



FOR RELEASE Tuesday, January 6, 2009

For more information, contact:

Ted Townsend, Vice President Business Development, arGentis Pharmaceuticals, LLC
901-448-2024

arGentis receives favorable opinion for European orphan drug designation

Memphis, TN--([BUSINESS WIRE](#))—[arGentis Pharmaceuticals, LLC](#) announced today that the [European Medicines Agency's](#) (EMA) Committee for Orphan Medicinal Products (COMP) adopted a positive opinion recommending the company's product candidate ARG201 (native type 1 bovine collagen) for the treatment of diffuse systemic sclerosis, also known as systemic sclerosis (SSc) for designation as an orphan medicinal product to the European Commission.

arGentis anticipates the confirmation process by the European Commission should be finalized in early 2009. Once confirmed, arGentis' subsidiary, arGentis Autoimmune Europe, LTD will gain access to incentives including 10 years marketing exclusivity throughout Europe for the therapeutic indication for which it was granted, facilitated access to the centralized procedure for the application for marketing approval, reduced fees associated with applying for marketing approval and protocol assistance, and access to EU research funding grants.

The COMP held its ninety-sixth plenary meeting on December 9-10 in London, UK. arGentis' Chief Scientific Officer, Arnold Postlethwaite, M.D. presented ARG201 to the committee where all 27 member states of the European Union (EU) were represented along with Norway, Iceland and patient organizations that utilize the EMA as their regulatory authority for medicines. The designation as an orphan medicinal product is required in order to benefit from EU incentives for the development of drugs for rare diseases. The European Commission decides on the designation based on the opinion of the Committee for Orphan Medicinal Products within the European Medicines Agency. Designation as an orphan drug is clearly different from marketing authorization. Criteria for the designation as an orphan drug are the low prevalence of the disease (less than 5 in 10,000 inhabitants of the EU), severity of the disease and the expected significant benefit for the patients.

[ARG201](#), pioneered by Arnold Postlethwaite, M.D., Director of the Division of Connected Tissue Disease at The University of Tennessee Health Science Center and Andrew Kang, M.D., Professor of Medicine in Rheumatology at The University of Tennessee Health Science Center, is an immunotherapy that induces low dose oral immune tolerance in SSc patients causing downregulation of the body's autoimmune response. It has completed a Phase II clinical trial. The trial included patients with the diffuse form of systemic sclerosis. These patients have cutaneous sclerosis over the limbs, trunk, and face with fibrosis of internal organs as well. In addition to the placebo group there were two prospectively defined subgroups in the Phase II

trial: patients who had been diagnosed for less than three years (early phase) and those diagnosed from three to ten years (late phase). Patients were treated at 13 major Rheumatology Centers for 12 months with follow up at 15 months. Data from the trial demonstrated a statistically and clinically significant improvement in Modified Rodnan Skin Scores (MRSS), a measure of the change in skin thickening and an FDA-mandated endpoint, at 15 months in late phase patients receiving the treatment versus the placebo group (p=0.006). ARG201 was also shown to be safe and well-tolerated.

“This is another very important milestone in our efforts to prepare ARG201 for late phase clinical development. Along with the US Orphan Drug designation received in 2008, arGentis identified that achieving the Orphan Drug designation in the EU would position ARG201 for quicker global market entry. With the incentives afforded by both designations, we look forward to working with the two regulatory agencies to finalize the protocols for late phase trials,” said Tom Davis, CEO of arGentis Pharmaceuticals.

About Systemic Scleroderma and Systemic Sclerosis

[Scleroderma](#) is a disease that causes thickened skin and varying degrees of organ dysfunction resulting from small-vessel vasculopathy and immune-mediated fibrosis. The clinical manifestations of this disease are extremely heterogeneous and depend on the presence and degree of internal organ involvement. Patients may present with a spectrum of illness ranging from localized skin fibrosis only (*localized scleroderma*) to a systemic disorder (*systemic scleroderma or systemic sclerosis*) with both cutaneous and internal organ involvement. The latter is the most severe with a median patient survival of 11 years (Mayes 2004) from diagnosis. Studies reflect there are approximately 80,000 systemic sclerosis patients in the U.S. with similar numbers in the EU.

About arGentis

[arGentis Pharmaceuticals, LLC](#) is a diversified growth-stage biopharmaceutical company located in Memphis, TN. The company seeks to in-license therapies for chronic diseases with demonstrated proof of concept for further development and commercialization. The company’s pipeline consists of mid- and late-stage platform technologies in both autoimmunity and ophthalmology. ARG201, the company’s lead compound for the treatment of systemic sclerosis, along with the ROT1 genetic biomarker highly associated with identifying patient responders to ARG201 therapy are being positioned for late phase clinical development. The ophthalmology pipeline includes three therapies for dry eye syndrome which are uniquely applied to the outer upper and lower eyelids for transdermal delivery to the affected glands.