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Mirum Pharmaceuticals and Incyte Announce Pivotal Late-Breaking Results for Zilurgisertib in Fibrodysplasia Ossificans Progressiva Accepted for Presentation at ENDO 2026

June 4, 2026

- Late-breaking rapid-fire presentation to include results from Cohort 1 of the placebo-controlled PROGRESS study evaluating zilurgisertib in fibrodysplasia ossificans progressiva (FOP)

FOSTER CITY, Calif. & WILMINGTON, Del.--(BUSINESS WIRE)--Jun. 4, 2026-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM) and Incyte (Nasdaq: INCY) today announced that pivotal Phase 2 results from the PROGRESS study evaluating zilurgisertib, an investigational ALK2 inhibitor, in patients with fibrodysplasia ossificans progressiva ("FOP") will be presented at ENDO 2026, the Endocrine Society's annual meeting, taking place June 13-16, 2026, in Chicago, Illinois.

This press release features multimedia. View the full release here: <https://www.businesswire.com/news/home/20260604863826/en/>

The late-breaking rapid-fire presentation will include results from Cohort 1 of the placebo-controlled PROGRESS study, which enrolled patients 12 years of age and older and formed the basis of the New Drug Application (NDA) for zilurgisertib to the U.S. Food and Drug Administration (FDA).

In April 2026, Mirum entered into an exclusive license agreement with Incyte for worldwide rights to zilurgisertib.

"ENDO 2026 is an important milestone for the zilurgisertib FOP program as we share pivotal results from the PROGRESS study in patients living with this debilitating disease," said Steven Stein, M.D., Executive Vice President, Chief Medical Officer and Head of Late-stage Development at Incyte. "These data add to the growing clinical understanding of zilurgisertib's potential in FOP as Incyte and Mirum continue to advance toward the FDA's Priority Review PDUFA date of September 26, 2026."

"FOP is a devastating, progressive disease that profoundly impacts patients and families," said Joanne Quan, M.D., Chief Medical Officer at Mirum Pharmaceuticals. "At Mirum, in partnership with Incyte, we are committed to advancing zilurgisertib with urgency as we work toward potentially bringing a needed new treatment option to people living with FOP."

Congress Presentation

Additional details regarding the presentation are as follows:

Abstract #	ORF37-04
Title	Zilurgisertib in Patients with Fibrodysplasia Ossificans Progressiva: Interim Results from the PROGRESS Study
Date/Time	Sunday, June 14, 3:30-4:15 pm CT

Abstracts accepted for presentation at ENDO 2026 will be available once published through the Endocrine Society's [ENDO 2026 website](#).

About Zilurgisertib

Zilurgisertib is an investigational, oral, small molecule, activin receptor-like kinase 2 (ALK2) inhibitor in development for the treatment of Fibrodysplasia Ossificans Progressiva (FOP). Zilurgisertib is designed to inhibit the ALK2 receptor, which is abnormally active in most patients with FOP and leads to bone formation in soft tissues, a process known as heterotopic ossification (HO). FOP is an ultra-rare genetic disease that affects approximately 300 patients in the U.S. and 900 worldwide, with diagnosis typically occurring in early childhood. Zilurgisertib was evaluated in the PROGRESS pivotal Phase 2 study, which formed the basis of a new drug application (NDA). The FDA has accepted the NDA for zilurgisertib in FOP under Priority Review with a Prescription Drug User Fee Act (PDUFA) date of September 26, 2026.

Mirum licensed zilurgisertib from Incyte for development and commercialization globally.

About the PROGRESS Study

PROGRESS is a global, randomized, double-blind, placebo-controlled Phase 2 study evaluating the efficacy and safety of zilurgisertib in patients with fibrodysplasia ossificans progressiva (FOP). PROGRESS Cohort 1 enrolled patients 12 years of age and older who were randomized 1:1 to receive zilurgisertib 100 mg once daily or placebo during a 24-week double-blind treatment period, followed by an open-label extension. Additional PROGRESS cohorts will evaluate the efficacy and safety of zilurgisertib in patients ages 6 to <12 years of age (Cohort 2) and in patients ages 2 to <12 years of age (Cohort 3).

The primary endpoint of the study is the proportion of Cohort 1 patients with new heterotopic ossification (HO) lesions at Week 24 as assessed by whole-body CT scan data. Key secondary endpoints include the number and total volume of new HO lesions, changes in total HO lesion volume and flare activity through Week 24.

About Mirum Pharmaceuticals

Mirum Pharmaceuticals (NASDAQ: MIRM) is a leading rare disease company with a global footprint of approved products and a broad pipeline of investigational medicines. Purpose-built to bring forward breakthrough medicines for people with overlooked conditions, Mirum focuses on rare liver and rare genetic diseases, where it has built deep expertise and strong connections to patient communities. The company's commercial portfolio includes LIVMARLI® (maralixibat) for Alagille syndrome (ALGS) and progressive familial intrahepatic cholestasis (PFIC), CHOLBAM® (cholic acid) for bile-acid synthesis disorders, and CTEXLI® (chenodiol) for cerebrotendinous xanthomatosis (CTX).

Mirum's clinical-stage pipeline includes volixibat, an IBAT inhibitor in late-stage development for primary sclerosing cholangitis (PSC) and primary biliary cholangitis (PBC), brelovitug, a fully human monoclonal antibody in late-stage development for chronic hepatitis delta virus (HDV), zilurgisertib, an ALK2 inhibitor under regulatory review with the FDA for fibrodysplasia ossificans progressiva (FOP), and MRM-3379, a PDE4D inhibitor being evaluated for Fragile X syndrome (FXS).

Mirum's success is driven by a team dedicated to advancing high impact medicines through strategic development, disciplined execution and purposeful collaboration across the rare disease ecosystem. Learn more at www.mirumpharma.com and follow Mirum on [Facebook](#), [LinkedIn](#), [Instagram](#) and [X](#).

About Incyte®

Incyte is redefining what's possible in biopharmaceutical innovation. Through deep scientific expertise and a relentless focus on patients, we have built an established portfolio of first-in-class medicines and an extensive portfolio of next-generation medicines across our key franchises: Hematology, Oncology and Inflammation & Autoimmunity.

To learn more, visit Incyte.com and Investor.Incyte.com. Follow us on social media: [LinkedIn](#), [X](#) and [Instagram](#).

Mirum Forward-Looking Statements

Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements include statements regarding, among other things, the Company's planned participation at a scientific congress, Mirum's continued advancement of zilurgisertib with Incyte, the FDA approval pathway for zilurgisertib and the potential benefit of zilurgisertib in real world settings versus scientific presentations of data. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Words such as "expected," "will," "could," "would," "guidance," "potential," "continue" and similar expressions are intended to identify forward-looking statements. These forward-looking statements are based upon Mirum's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include, without limitation, risks and uncertainties associated with Mirum's business in general, the impact of geopolitical and macroeconomic events, and the other risks described in Mirum's Annual Report for the year ended December 31, 2025, filed with the Securities and Exchange Commission on February 25, 2026, and subsequent filings with the Securities and Exchange Commission, which are available at www.sec.gov. All forward-looking statements contained in this press release speak only as of the date on which they were made and are based on management's assumptions and estimates as of such date. Mirum undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made, except as required by law.

Incyte Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995 and other federal securities laws, including statements regarding the potential and promise suggested by the Phase 2 PROGRESS results, the potential for zilurgisertib to become a treatment option for people living with FOP, Incyte's plans and expectations for the PROGRESS study and Incyte's aspirations and goals as set forth under the heading "About Incyte".

Actual results may differ materially from those indicated in the forward-looking statements as a result of various important factors, including the sufficiency of clinical trial data to meet applicable regulatory standards or warrant continued development; the ability to enroll sufficient numbers of subjects in clinical trials; actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials and marketing approval; the ability of Incyte's collaborators to achieve commercial success for their marketed products and product candidates, if approved; Incyte's and Incyte's collaborators' ability to obtain and maintain protection of intellectual property for their products and technology; Incyte's reliance on third parties and partners; the acceptance of Incyte's collaborators' products in the marketplace; market competition, sales, marketing, manufacturing and distribution requirements; and those risks and uncertainties discussed in greater detail in Incyte's reports filed with the U.S. Securities and Exchange Commission, including its annual report on Form 10-K and its quarterly report on Form 10-Q for the quarter ended March 31, 2026. Incyte disclaims any intent or obligation to update these forward-looking statements.

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