Mirum Pharmaceuticals and Incyte Announce U.S. FDA Approval of Atebrioz™ (zilurgisertib) for Adult and Pediatric Patients with Fibrodysplasia Ossificans Progressiva

September 25, 2026

-Once-daily oral ALK2 inhibitor approved to reduce the volume of total new heterotopic ossification in patients aged 12 years and older with FOP

-Atebrioz expected to be available in the United States in October through Mirum Access Plus (MAP), with eligible patients paying as little as $0 per month

-PROGRESS pediatric development program continues to evaluate zilurgisertib in children aged 2 to <12 years

FOSTER CITY, Calif. & WILMINGTON, Del.--(BUSINESS WIRE)--Sep. 25, 2026-- Mirum Pharmaceuticals, Inc. (Nasdaq:MIRM) and Incyte (Nasdaq:INCY) today announced that the U.S. Food and Drug Administration (FDA) has approved Atebrioz™ (zilurgisertib) tablets to reduce the volume of total new heterotopic ossification (HO) in adult and pediatric patients aged 12 years and older with fibrodysplasia ossificans progressiva (FOP). The recommended dose of Atebrioz is 100 mg administered orally, once daily.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20260925436454/en/

“Today marks an important milestone for people living with FOP, bringing a new treatment option to adult and pediatric patients living with this devastating disease,” said Chris Peetz, Chief Executive Officer at Mirum. “At Mirum, we are driven to serve rare disease communities where the unmet need is significant and the opportunity to make a difference is profound. The approval of Atebrioz reflects what can be achieved when industry, researchers and patient communities work together, and we remain committed to fostering that spirit of collaboration in FOP.”

Atebrioz was developed by Incyte and licensed to Mirum Pharmaceuticals, Inc. for worldwide development and commercialization.

Atebrioz is a once-daily oral activin receptor-like kinase 2 (ALK2) inhibitor designed to target the disease-driving pathway at the center of FOP biology. In people living with FOP, pathogenic variants in the ACVR1 gene result in the abnormal activation of ALK2, leading to the formation of bone in muscles, tendons, ligaments and other soft tissues through a process known as heterotopic ossification (HO). As HO lesions develop and accumulate over time, they can progressively restrict movement and lead to significant disability.

“For families living with FOP, having additional treatment options means having greater flexibility in managing a complex, lifelong disease,” said Michelle Davis, Executive Director at the International Fibrodysplasia Ossificans Progressiva Association (IFOPA). “Every person’s experience with FOP is different, and expanding treatment options gives patients, families and their physicians the opportunity to consider what may be right for their individual needs.”

“FOP is a lifelong disease in which the accumulation of HO leads to increasing disability and loss of function,” said Robert Pignolo, M.D., Ph.D., Robert and Arlene Kogod Professor of Geriatric Medicine at the Mayo Clinic College of Medicine and lead investigator for the PROGRESS study. “Having another treatment option is meaningful in a progressive disease like FOP, particularly for adolescents who may be earlier in the course of their disease.”

Atebrioz was approved based on data from Cohort 1 of the PROGRESS study evaluating zilurgisertib in adult and pediatric patients aged 12 years and older with FOP. Efficacy was established based on total new HO lesion volume. Total new HO lesion volume includes expansion of baseline HO lesion burden as well as any new discrete HO that developed during the 24-week double-blind period. At Week 24, mean total new HO lesion volume decreased by 3.2 cm^3 in patients receiving zilurgisertib compared with an increase of 24.6 cm^3 in placebo-treated patients. Treatment effects were maintained through Week 48 of the open-label extension.

Zilurgisertib was generally well tolerated during the 24-week placebo-controlled period of the study. The most common adverse reactions were headache, arthralgia, upper respiratory tract infection, epistaxis and nausea. Most adverse events were mild or moderate in severity, and no adverse events led to treatment discontinuation or dose reduction.

Atebrioz will be available through Mirum Access Plus (MAP), a patient support program designed to help patients, families and healthcare providers navigate treatment access. MAP provides insurance coverage and access support, financial assistance for eligible patients, personalized patient support and educational resources for patients and caregivers. Atebrioz is expected to be commercially available in the U.S. in October, with eligible patients paying as little as $0 per month through MAP. To learn more about MAP, call 855-MRM-4YOU (1-855-676-4968).

With this approval, the FDA also issued a Rare Pediatric Disease Priority Review Voucher (PRV) to Incyte. The voucher can be used for a subsequent drug application that would not otherwise qualify for a priority review.

In the European Union, a marketing authorization application (MAA) for zilurgisertib is currently under review by the European Medicines Agency (EMA), supported by data from Cohort 1 (patients aged 12 years and older) of the PROGRESS study.

The PROGRESS development program also continues to advance, with enrollment completed in Cohort 2 of children aged 6 to <12 years, and enrollment underway in Cohort 3 of children aged 2 to <12 years.

About Atebrioz™ (zilurgisertib) tablets

Atebrioz™ (zilurgisertib) tablets are a once-daily oral activin receptor-like kinase 2 (ALK2) inhibitor approved by the U.S. Food and Drug Administration (FDA) to reduce the volume of total new heterotopic ossification in adult and pediatric patients aged 12 years and older with Fibrodysplasia Ossificans Progressiva (FOP). In people living with FOP, pathogenic variants in the ACVR1 gene result in abnormal activation of ALK2, leading to the formation of

bone in muscles, tendons, ligaments and other soft tissues through a process known as heterotopic ossification (HO).

Mirum Pharmaceuticals, Inc. licensed zilurgisertib from Incyte for worldwide development and commercialization.

IMPORTANT SAFETY INFORMATION

Atebrioz can cause fetal harm based on data from animal studies. Patients of reproductive potential should use effective contraception and should immediately discontinue Atebrioz and contact their healthcare provider if pregnancy occurs.

US Prescribing Information

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 63 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. Results from Cohort 1 have been reported through Week 48.

The PROGRESS pediatric development program is evaluating the safety and efficacy of zilurgisertib in younger patients with FOP, including patients aged 6 to <12 years in Cohort 2 and patients aged 2 to <12 years in Cohort 3.

About Fibrodysplasia Ossificans Progressiva (FOP)

Fibrodysplasia ossificans progressiva (FOP) is an ultra-rare, progressive genetic disease affecting approximately 300 people in the United States and 900 worldwide. FOP is characterized by heterotopic ossification (HO), a process in which bone forms in muscles, tendons, ligaments and other soft tissues. Symptoms typically become apparent in early childhood and the number and volume of HO lesions increase over time, progressively restricting movement and limiting mobility, daily function, and independence.

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), Atebrioz™ (zilurgisertib) for fibrodysplasia ossificans progressiva (FOP), 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) 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, Mirum's continued advancement of zilurgisertib with Incyte, expectations regarding the timing or results of clinical trials for zilurgisertib, the potential benefit of zilurgisertib in real world settings versus clinical trial settings, the importance of an additional therapy for the treatment of FOP, and the expected commercial availability of Atebrioz™, including the expected timing, cost to patients and methods of availability. 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 whether and when Atebrioz will become available as a treatment option for patients with FOP; the potential offered by Atebrioz for patients with FOP; expectations regarding the cost of Atebrioz for eligible patients; expectations regarding ongoing and future clinical trials for zilurgisertib, including the PROGRESS development program; expectations regarding the regulatory review of the MAA for

zilurgisertib by the EMA; 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 and the ability to enroll subjects in accordance with planned schedules; actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials and marketing approval; the efficacy or safety of Incyte's and its partners' products; the ability of Incyte and its partners to achieve commercial success for their marketed products and product candidates, if approved; Incyte's and its partners' 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 and its partners' products in the marketplace; market competition, sales, marketing, manufacturing and distribution requirements; greater than expected expenses, including expenses relating to litigation or strategic activities; 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 for the year ended December 31, 2025, and its quarterly report on Form 10-Q for the quarter ended June 30, 2026. Incyte disclaims any intent or obligation to update these forward-looking statements.

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