tips of fingers and toes are deprived of circulation due to sickened

or obliterated blood vessels.

Buerger's disease is a highly debilitating vascular disease that can lead to severe limb damage and amputation, often occurring in relatively young patients. There are no effective treatments, making any new therapies critically important.

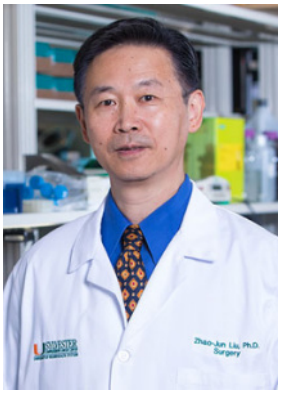
AMB-301 is a promising gene therapy candidate for enhancing blood vessel formation, restoring tissue integrity, and eliminating the need for amputation as a treatment. It is a first-in-class gene therapy vector encoding a cell adhesion molecule, E-selectin. More than a decade of research in animal models with vascular disease suggests that providing E-selectin to damaged blood vessels and surrounding tissue promotes therapeutic angiogenesis, supports robust tissue regeneration, and improved limb function.

Dr. Omaida C. Velazquez

Ambulero's gene therapy approach uses an Adeno-associated virus (AAV) construct to express the E-selectin gene payload as a membrane-bound protein on the surface of cells within the affected limb. AAV gene therapy systems are generally considered clinically safe, and the FDA has already approved

an AAV gene delivery approach using a different payload for the treatment of an inherited type of childhood blindness.

“Current day standard of care for BD patients addresses only the initiating modifiable risk factors. However, for the restoration of the vascular tissue damage at diagnosis, or the halting of disease progression, no therapies have been developed to-date,” said Dr. Velazquez, who is a renowned expert in treating vascular disease. “AMB-301 is a promising new drug candidate. It brings hope to BD patients who often suffer debilitating major limb amputations.”



Zhao-Jun Liu, M.D., Ph.D.

Dr. Liu, who also serves as the chief scientific officer of Ambulero, added “Receiving orphan drug designation for AMB-301 is an important milestone to emerge from our vascular research program. We are pleased with this achievement and believe AMB-301 would address a significant unmet medical need for BD patients, if approved.”

The FDA grants orphan designations to medical products showing promise to treat diseases that affect 200,000 or fewer Americans. This designation provides AMB-301 with seven years

of market exclusivity for the treatment of Buerger’s disease, if approved.

“Ambulero is honored to receive this important FDA recognition,” said Robert L. Buchanan, chief executive officer of Ambulero, Inc. “We believe our first-in-class gene therapy platform will provide new treatment options to patients suffering from BD and potentially other severe vascular diseases.”

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