

# REGENERON

October 30, 2020

## **Regeneron Provides Update on the Garetosmab Phase 2 LUMINA-1 Trial in Fibrodysplasia Ossificans Progressiva (FOP)**

Regeneron today notified clinical investigators to pause dosing of the investigational therapy garetosmab (REGN2477) in the ongoing Phase 2 LUMINA-1 trial in patients with the ultra-rare genetic disorder fibrodysplasia ossificans progressiva (FOP). The decision was based on reports of fatal serious adverse events in the trial during the open-label extension, during which all patients received active treatment. These deaths are being further investigated to understand if they are related to garetosmab treatment. During the 28-week double-blind treatment period, there were no deaths in the trial.

Regeneron also shared this update with the trial's Independent Data Monitoring Committee and regulatory authorities, and will conduct a review of the trial data to date, to better understand the benefit/risk profile of garetosmab in people with FOP. Regeneron [announced](#) topline 28-week results from the 44-patient LUMINA-1 trial earlier this year; this is the only active trial evaluating garetosmab.

### **About Fibrodysplasia Ossificans Progressiva (FOP)**

Fibrodysplasia ossificans progressiva (FOP) is a relentless, progressive, ultra-rare genetic disorder in which muscles, tendons and ligaments are progressively replaced by bone, a process known as heterotopic ossification (HO). There are believed to be approximately 900 patients diagnosed with FOP worldwide. HO of the jaw, spine, hip and rib cage can make it difficult to speak, eat, walk or breathe, leading to weight loss and escalating loss of mobility and skeletal deformity. Most people with FOP are wheelchair bound by 30 years old and the median age of survival is approximately 56 years. Death often results from complications, such as pneumonia, heart failure and aspiration stemming from HO and loss of mobility in the chest, neck and jaw.

### **Regeneron Forward-Looking Statements and Use of Digital Media**

*This statement includes forward-looking statements that involve risks and uncertainties relating to future events and the future performance of Regeneron Pharmaceuticals, Inc. ("Regeneron" or the "Company"), and actual events or results may differ materially from these forward-looking statements. Words such as "anticipate," "expect," "intend," "plan," "believe," "seek," "estimate," variations of such words, and similar expressions are intended to identify such forward-looking statements, although not all forward-looking statements contain these identifying words. These statements concern, and these risks and uncertainties include, among others, the nature, timing, and possible success and therapeutic applications of Regeneron's products and product candidates and research and clinical programs now underway or planned, including without limitation garetosmab (REGN2477); safety issues resulting from the administration of Regeneron's product*

*candidates (such as garetosmab) in patients, including serious complications or side effects in connection with the use of Regeneron's product candidates in clinical trials such as the fatal serious adverse events in the Phase 2 LUMINA-1 trial evaluating garetosmab discussed in this statement; the likelihood, timing, and scope of possible regulatory approval and commercial launch of Regeneron's product candidates, such as garetosmab; determinations by relevant authorities which may delay or restrict Regeneron's ability to continue to develop or commercialize Regeneron's product candidates, such as garetosmab; and the extent to which the results from the research and development programs conducted by Regeneron and/or its collaborators (such as the garetosmab program) may lead to advancement of product candidates to clinical trials, therapeutic applications, or regulatory approval. A more complete description of these and other material risks can be found in Regeneron's filings with the U.S. Securities and Exchange Commission, including its Form 10-Q for the quarterly period ended June 30, 2020. Any forward-looking statements are made based on management's current beliefs and judgment, and the reader is cautioned not to rely on any forward-looking statements made by Regeneron. Regeneron does not undertake any obligation to update (publicly or otherwise) any forward-looking statement, including without limitation any financial projection or guidance, whether as a result of new information, future events, or otherwise.*

*Regeneron uses its media and investor relations website and social media outlets to publish important information about the Company, including information that may be deemed material to investors. Financial and other information about Regeneron is routinely posted and is accessible on Regeneron's media and investor relations website (<http://newsroom.regeneron.com>) and its Twitter feed (<http://twitter.com/regeneron>).*

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