REGENERON
SCIENCE TO MEDICINE®

8/13/2021

Dear FOP Community,

Since our last communication of June 1, 2021, we have an update on the garetosmab FOP program.

We met with the U.S. Food and Drug Administration to discuss the garetosmab program, which the agency had placed on clinical hold.

Following alignment with the FDA, and in order to lift garetosmab off clinical hold, Regeneron has initiated plans for a Phase 3 confirmatory trial of garetosmab in people living with FOP. It is expected in most diseases that regulatory authorities will require data from confirmatory Phase 3 trials in order to learn more about how people react to the drug, as well as confirm the data observed in earlier trials.

Based on the FDA's feedback, and the current clinical hold on garetosmab, the LUMINA-1 trial will be discontinued. We are committed to sharing scientific data and are currently preparing a publication of LUMINA-1 results to submit to a peer-reviewed scientific journal.

We expect the Phase 3 trial will commence enrollment in 2022, and we will continue to collaborate with the FOP community as we finalize plans and begin recruitment. We will keep in mind the participants of the LUMINA-1 clinical trial, when designing the Phase 3 study, but this will require further discussion and agreement, with the FDA and other regulatory authorities.

Regeneron has been engaged in FOP research for more than two decades and helped to provide fundamental insights in the biology and natural history of the disease. We remain committed to finding safe and effective treatment solutions for people living with FOP, including the potential for garetosmab.

We are extremely grateful to the medical and FOP community for the support of the garetosmab clinical program and we look forward to continuing to work with the community in planning this program. We will continue to keep the leadership of the IFOPA, National FOP organizations and the community apprised of significant updates as they arise.

Amanda Seeff-Charny, Executive Director, Patient Advocacy, Regeneron Pharmaceuticals, Inc.
Andrew Rankin, Vice President Clinical Development, Garetosmab Global Program Head, Regeneron Pharmaceuticals, Inc.
Eduardo Forleo Neto, MD, LUMINA-1 Executive Medical Director, Clinical Sciences, Global Development, Regeneron Pharmaceuticals, Inc.