



August 3, 2020

Dear MO Community,

We would like to inform you that we have made the very difficult decision to end the multiple osteochondromas (MO) clinical development program for palovarotene due to insufficient efficacy signals in the analysis of the Phase 2 MO-Ped (PVO-2A-201) trial. It was not an easy decision to make, and we realize that our MO Program provided hope for so many living with the condition.

We are grateful for the engagement of the community of patients, families, researchers and clinicians who supported the program through research, disease awareness and participating in the clinical trial. While this outcome was not what we had hoped for, we are optimistic that the knowledge gained from the trial will advance the collective understanding of the disease and potential future treatments. We are committed to sharing the data from the MO-Ped trial once it is complete with the broader community in a publication or at a scientific meeting.

In March 2020, we shared our decision to terminate the MO-Ped trial to analyze the accumulated data and evaluate the efficacy, safety and future of palovarotene in MO. Our hope was to establish a path forward for palovarotene in MO, including an assessment of its potential for submission to worldwide regulatory agencies. During this evaluation, we unfortunately did not observe a signal of efficacy that would warrant the initiation of a new MO trial and continuation of the program.

We are working with the clinical study investigators to continue post-treatment follow-up visits of clinical trial participants within the MO-Ped trial to complete the protocol-specified safety assessments and close out the study. If study participants have questions, they should contact their physician or clinical trial investigator.

It is our hope that the learnings from the MO-Ped trial will be used by researchers, the community and other companies to continue the journey toward a potential treatment.

Sincerely,

Jim Roach, MD

Senior Vice President and Global Head, Rare Diseases Therapeutic Area

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