December 12, 2019

Dear FOP and MO Communities,

As you may be aware, following discussions with the U.S. Food and Drug Administration (FDA), a partial clinical hold for children under the age of 14 was issued for our Phase 2 and Phase 3 clinical trials evaluating investigational palovarotene for the chronic treatment of fibrodysplasia ossificans progressiva (FOP) and multiple osteochondromas (MO). This action was taken following recent safety reports submitted by Ipsen to the FDA of cases of early growth plate closure in some children with FOP who received investigational drug palovarotene.

The FDA is allowing the FOP clinical trials to continue in study patients ages 14 and older. Although these serious adverse events have not been observed to date in the MO trial, the FDA included this study in the partial clinical hold because early growth plate closure has occurred with chronic dosing in the FOP program. Since the MO trial is a pediatric study with the upper age of enrollment at 14 years, the decision was made to stop dosing for all study patients currently participating in the trial, and no new study patients will be enrolled while the partial clinical hold is in effect.

The safety of our patients is our top priority, and we know that parents, study patients and caregivers place a large degree of trust in us when they participate in any clinical trial. Developing potentially life-changing treatments sometimes comes with unexpected challenges, and we are working diligently with the FDA to provide all requested information with the goal of resolving the partial clinical hold. We continue to prepare to file the FDA New Drug Application (NDA) for the acute/flare-up dose regimen in patients with FOP.

This is difficult news and we share in your concern and disappointment. Every day poses challenges for those living with FOP and MO, and we understand the urgency in searching for a treatment. We want to reiterate our commitment to researching and developing therapies for children and adults living with FOP and MO.

We will continue to provide updates as information becomes available. If you have further questions, please consult your treating physician.

We thank you – study patients, parents and caregivers – for the trust you have placed in us.

Sincerely,

Dr. Howard Mayer
Executive Vice President and Head of Research and Development

Dr. Jim Roach
Senior Vice President and Global Head, Rare Diseases Therapeutic Area