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Clementia Pharmaceuticals Initiates Phase 2 Study of Palovarotene in Patients with Fibrodysplasia Ossificans Progressiva (FOP)

MONTREAL, CANADA and BOSTON, MA, July 14, 2014 (Marketwired) -- Clementia Pharmaceuticals, Inc. announced today the initiation of a Phase 2 clinical trial of palovarotene for the treatment of Fibrodysplasia Ossificans Progressiva (FOP). FOP is a rare, severely disabling genetic disease characterized by painful, recurrent episodes of soft tissue swelling (flare-ups) and abnormal bone formation in muscles, tendons, and ligaments. The new, abnormal bone formation, or heterotopic ossification (HO), causes morbidities and progressive disability.

Jeannie Peeper, President and Founder of the International Fibrodysplasia Ossificans Progressiva Association (IFOPA), stated, "Over the last several decades, the IFOPA has been focused on connecting families and researchers in the search for a cure. With this announcement, the work of the entire FOP community advances out of the realm of basic research and into the first of what we hope will be many controlled clinical trials, the next step in the ultimate goal of finding a successful treatment for FOP."

The double-blind, placebo-controlled Phase 2 clinical trial evaluates the effect of different doses of palovarotene on new bone formation during and after a flare-up in 24 patients with FOP who are 15 years of age or older. Treatment will be initiated within seven (7) days from flare-up onset and continue for six (6) weeks with an additional six (6) weeks of follow-up. A number of clinical endpoints will evaluate the efficacy and safety of palovarotene, including imaging endpoints for new bone formation and clinical assessments of physical function and patient-reported outcomes. "Ever since we first identified the mutation responsible for FOP, work has expanded throughout the world to find molecules that could block this target and one day lead to clinical trials," stated the trial principal investigator, Frederick S. Kaplan, M.D., the Isaac & Rose Nassau Professor of Orthopaedic Molecular Medicine and Chief of the Division of Molecular Orthopedic Medicine at the University of Pennsylvania School of Medicine. "Palovarotene, a retinoic acid receptor gamma agonist that appears to potently inhibit endochondral bone formation in animal models, is the first to enter well-controlled clinical trials."

Palovarotene prevented heterotopic bone formation in mouse models of FOP. This Phase 2 clinical trial was designed to determine whether the effects seen in these mouse models could be recapitulated in patients with FOP. "The adaptive design maximizes our chance of success in meeting the trial objectives in the shortest amount of time," stated Donna Grogan, M.D., Chief Medical Officer of Clementia. The trial will be conducted at three investigational sites: the University of Pennsylvania, the University of California, San Francisco, and the Hôpital Necker Enfants Malades, Paris. Please refer to www.clinicaltrials.gov (using the search term, "palovarotene") periodically for updated site information.

The Company expects to complete the trial during 2015. "We are privileged to announce the initiation of our first clinical trial in FOP. We could not have arrived at this point without the support and collaboration of countless members of the FOP community including scientists, physicians, and most importantly patients and their families, who inspire our work everyday," stated Clarissa Desjardins, Ph.D., Chief Executive Officer of Clementia. "We are committed to doing everything we can to support

the clinical development of palovarotene in FOP. As such, we will continue to invest our resources toward the ultimate goal of providing a potential new treatment to people living with FOP."

Additional information on the clinical trial can be found at www.clinicaltrials.gov (using the search term, "palovarotene") or at www.clementiapharma.com.

About Fibrodysplasia Ossificans Progressiva (FOP)

FOP is a rare, severely disabling disease characterized by painful, recurrent episodes of soft tissue swelling (flare-ups) that result in new, abnormal bone formation in muscles, tendons, and ligaments. Flare-ups begin early in life and may occur spontaneously or after soft tissue trauma, vaccinations, or influenza infections. Recurrent flare-ups progressively restrict movement by locking joints leading to cumulative loss of function and disability. FOP is caused by a point mutation in the ALK2/BMP Type I receptor rendering it overactive. Virtually all known patients have the same point mutation and have congenital malformations of the great toes at birth. FOP is thought to affect less than one individual for every million lives.

About Palovarotene

Palovarotene is a retinoic acid receptor gamma agonist in-licensed from Roche in 2013 where it was previously evaluated in over 800 individuals including healthy volunteers and patients with chronic obstructive pulmonary disease. Palovarotene has been shown to block bone formation in a variety of mouse models of heterotopic ossification and is being investigated as a potential treatment for FOP.

About Clementia Pharmaceuticals Inc.

Clementia is a privately held clinical stage biopharmaceutical company dedicated to the development and commercialization of treatments for people living with rare diseases by exploiting the science of novel retinoic acid receptor gamma agonists to address diseases of heterotopic ossification, including Fibrodysplasia Ossificans Progressiva. For more information, please visit www.clementiapharma.com.

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Source: Clementia Pharmaceuticals, Inc.

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