



## **Araim Pharmaceuticals Receives Orphan Drug Designation from the US FDA for ARA 290 for the Treatment of Sarcoidosis**

TARRYTOWN, NY, July 5, 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 US Food and Drug Administration (FDA) has granted Orphan Drug Designation for its lead product candidate, Innate Repair Receptor activator ARA 290, for the treatment of sarcoidosis. ARA 290 has previously been granted EU Orphan Drug Designation for the treatment of sarcoidosis, and has received US Orphan Drug and Fast Track designations for the treatment of neuropathic pain in patients with sarcoidosis.

“The granting of Orphan Drug Designation for the treatment of sarcoidosis in both the US and Europe highlights the significant need for a drug that could transform the treatment of sarcoidosis”, commented Dr. Daniel Culver, Respiratory Institute, Cleveland Clinic. “It is important to drive therapeutic research forward in this area. Patients with sarcoidosis have many unmet needs, as the condition is underappreciated and undertreated. Sarcoidosis has a substantial impact on people in the prime of their life because of the disease, and affects their quality of life and productivity over many decades. ARA 290 has the potential to become an important disease modifying therapy for sarcoidosis patients as well as other diseases characterized by chronic inflammation and persistent tissue injury.”

Sarcoidosis is an uncommon inflammatory disorder that affects multiple organ systems and results in varied clinical signs and symptoms, often severe and disabling. Small fiber neuropathy (SFN) is a serious, potentially devastating complication of sarcoidosis that is being recognized with increasing frequency, with debilitating pain, abnormal sensory function, and severe dysautonomia as prominent manifestations. The precise pathophysiology of sarcoidosis SFN is unknown. No approved therapies are available, and off-label treatments remain symptomatic, inconsistent and limited.

Extensive preclinical data suggest a potential benefit of ARA 290 in treatment of SFN, as ARA 290 has demonstrated efficacy in a number of animal models of neuropathy, neuroprotection, and neuro-inflammation. Because of the broad range of efficacy noted with ARA 290 in preclinical models of neuropathy, ARA 290 is currently being evaluated in the clinical developmental program not only for its ability to relieve symptoms associated with sarcoidosis SFN, but also for its potential for disease modification and long-term functional improvement in this condition.

### **About Orphan Drug Designation**

The Orphan Drug Designation program provides orphan status to drugs and biologics, which are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people in the US<sup>1</sup>.

This designation provides for a seven year marketing exclusivity period against competition, as well as certain incentives, including federal grants, tax credits and a waiver of PDUFA filing fees.

### **About Sarcoidosis**

Sarcoidosis<sup>ii</sup>,<sup>iii</sup>,<sup>iv</sup> is a chronic systemic granulomatous disease of unknown etiology, most commonly affecting young adults. The disease is chronic and progressive in more than a third of cases, leading to clinically significant organ impairment. While variable for each patient, the symptoms can cause a major loss in quality of life and inability to participate in the economic work force. Morbidity in sarcoidosis is significant and multifactorial. Mortality is infrequent, but may be increasing over the years.

### **About Araim Pharmaceuticals, Inc.**

[Araim Pharmaceuticals, Inc.](http://www.araimpharma.com) 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. Their novel peptide library of IRR specific ligands 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. ARA 290 is a first-in-class synthetic 11-amino acid peptide IRR agonist. 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](http://www.araimpharma.com)

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<sup>i</sup> US Food and Drug Administration. Developing Products for Rare Diseases & Conditions <http://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/default.htm>. Accessed May 2016.

<sup>ii</sup> Ianuzzi MC, Rybicki BA, Teirstein AS. Sarcoidosis. N Engl J Med 2007;357:2153-65.

<sup>iii</sup> Tavee J, Culver D. Sarcoidosis and Small-fiber Neuropathy. Curr Pain Headache Rep 2011;15:201-206.

<sup>iv</sup> Gerke AK. Morbidity and Mortality in Sarcoidosis. Curr Opin Pulm Med 2014;20(5):472-478.