Clementia Clinical Program: Necker Begins Enrolling Patients in Palovarotene 2B Extension Study

November 18, 2016

MONTREAL, CANADA, November, 18, 2016 – Clementia is pleased to announce that the Hôpital Necker of Paris is ready to enroll FOP patients into Part B of its Phase 2 Open-label Extension Trial, which is a new study in France called PVO-1A-204.

Dr Geneviève Baujat is principal investigator at Necker trial site. Any adult or older adolescent subject, who meets the eligibility criteria can be enrolled into Part B including those currently participating in the Natural History Study. Eligible new participants for the trial at the Hôpital Necker must:

- Reside in France, due to regulatory requirements (subjects residing in the Argentina, Canada, US or UK may be able to enroll at sites in those countries)
- Have had at least two self-reported flare-ups in the last two years but cannot have had flare-up symptoms in the four weeks prior to enrollment
- Have achieved 90% skeletal maturity (if under age 18), which means that the bones are almost finished growing as measured by wrist x-ray at enrollment screening
- Have joint movement as assessed by the cumulative analogue joint involvement scale of 6 to 16, inclusive, demonstrating some limitation of movement, but sufficient movement to allow for participation in the trial
- Have the most common mutation, R206H, associated with FOP as confirmed by genetic testing performed at enrollment screening
- Be able to attend all scheduled site visits during the trial

More details and all enrollment criteria can be found on www.clinicaltrials.gov/ct2/show/NCT02979769.

Other clinical research sites that are hosting the study are the University of Pennsylvania in Philadelphia, the University of California San Francisco, the Royal National Orthopaedic Hospital in London, the Mayo Clinic, Rochester, Minnesota, and the Hospital Italiano, Buenos Aires.

We recognize and greatly appreciate the effort made by study participants and their families, the FOP community, and the clinical trial teams. None of this would be possible without your commitment. Our goal is to develop the evidence necessary to demonstrate the potential of palovarotene as a safe and effective treatment for FOP and the commencement of Part B moves us closer to that goal.

More information on palovarotene can be found at http://clementiapharma.com/our-pipeline/palovarotene/. Anyone interested in participating in this study should contact the clinical trial site closest to where they live.

About Fibrodysplasia Ossificans Progressiva (FOP)
FOP is a rare, severely disabling myopathy characterized by heterotopic ossification (HO) of muscle and soft tissues. Heterotopic bone formation progressively restricts movement by locking joints and leads to cumulative loss of function, disability, and increased risk of early death. Virtually all newborns with FOP have a hallmark toe malformation in which both big toes are shortened and bent inwards. FOP is caused by a mutation in the ACVR1 gene resulting in increased activity of the activin receptor type I (ALK2) involved in the bone morphogenic protein (BMP) pathway, a key pathway controlling bone growth and development. There are currently no approved treatments for FOP.

About Palovarotene
Palovarotene is a retinoic acid receptor gamma agonist being investigated as a treatment for FOP. Preclinical studies demonstrated that palovarotene blocked both injury-induced and spontaneous heterotopic ossification, maintained mobility, and restored skeletal growth in mouse models of FOP. Clementia licensed palovarotene from Roche Pharmaceuticals, which previously evaluated the compound in more than 800 subjects. Palovarotene received Fast Track designation from the U.S. Food and Drug Administration (FDA) and orphan designations for the treatment of FOP from both the FDA and the European Medicines Agency (EMA).

About Clementia Pharmaceuticals Inc.
Clementia is a clinical stage biopharmaceutical company committed to delivering treatments to people who have none. The company is developing its lead candidate palovarotene, a novel retinoic acid receptor gamma agonist, to treat fibrodysplasia ossificans progressiva (FOP) and other diseases. For more information, please visit www.clementiapharma.com.