

Clementia Advances Natural History Study of Patients with Fibrodysplasia Ossificans Progressiva

Study Now Enrolling Patients Ages Two through 65 Years Old

MONTREAL, CANADA, May 28, 2015 – [Clementia Pharmaceuticals, Inc.](#) today announced that it has commenced enrollment in the second part (Part B) of its natural history study in patients with fibrodysplasia ossificans progressiva (FOP), a rare, severely disabling congenital myopathy characterized by painful, recurrent episodes of soft tissue swelling (flare-ups) that result in the formation of new, abnormal (heterotopic) bone in muscles, tendons and ligaments.

The multi-center, non-interventional, two-part longitudinal study is designed to measure disease progression over three years in patients with FOP. The study will evaluate the relationship between abnormal bone formation, which progressively restricts movement, and physical function as assessed by range of motion and patient-reported outcomes. Part B will enroll 40 patients from two to 65 years of age.

A committee comprised of FOP and imaging experts reviewed data from ten patients in the first part of the study (Part A), and determined that low-dose whole body CT scan (excluding head) provided better assessment of presence and amount of heterotopic bone throughout the body than bone density (DEXA) scans and CT scout scans. Based on these findings, low-dose whole body CT scan (excluding head) will be used in Part B to detect the presence and progression of heterotopic bone.

“The commencement of Part B of the natural history study brings us closer to our goal of advancing the global community’s understanding of FOP and developing a treatment for this debilitating disease,” said Donna Grogan, M.D., Chief Medical Officer of Clementia. “The enrollment of children will enable us to provide a more complete picture of FOP’s natural progression.”

The natural history study, which was designed in collaboration with investigators and with the assistance of the International FOP Association (IFOPA), is being conducted in parallel with Clementia’s interventional trials with palovarotene, an investigational agent for the prevention of abnormal bone formation following a flare-up. Importantly, data from the natural history study will be shared with the IFOPA to advance its mission of finding a cure for FOP.

The University of California, San Francisco, University of Pennsylvania, Hospital Italiano de Buenos Aires, Argentina and Gaslini Institute, Genoa are currently recruiting patients for this study. Please refer to www.clinicaltrials.gov (using the identifier number NCT02322255) periodically for details about the study and updated site information. For more information and

answers to frequently asked questions about palovarotene, please visit www.clementiapharma.com.

About Fibrodysplasia Ossificans Progressiva (FOP)

FOP is a rare, severely disabling congenital myopathy 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; the mutation results in over-activity of the receptor. Virtually all known patients have the same point mutation and have congenital malformations of the big 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 Pharmaceuticals, where it was previously evaluated in more than 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 FOP and is being investigated as a potential treatment for FOP.

About Clementia Pharmaceuticals, Inc.

Clementia is a privately held, clinical-stage biopharmaceutical company focused on developing and commercializing innovative therapies for people living with rare diseases. The company is advancing a novel retinoic acid receptor gamma agonist to address diseases of heterotopic ossification, including fibrodysplasia ossificans progressiva. For more information, please visit www.clementiapharma.com.

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