We would like to share with the global fibrodysplasia ossificans progressiva (FOP) community the following update on the U.S. Food and Drug Administration (FDA) review of palovarotene.

Dear FOP community,

Ipsen has today announced, following very recent discussions with the U.S. FDA that we will be withdrawing the New Drug Application (NDA) for palovarotene currently in review. This decision by Ipsen follows ongoing dialogue with the FDA since the NDA was accepted for a priority review in May 2021. Ipsen plans to resubmit the NDA as soon as possible.

During the NDA review, it was recognized that additional analyses and evaluation of the data from the Phase III MOVE clinical trial and FOP program would be required to progress and complete the review process. It was agreed between Ipsen and the FDA that it would not be possible to complete these additional analyses within the current NDA review cycle – estimated to complete in November 2021. Ipsen has therefore confirmed the withdrawal of the NDA for palovarotene with plans to resubmit to the FDA upon completion of the additional analyses and evaluations. The additional analyses requested are not related to the safety of palovarotene.

The FDA’s process for reviewing investigational medicines is intended to ensure a robust review of the data available to inform a license decision. After the FDA accepts an NDA, the review process involves regular dialogue between FDA and the company submitting the NDA. The final decision of the FDA is released as a PDUFA. Due to the complexity of the data and the ultra-rare nature of FOP, the FDA had confirmed an Advisory Committee meeting would take place as part of this process for palovarotene. Advisory Committees include detailed data discussion with a panel of experts, and a public hearing so that members of the community can participate and contribute. With the withdrawal of the NDA, the palovarotene Advisory Committee will not take place this fall as originally planned, rather it will be re-scheduled. The FDA has the option to hold Advisory Committees, and future plans for a palovarotene Advisory Committee are unknown.

Dr. Howard Mayer, Executive Vice President and Head of Research and Development, Ipsen, said “We remain committed to the FOP community through our clinical programs for Ipsen’s two investigational therapies palovarotene and IPN60130. We recognize the urgency from this community to bring a much-needed treatment option to people living with FOP around the world. Unfortunately, as there is no regulatory mechanism to “pause” the current ongoing review process, we have taken the decision to withdraw the NDA for palovarotene to undertake the additional analyses and evaluation needed, with plans to resubmit the data for palovarotene as soon as possible.”

We recognize this news may be disappointing, but we want to reinforce our commitment to the FOP community and thank the entire community again for their ongoing support and participation in the clinical programs for both palovarotene and IPN60130 as well as the natural history study. Our teams are working hard to explore the additional analyses needed with the aim of re-submitting the palovarotene data to the FDA as soon as possible.

If you have any questions about this please do not hesitate to contact your healthcare team, or if you wish to submit a question directly to Ipsen please contact our US Medical Information Group at: 855-463-5127 or email to: medinfo.USA@ipsen.com.

We also want to thank the FDA for their continued collaboration. We will be sure to keep you informed of the next steps when those details are available and continue to provide regular updates on these processes as they progress.

Best regards,

Jennifer Schranz
Senior Vice President
Global Head Rare Disease, Research & Development