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Dear MO Community,

Last April we were just about to launch our MO-Ped Trial (**M**ultiple **O**steochondromas **Ped**iatric Trial), the first ever study to evaluate a potential therapy for MO. I am pleased to report to you that MO-Ped is underway at 15 clinical trial sites in 4 countries, and we are well on our way to expanding enrolment to 12 countries around the world.

MO-Ped is the first clinical trial to study MO in a systematic manner. The primary endpoint of MO-Ped is the rate of new osteochondromas (OCs) as measured by whole body MRI. Several other clinical outcome measures include the volume of OCs, the number of surgeries, the number of new or worsening deformities and functional limitations as well as quality of life and pain measures. This first clinical trial in MO will therefore serve as a reference for generations to come and will likely facilitate further research and understanding of MO.

I want to convey my sincere thanks to those of you participating in MO-Ped. Your dedication will benefit the entire MO community. We know there are still many of you anxiously awaiting the opening of new clinical sites and we are working diligently with these sites to ensure that they are ready to begin and expand recruitment as soon as possible. With your assistance, we hope to complete enrollment in this important study around mid 2019.

We also want you to know that beyond MO-Ped, our upcoming filing to seek US FDA approval of palovarotene for FOP may have implications for the MO community. First, our studies and our discussions with regulators have resulted in many learnings that may also help rapidly advance investigational palovarotene as a potential treatment for MO. Second, all of our preparations for a potential FOP approval and eventual commercial launch, from building a global supply chain, scaling up manufacturing of palovarotene, to hiring people to help serve patients worldwide, will be the foundation for serving the MO community in the future. I am very proud of the team we are building, and I look forward to the day that they can assist you as well.

In a few weeks, on February 28th, communities around the world will recognize Rare Disease Day 2019 and reflect on just how many “rare” individuals there are, and how much need there is for new treatments for the thousands of rare disorders, like MO. Thank you for engaging with us on this pioneering journey as we help educate the world on MO, and as we resolutely advance with the MO-Ped trial.

All the best to you and your families in 2019,

Clarissa Desjardins