

## **Clementia Clinical Program: Next Step in the Clementia's Clinical Program for Fibrodysplasia Ossificans Progressiva (FOP)**

**MONTREAL, CANADA, June 13, 2016** – Clementia is pleased to announce that the Phase 2 Open-label Extension Trial (PVO-1A-202) has been modified to enroll up to 20 new participants and to investigate new palovarotene dosing regimens in participants with fibrodysplasia ossificans progressiva (FOP). The modification to the Phase 2 Open-label extension trial is designated as Part B.

The Phase 2 Trial (Study PVO-1A-201), which is now complete, was designed as an exploratory dose-ranging study that examined the safety and efficacy of two different dosing regimens of palovarotene in participants for acute flare-up. All 40 individuals who completed the Phase 2 trial have enrolled into the Phase 2 Open-label Extension Trial, which provides access to palovarotene to any participant experiencing an eligible flare-up and continues to evaluate the long-term safety and efficacy of palovarotene.

Much has been learned from these studies. Emerging data suggests that the risk to develop heterotopic ossification may not be the same for all flare-ups. This and other learnings point to the benefit of continued dose exploration, which is paramount to move palovarotene to the next step in Clementia's clinical program for FOP.

Part B is designed to explore a modified dosing regimen, which includes chronic dosing between flare-ups and acute dosing during an eligible flare-up that is higher in dose and longer in duration than what was studied in the Phase 2 trial. Chronic dosing helps to ensure that drug is present before recognition of flare-up symptoms and has been successfully used in other diseases with flare-up or acute presentations. Chronic dosing will first be evaluated in adults and nearly grown teenagers before it can be used in actively growing children. All participants will receive acute dosing for eligible flare-ups. In order to evaluate these new dosing regimens as quickly and efficiently as possible, the trial is now enrolling 20 new adults as well as teenagers who are nearly grown. Due to regulatory requirements, new participants must reside in the US, Canada, UK, or France.

All four clinical trial sites, the University of Pennsylvania in Philadelphia, the University of California San Francisco (UCSF), the Necker Institute in Paris France and the Royal National Orthopaedic Hospital in London United Kingdom, will initiate enrollment once the necessary approvals are received.

Together, we have greatly enhanced our knowledge of FOP. At Clementia, we recognize and appreciate the effort made by and are grateful to the study participants and their families, the FOP community and the clinical trial teams without whom none of this would be possible. 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, including detailed enrollment criteria, can be found at [www.clinicaltrials.gov/ct2/show/NCT02279095](http://www.clinicaltrials.gov/ct2/show/NCT02279095) and [www.orpha.net](http://www.orpha.net). Anyone interested in participating in this study should contact the clinical trial site closest to where they live. Contact information can be found at <http://clementiapharma.com/clinical-trials/participating-clinical-trial-sites/>.