UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): September 25, 2026

Mirum Pharmaceuticals, Inc.
(Exact name of Registrant as Specified in Its Charter)

Delaware	**001-38981**	**83-1281555**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**
989 East Hillsdale Boulevard Suite 300		
Foster City, California		**94404**
(Address of Principal Executive Offices)		**(Zip Code)**

Registrant's Telephone Number, Including Area Code: (650) 667-4085

N/A
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common stock, par value $0.0001 per share	MIRM	Nasdaq Global Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 8.01 Other Events.

Approval of Atebrioz

On September 25, 2026, Mirum Pharmaceuticals, Inc. (the "Company") and Incyte Corporation ("Incyte") announced that the U.S. Food and Drug Administration ("FDA") 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.

Atebrioz was developed by Incyte and licensed to the Company 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 HO. As HO lesions develop and accumulate over time, they can progressively restrict movement and lead to significant disability.

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 cm3 in patients receiving zilurgisertib compared with an increase of 24.6 cm3 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.

In the European Union, a marketing authorization application for zilurgisertib is currently under review by the European Medicines Agency, 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.

Announcement of Data from the Phase 3 AZURE-1 Study of Brelovitug in Chronic Hepatitis Delta

On September 28, 2026, the Company announced the primary endpoint was met in the Phase 3 portion of the AZURE-1 study evaluating brelovitug, an investigational fully human monoclonal antibody that binds to hepatitis B surface antigen (HBsAg), for the treatment of chronic hepatitis delta virus ("HDV"). In addition, 48-week data from the Phase 2b portion of AZURE-1 demonstrated deepening viral suppression, with a greater proportion of patients achieving HDV RNA below the lower limit of quantification (LLOQ, <10 IU/ml) and target not detected ("TND"), and increased rates of alanine aminotransferase ("ALT") normalization. AZURE-1 is one of two pivotal Phase 3 studies intended to form the basis of the Company's U.S. registration package for brelovitug.

Phase 3 AZURE-1 Results: Brelovitug Met the Primary Endpoint at Week 24

The Phase 3 portion of the AZURE-1 study enrolled 153 treatment-naive patients randomized in a 2:2:1 ratio to receive brelovitug 300 mg self-administered once weekly ("QW") by subcutaneous ("SC") injection , brelovitug 900 mg administered once every four weeks ("Q4W") by SC injection or delayed treatment starting at Week 24. This global study enrolled a broad patient population including patients with advanced disease.

At Week 24, treatment with brelovitug met the primary endpoint, with 56% of patients in the 300 mg QW arm and 45% in the 900 mg Q4W arm achieving the combined endpoint of virologic response (≥2 log10 reduction in HDV RNA from baseline or undetectable HDV RNA [<LLOQ, TND]) and ALT normalization, compared to 0% in the delayed treatment arm ($p<0.0001$ for both comparisons). The efficacy results by treatment arm at Week 24 are presented below in Phase 3 Key Efficacy Endpoints.

Treatment with brelovitug was well tolerated across dose groups, with a safety profile consistent with previously reported AZURE-1 data. The safety profile summary is presented below in Phase 3 Summary of Safety Through Week 24.

Phase 3 Key Efficacy Endpoints

Endpoint	24 Weeks		
	300 mg QW (n=59)	**900 mg Q4W (n=65)**	**Delayed Treatment Arm (n=29)**
Primary Endpoint (Virologic Response + ALT Normalization)	56%	45%	0%
P-value	*<0.0001*	*<0.0001*	
Virologic Response (HDV RNA ≥2 log10 reduction or TND)	86%	85%	0%
HDV RNA LLOQ, <10 IU/ml	25%	26%	0%
HDV RNA TND	17%	17%	0%
ALT Normalization	63%	54%	0%

Full analysis set; participants who were randomized to brelovitug treatment and received at least one dose of brelovitug after randomization or randomized to the delayed treatment arm.
P-values compare each treatment group against delayed treatment using a stratum-adjusted Miettinen Nurminen test.

Phase 3 Summary of Safety Through Week 24

Participants who experienced, n (%)	**300 mg QW (n=59)**	**900 mg Q4W (n=65)**	**Delayed Treatment Arm (n=29)**
AEs			
Any	27 (45.8)	40 (61.5)	8 (27.6)
Related to treatment	14 (23.7)	22 (33.8)	0
Grade 3+	0	0	1 (3.4)*
Serious	0	0	1 (3.4)
AE leading to discontinuation of study drug	0	0	0
Injection site reactions	6 (10.2)	12 (18.5)	0
Flu-like symptoms	3 (5.1)	7 (10.8)	0

** Grade 4 acute myocardial infarction.*

48-Week Data from the Phase 2b Portion of AZURE-1: Deepening Viral Suppression and Increased Rates of ALT Normalization

The Phase 2b portion of AZURE-1 included the first 53 patients enrolled in the study. Following the previously reported Week 24 primary analysis, patients continued treatment in the open-label extension. Between Week 24 and Week 48, viral suppression deepened and rates of ALT normalization increased. The Week 48 results are presented below in Phase 2b Key Efficacy Endpoints (Weeks 24 and 48).

Phase 2b Key Efficacy Endpoints (Weeks 24 and 48)

Endpoint	300 mg QW [a]		900 mg Q4W	
	24 Weeks (n=20)	**48 Weeks (n=20)**	**24 Weeks (n=20)**	**48 Weeks (n=20)**
Primary Endpoint (Virologic Response + ALT Normalization)	45%	55%	35%	55%
P-value	*0.003*		*0.0024*	
Virologic Response (HDV RNA ≥2 log10 reduction or TND)	100%	95%[b]	75%[c]	95%[c]
HDV RNA LLOQ, <10 IU/ml	45%	80%	10%	40%
HDV RNA TND	35%[d]	40%	5%	25%
ALT Normalization	45%	55%	40%	55%

Full analysis set; participants receiving at least one post-baseline efficacy assessment.
[a] One participant discontinued after first dose and did not provide on-treatment results.
[b] One participant considered a virologic responder at Week 24 was lost to follow-up due to relocation and had missing Week 48 data. This participant was considered a non-responder at Week 48.
[c] One participant had Week 12 virologic response but was subsequently lost to follow-up. This participant was considered a non-responder at Week 24 and Week 48.
[d] One participant had a late Week 24 result (post-Week 24 analysis cutoff) that was TND.
P-values compare each treatment group against delayed treatment using a stratum-adjusted Cochran-Mantel-Haenszel (CMH) test.

Safety in the Phase 2b portion through Week 48 was consistent with previously reported results, with no new safety signals observed.

The full results from the Phase 3 AZURE-1 study will be presented at an upcoming medical congress. Topline results from the Phase 3 AZURE-4 study, the second of the two pivotal studies supporting the U.S. registration package, are expected in the fourth quarter of 2026. The Company expects to submit a Biologics License Application ("BLA") to the FDA in the first half of 2027, with potential commercial launch in the U.S. in the fourth quarter of 2027. Topline results from the Phase 3 AZURE-2 and AZURE-3 studies are expected in the second half of 2027.

We now estimate total annual revenue potential of brelovitug in HDV to be more than $1 billion, and we estimate the total annual revenue potential for Atebrioz tablets to reduce the volume of total new HO in adult and pediatric patients aged 12 years and older with FOP to be greater than $200 million.

Forward-Looking Statements

Certain statements contained in this report are forward-looking statements that involve a number of risks and uncertainties. Such forward-looking statements include, without limitation, statements regarding the Company's continued advancement of zilurgisertib with Incyte, expectations regarding the expected commercial availability of Atebrioz, including the expected timing, cost to patients and methods of availability, the expected timing of topline data for the AZURE studies and potential BLA submission and commercial launch, the potential benefits of brelovitug and the success or approval of any potential regulatory submission for brelovitug. The inclusion of forward-looking statements should not be regarded as a representation by the Company that any of these results will be achieved. Actual results may differ from those set forth in this report due to the risks and uncertainties associated with research and development of pharmaceutical product candidates, as well as risks and uncertainties inherent in the Company's business, including those described in the Company's other filings with the U.S. Securities and Exchange Commission. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof, and the Company undertakes no obligation to revise or update any forward-looking statements to reflect events or circumstances after the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Mirum Pharmaceuticals, Inc.

Date: September 28, 2026

By: /s/ Christopher Peetz

Christopher Peetz

Chief Executive Officer