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 28, 2021

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)**	**(I.R.S. Employer Identification No.)**

950 Tower Lane, Suite 1050 **Foster City, California** **(Address of principal executive offices)**	**94404** **(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 1.01 Entry into a Material Definitive Agreement.

On September 28, 2021, Mirum Pharmaceuticals, Inc. (the "Company") entered into Amendment No. 1 to Revenue Interest Purchase Agreement (the "Amendment") with Mulholland SA LLC ("Purchaser Agent") and the purchasers (the "Purchasers") party to the Revenue Interest Purchase Agreement, dated as of December 8, 2020 (the "RIPA"), by and among the Company, Purchaser Agent and the Purchasers. The Amendment amends the RIPA to, among other things, permit the Company to transfer its Specified Priority Review Voucher (as defined therein).

As consideration for the Purchasers agreeing to permit the transfer of the Specified Priority Review Voucher, the Company has agreed to maintain the proceeds received by the Company in respect of the transfer in a segregated account, subject to the Purchaser Agent's control, and maintain the balances therein in cash or cash equivalents (the "Segregated Account"). Further, should the Company fail to comply with the agreements governing the Segregated Account and maintenance of the balances therein, an automatic put option event shall be deemed to have occurred under the RIPA.

All other material terms, including the rights of the Purchaser Agent following the occurrence of a put option event, and covenants in the RIPA remain unchanged.

The foregoing description of the material terms of the Amendment does not purport to be complete and is qualified in its entirety by reference to the complete text of the Amendment, a copy of which is filed herewith and incorporated herein by reference.

Item 8.01 Other Events.

On September 29, 2021, the Company announced that the U.S. Food and Drug Administration (the "FDA") completed its review of the Company's new drug application ("NDA") for LIVMARLI™ (maralixibat) oral solution, a minimally absorbed ileal bile acid transporter inhibitor, for the treatment of cholestatic pruritus in patients with Alagille syndrome one year of age and older, and approved the NDA. In connection with approval of the NDA, the FDA issued the Company a rare pediatric disease priority review voucher pursuant to Section 529 of the Federal Food, Drug, and Cosmetic Act. The full text of this press release is filed as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit No.	Description
10.1	Amendment No. 1 to Revenue Interest Purchase Agreement, dated September 28, 2021
99.1	Press Release, dated September 29, 2021
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

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 29, 2021

By: /s/ Christopher Peetz
Christopher Peetz
President and Chief Executive Officer

Exhibit 10.1

Execution Version

AMENDMENT NO. 1 TO REVENUE INTEREST PURCHASE AGREEMENT

This Amendment No. 1 (this "Amendment") is entered into by and among Mirum Pharmaceuticals, Inc., a Delaware corporation (the "Company"), Mulholland SA LLC, as Purchaser Agent for the Purchasers (in such capacity, the "Purchaser Agent") and the Purchasers party to that certain Revenue Interest Purchase Agreement, dated as of December 8, 2020 (as amended, modified, restated or supplemented from time to time, the "RIPA"), effective as of September 28, 2021 (the "First Amendment Effective Date").

Capitalized terms not otherwise defined in this Amendment shall have the meanings set forth in the RIPA. The Company, Purchasers and Purchaser Agent are sometimes referred to herein individually as a "Party" and collectively as the "Parties."

WHEREAS, the Parties wish to amend the RIPA pursuant to Section 8.08 thereof as more fully set forth in this Amendment.

NOW, THEREFORE, for good and valuable consideration, the sufficiency and receipt of which are hereby acknowledged, the Parties hereto intending to be legally bound do hereby agree as follows:

1. **Amendments.** Subject to Section 2 of this Amendment, the Parties hereto agrees to the following amendments to the RIPA:

1.1 The following defined terms are added to Section 1.01 of the RIPA:

""Segregated Account" has the meaning given such term in Section 5.10(c)(i)."

""Segregated Account Control Agreement" has the meaning given such term in Section 5.10(c)(i)."

""Specified Priority Review Voucher" means any rare pediatric disease priority review voucher issued by the FDA pursuant to Section 529(a) of the FD&C Act to Mirum, as the sponsor of maralixibat in connection with the Regulatory Approval thereof."

1.2 Clause (e) of the definition of "Put Option Event" is hereby amended to read in its entirety as follows:

"the Company breaches in any respect (i) any term, covenant or agreement in any Transaction Document (other than Section 5.10(c) hereof or any term, covenant or agreement contained in the Segregated Account Control Agreement) which such breach, if capable of cure, is not cured within ten (10) Business Days after the earlier of (x) receipt of written notice of such breach from the Purchaser Agent and (y) Knowledge of the Company of such breach or (ii) Section 5.10(c) hereof or any term, covenant or agreement contained in the Segregated Account Control Agreement"

1.3 Section 2.03(c)(ii) of the RIPA is hereby amended to read in its entirety as follows:

"(ii) upon the occurrence of a Bankruptcy Event or a breach of Section 5.10(c) hereof,"

1.4 The second to last sentence of Section 5.07(a) of the RIPA is hereby amended to read in its entirety as follows:

"Notwithstanding anything to the contrary contained herein, immediately upon the occurrence of a Bankruptcy Event or a breach of Section 5.10(c) hereof, each Purchaser shall be deemed to have automatically and simultaneously elected to terminate the Purchaser Commitments and have the Company repurchase from such Purchaser the Revenue Interests for the Put/Call Price in cash and the Purchaser Commitments shall immediately terminate and the Put/Call Price shall be immediately due and payable without any further action or notice by any party."

1.5 Section 5.10(a)(x) of the RIPA is hereby amended to read in its entirety as follows:

"(x) Transfer any Collateral, other than

(A) the use of cash and cash equivalents, disposition of inventory and the disposition of obsolete, worn-out or surplus equipment, in each case in the ordinary course of business;

(B) the incurrence of Permitted Liens;

(C) the entry into Permitted Licenses;

(D) the use of cash and cash equivalents to make Permitted Investments;

(E) a Transfer to an Obligor, provided that in the case of a Transfer from an Obligor such Transfer does not impair the Liens of the Purchaser Agent in the Collateral subject to such Transfer;

(F) Transfers from any Obligor to any Subsidiary that is not a Subsidiary Guarantor; provided that (i) the sum of all such Transfers made pursuant to this clause (F) shall not exceed $1,000,000 in the aggregate during any fiscal year and (ii) such Transfers shall not include any Product Asset;

(G) Transfers among Subsidiaries that are not Subsidiary Guarantors;

(H) leases or subleases of real property in the ordinary course of business;

(I) the sale or discount without recourse of accounts receivable arising in the ordinary course of business in connection with the compromise or collection thereof;

(J) the abandonment, cancellation, non-renewal or discontinuance of use or maintenance of immaterial Intellectual Property (or rights relating thereto) that the Company reasonably determines in good faith, and, in respect of immaterial Intellectual Property related to any Included Product, after reasonable consultation with the Purchaser Agent, is desirable in the conduct of its business and not disadvantageous to the interests of the Purchasers, including that such abandonment, cancellation, non-renewal or discontinuance of use or maintenance could not reasonably be expected to have an adverse effect on the Revenue Interests (including, without limitation, timing, amount or duration thereof) or to have a Material Adverse Effect; or

(K) the sale of the Specified Priority Review Voucher so long as (i) the Company has satisfied its obligations under Section 5.10(c)(i) as of the date of such sale and (ii) any net cash proceeds of such sale received by the Company or its Subsidiaries (the "SPRV Proceeds") are wired in immediately available cash on the date of such sale into the Segregated Account and maintained therein in accordance with Section 5.10(c)(ii) and the Segregated Account Control Agreement.

1.6 A new Section 5.10(c) is hereby added to the RIPA which reads in its entirety as follows:

"(c) (i) Prior to the sale of the Specified Priority Review Voucher, the Company shall open a deposit and/or securities account located at a bank reasonably acceptable to Purchaser Agent (such accounts, collectively, the "Segregated Account"), it being agreed that Silicon Valley Bank is a bank reasonably acceptable to Purchaser Agent, which account shall be subject to a Control Agreement, in form and substance acceptable to Purchaser Agent in its sole discretion (the "Segregated Account Control Agreement") which shall provide (A) that, after delivery of a block notice, the account bank shall not permit the Company to withdraw any funds from the Segregated Account without the written consent of Purchaser Agent and (B) "read-only" at all times access to Purchaser Agent with regard to account information, including balance. For the avoidance of doubt, the Borrower shall be permitted to direct the investment of any balances maintained in the account in a manner permitted by clause (ii) below at all times prior to the delivery of a block notice; provided, further, that the Purchaser Agent shall be permitted to deliver a block notice at any time at its sole discretion.

(ii) the Company will cause the SPRV Proceeds to be wired to the Segregated Account directly, and, to the extent the Company or any Subsidiary receives such proceeds in another account, the Company shall within one Business Day cause such proceeds to be deposited in the Segregated Account. Without the prior written consent of the Purchaser Agent, the Company shall not permit the balance on deposit in the Segregated Account, at any time, to be less than the lesser of (x) $100,000,000.00 and (y) the amount of the SPRV Proceeds received by the Company and its Subsidiaries. The Company shall comply with all terms of the Segregated Account Control Agreement. Notwithstanding the foregoing or any term in this Agreement to the contrary, at all times prior to the delivery of a block notice, the Company shall be permitted to maintain the balances on deposit in the Segregated Account in the form of cash or cash equivalents (including Investments made in accordance with the Investment Policy).

2. Conditions to Effectiveness. The effectiveness of this Amendment shall be subject to the following conditions:

2.1 The Purchaser Agent shall have received this Amendment, duly executed by the Company, the Purchaser Agent and all of the Purchasers as required by Section 8.08 of the RIPA;

2.2 Each of the representations and warranties in Article III of the RIPA and Section 4.2 of this Amendment shall be true, accurate and complete in all material respects as of the date hereof; provided, however, that such materiality qualifier shall not be applicable to any representations and warranties that already are qualified or modified by materiality in the text thereof; provided further that those representations and warranties expressly referring to a specific date shall be true, accurate and complete in all material respects as of such date;

2.3 The Company has satisfied its obligations under Section 4.3 of this Amendment; and

2.4 No Put Option Event or breach or default under any of the Transaction Documents shall have occurred and be continuing, immediately prior to and after giving effect to this Amendment.

3. Release of Claims

3.1 The Company hereby absolutely and unconditionally releases and forever discharges the Purchaser Agent and each Purchaser, and any and all participants, parent corporations, subsidiary corporations, affiliated corporations, insurers, indemnitors, successors and assigns thereof, together with all of the present and former directors, officers, agents, attorneys and employees of any of the foregoing (each a "***Releasee***" and collectively, the "***Releasees***"), from any and all claims, demands or causes of action of any kind, nature or description, whether arising in law or equity or upon contract or tort or under any state of federal law or otherwise (each, a "***Claim***" and collectively, the "***Claims***"), which the Company has had, now has, or has made claim to have against such person for or by reason of any act, omission, matter, cause or thing whatsoever arising from the beginning of time to and including the date of this Amendment, whether such claims, demands and causes of action are matured or unmatured or known or unknown. The Company understands, acknowledges and agrees that the release set forth above may be pleaded as a full and complete defense to any Claim and may be used as a basis for an injunction against any action, suit or other proceeding which may be instituted, prosecuted or attempted in breach of the provisions of such release. The Company agrees that no fact, event, circumstance, evidence or transaction which could now be asserted or which may hereafter be discovered will affect in any manner the final, absolute and unconditional nature of the release set forth above.

3.2 The Company hereby absolutely, unconditionally and irrevocably covenants and agrees with and in favor of each Releasee that it will not sue (at law, in equity, in any regulatory proceeding or otherwise) any Releasee on the basis of any Claim released, remised or discharged by the Company pursuant to Section 3.1 above. If the Company violates the foregoing covenant, the Company, for itself and its successors and assigns, agrees to pay, in addition to such other damages as any Releasee may sustain as a result of such violation, all attorneys' fees, costs and expenses incurred by any Releasee as a result of such violation.

4. General.

4.1 The Company hereby represents and warrants to the Purchaser Agent and the Purchasers, as of the First Amendment Effective Date, the following:

4.1.1 The Company has all necessary power and authority to enter into, execute and deliver this Amendment and to perform all of the obligations to be performed by it under this Amendment and to consummate the transactions contemplated hereunder. This Amendment has been duly authorized, executed and delivered by the Company, and the Amendment constitutes the valid and binding obligation of the Company, enforceable against the Company in accordance with its terms, subject, as to enforcement of remedies, to bankruptcy, insolvency, reorganization, moratorium or similar laws affecting creditors' rights generally or general equitable principles;

4.1.2 The execution and delivery by the Company of the Amendment, and the performance by the Company of its obligations hereunder, does not require any notice to, action or consent by, or in respect of, or filing with, any Governmental Authority, except for any filings with the SEC; and

4.1.3 All information heretofore furnished to the Purchaser Agent or any Purchaser by or on behalf of the Company for purposes of or in connection with this Amendment or any transaction contemplated hereby, after giving effect to all supplements thereto made prior to the Second Amendment Effective Date, is or will be, true, complete and correct in every material respect; provided that projections and other forward looking information are based on reasonable estimates on the date as of which such information is stated or certified (it being understood that forecasts and projections are subject to contingencies and no assurance can be given that any forecast or projection will be realized).

4.2 The Company, hereby (i) acknowledges and agrees that all of its obligations under the RIPA and each other Transaction Document and under any other document or instrument executed and delivered or furnished in connection with such Transaction Documents are reaffirmed and remain in full force and effect on a continuous basis, including, for the avoidance of doubt, after giving effect to this Amendment, (ii) acknowledges, agrees and reaffirms that each Lien granted by it to Purchaser Agent under the Transaction Documents (including, prior to any Transfer permitted by Section 5.10(a)(x)(K), on the Specified Priority Review Voucher) for the ratable benefit of the Purchasers is and shall remain in full force and effect after giving effect to this Amendment and (iii) agrees that the Obligations secured by the Security Agreement and each other Transaction Document to which it is a party shall include all Obligations arising after giving effect to this Amendment.

4.3 The Company shall pay to the Purchaser Agent all Reimbursable Expenses (including reasonable attorneys' fees and expenses) for documentation and negotiation of this Amendment, or otherwise submitted in writing for reimbursement prior to the date of this Amendment, in each case in accordance with Section 2.02(b) of the RIPA.

4.4 (i) The execution, delivery and effectiveness of this Amendment shall not operate as a waiver of any rights, power or remedy of the Purchasers or the Purchaser Agent under the RIPA or any other documents executed in connection with the RIPA or constitute a waiver of any provision of the RIPA or any other document executed in connection therewith and (ii) this Amendment shall not by implication, course of dealing or otherwise limit, modify, amend or in any way affect any of the terms, conditions, obligations, covenants or agreements in the Transaction Documents, in each case, except to the extent limited, modified, amended or affected by this Amendment.

4.5 Except as expressly modified by this Amendment, the terms and provisions of the RIPA shall remain unchanged and in full force and effect in accordance with its terms. In the event of any inconsistencies between the provisions of this Amendment and the provisions of RIPA or any other Transaction Document, the provisions of this Amendment shall govern and prevail. For the avoidance of doubt, this Amendment is a Transaction Document.

4.6 This Amendment shall be governed by, and construed, interpreted and enforced in accordance with, the laws of the state of New York, without giving effect to the principles of conflicts of law thereof.

4.7 The provisions of Sections 8.02 (Notice), 8.07 (Entire Agreement), 8.08 (Amendments, No Waivers), 8.11 (Counterparts; Effectiveness), and 8.14(b) and (c) (Jurisdiction) of the RIPA are hereby incorporated by reference into this Amendment, *mutatis mutandis*.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, the Parties have caused this Amendment to be duly executed by their respective duly authorized officers as of the Effective Date.

Company:

MIRUM PHARMACEUTICALS, INC.

By: /s/ Ian Clements

Name: Ian Clements

Title: Chief Financial Officer

Purchaser Agent:

MULHOLLAND SA LLC

By: /s/ David Dubinsky
Name: David Dubinsky
Title: Authorized Person

Purchaser:

TPC INVESTMENTS II LP

By: /s/ David Dubinsky
Name: David Dubinsky
Title: Authorized Signatory

TPC INVESTMENTS SOLUTIONS LP

By: /s/ David Dubinsky
Name: David Dubinsky
Title: Authorized Signatory

TPC INVESTMENTS SOLUTIONS CO-INVEST LP

By: /s/ David Dubinsky
Name: David Dubinsky
Title: Authorized Signatory

Exhibit 99.1

U.S. FDA Approves LIVMARLI (maralixibat) as the First and Only Approved Medication for the Treatment of Cholestatic Pruritus in Patients with Alagille Syndrome One Year of Age and Older

- Mirum to host an investor call on September 29, 2021 at 4:30 p.m. ET

FOSTER CITY, Calif. – September 29, 2021 - Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM), a leader in rare liver disease, today announced that the U.S. Food and Drug Administration (FDA) has approved LIVMARLI™ (maralixibat) oral solution for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS) one year of age and older. LIVMARLI, a minimally absorbed ileal bile acid transporter (IBAT) inhibitor, is the first and only FDA-approved medication in this rare liver disease which affects 2,000 to 2,500 children in the United States. LIVMARLI is now available for prescribing. In conjunction with the approval, Mirum received a rare pediatric disease priority review voucher.

“Children with Alagille syndrome suffer from cholestatic pruritus, which is serious, unremitting, and debilitating. Their sleep is disrupted, and they endure bleeding and scarring of the skin due to unrelenting scratching,” said Binita M. Kamath, MBBChir, Pediatric Hepatologist, The Hospital for Sick Children (SickKids), Toronto, Ontario, Canada. “There have been no approved treatments to date for cholestatic pruritus in Alagille syndrome, and many children ultimately require major surgical interventions such as liver transplantation for refractory pruritus. The approval of LIVMARLI signifies a meaningful shift in the treatment paradigm for Alagille syndrome and provides hope for the many families who have lived with persistent itch for far too long.”

ALGS is a rare genetic disorder caused by abnormalities in bile ducts that can lead to progressive liver disease. Malformed or reduced bile ducts cause cholestasis, the accumulation of bile acids in the liver, which leads to inflammation and liver injury, and prevents the liver from working properly. Cholestasis in ALGS is associated with pruritus which is among the most common indications for liver transplant in ALGS.

The approval of LIVMARLI is based on the pivotal ICONIC study as well as five years of data from supportive studies resulting in a robust body of evidence in 86 patients with ALGS. Data from ICONIC demonstrated statistically significant reductions in pruritus, one of the most common and arduous symptoms associated with the disease, which was maintained through four years.

“Today is a great day for the Alagille syndrome community with the approval of a much-needed new treatment option to address one of the most debilitating effects of this disease,” said Chris Peetz, president and chief executive officer of Mirum. “We are grateful to the patients, families, and healthcare professionals who advanced the research and participated in the LIVMARLI clinical studies. Today is also a landmark day for Mirum as we take steps forward in developing potentially life-changing medicines for rare liver disease.”

“We have had the pleasure of being part of and closely following the clinical progress of LIVMARLI in many ways. Since the first study’s initiation more than a decade ago, we have dreamed of today, seeing LIVMARLI receive FDA approval, marking an incredibly meaningful milestone for the ALGS community,” said Roberta Smith, president, Alagille Syndrome Alliance and an ALGS mom. “Until now, patients have had limited-to-no treatment options to address the severe and unrelenting itch that significantly impacts both patients and their families. Additionally, because pruritus associated with ALGS greatly impacts caregivers, having a strong support program like Mirum Access Plus to reduce the strain on families is so important. The ALGS community has been waiting for a long time for a treatment and we’re so pleased that LIVMARLI is now available in the United States.”

Mirum Access Plus (MAP)

LIVMARLI will be accessible to patients with a prescription through Mirum Access Plus (MAP), the company’s patient support services program and single-source specialty pharmacy. The MAP program has fully dedicated and experienced coordinators who will work with healthcare providers and families to provide insurance coverage and access support, as well as help with financial support options for LIVMARLI. A dedicated Navigator team will also provide health education and will connect families to resources and tools to support their disease. MAP staff are available Monday through Friday, 8:00 a.m. to 8:00 p.m. ET by calling 855-MRM-4YOU (1-855-676-4968) or more information, visit www.livmarli.com.

Patients Enrolled in the U.S. Expanded Access Program

For patients who are currently enrolled in the U.S. Expanded Access Program (EAP), MAP coordinators will assist patients who wish to continue on LIVMARLI with the conversion to prescription LIVMARLI. The EAP will remain open for eligible patients with ALGS in Australia, Canada, the UK and several countries in Europe until LIVMARLI is approved in the respective country. For more information about the maralixibat EAP, visit algseap.com.

Investor Conference Call

Mirum will host a conference call to discuss LIVMARLI’s approval in further detail today, September 29, at 4:30 p.m. ET. The live webcast and archived event will be available within the Events section on Mirum’s website. To participate in the live call, dial (844) 200-6205 (toll free) or (646) 904-5544 (international) and use the passcode 986568.

About LIVMARLI™ (maralixibat) oral solution

LIVMARLI™ (maralixibat) oral solution is an orally administered, once-daily, ileal bile acid transporter (IBAT) inhibitor approved by the U.S. Food and Drug Administration for the treatment of cholestatic pruritus in patients with Alagille syndrome one year of age and older. For more information, please visit LIVMARLI.com.

LIVMARLI is the only FDA-approved medication to treat cholestatic pruritus associated with Alagille syndrome.

LIVMARLI is currently being evaluated in late-stage clinical studies in other rare cholestatic liver diseases including progressive familial intrahepatic cholestasis and biliary atresia, of which both have received
Breakthrough Therapy designation and Orphan Drug designation. To learn more about ongoing clinical trials with LIVMARLI, please visit Mirum’s clinical trials section on the company’s website.

About Alagille syndrome

Alagille syndrome (ALGS) is a rare genetic disorder in which bile ducts are abnormally narrow, malformed and reduced in number, which leads to bile accumulation in the liver and ultimately progressive liver disease. The estimated incidence of ALGS is one in every 30,000 people.[1] In patients with ALGS, multiple organ systems may be affected by the mutation, including the liver, heart, kidneys and central nervous system.[2] The accumulation of bile acids prevents the liver from working properly to eliminate waste from the bloodstream and, according to recent reports, 60% to 75% of patients with ALGS have a liver transplant before reaching adulthood.[3] Signs and symptoms arising from liver damage in ALGS may include jaundice (yellowing of the skin), xanthomas (disfiguring cholesterol deposits under the skin), and pruritus (itch)[2]. The pruritus experienced by patients with ALGS is among the most severe in any chronic liver disease and is present in most affected children by the third year of life.[4]

IMPORTANT SAFETY INFORMATION

LIVMARLI can cause serious side effects, including:

Changes in liver tests. Changes in certain liver tests are common in patients with Alagille syndrome and can worsen during treatment with LIVMARLI. These changes may be a sign of liver injury and can be serious. Your healthcare provider should do blood tests before starting and during treatment to check your liver function. Tell your healthcare provider right away if you get any signs or symptoms of liver problems, including nausea or vomiting, skin or the white part of the eye turns yellow, dark or brown urine, pain on the right side of the stomach (abdomen) or loss of appetite.

Stomach and intestinal (gastrointestinal) problems. LIVMARLI can cause stomach and intestinal problems, including diarrhea, stomach pain, and vomiting during treatment. Tell your healthcare provider right away if you have any of these symptoms more often or more severely than normal for you.

A condition called **Fat Soluble Vitamin (FSV) Deficiency** caused by low levels of certain vitamins (vitamin A, D, E, and K) stored in body fat. FSV deficiency is common in patients with Alagille syndrome but may worsen during treatment. Your healthcare provider should do blood tests before starting and during treatment.

Other common side effects reported during treatment were bone fractures and gastrointestinal bleeding.

Prescribing information

About Mirum Pharmaceuticals, Inc.

Mirum Pharmaceuticals, Inc. is a biopharmaceutical company dedicated to transforming the treatment of rare liver diseases. Mirum's approved medication is LIVMARLI™ (maralixibat) oral solution which is approved in the U.S. for the treatment of cholestatic pruritus in patients with Alagille syndrome one year of age and older.

Mirum's late-stage pipeline includes two investigational treatments for debilitating liver diseases affecting children and adults. Maralixibat (LIVMARLI), an oral ileal bile acid transporter (IBAT) inhibitor, is currently being evaluated in clinical trials for pediatric liver diseases and includes the MARCH Phase 3 study for progressive familial intrahepatic cholestasis (PFIC) and the EMBARK Phase 2b study for patients with biliary atresia. In addition, Mirum has an expanded access program open in Canada, Australia, the UK and several countries in Europe for eligible patients with Alagille syndrome.

Mirum has submitted a Marketing Authorization Application to the European Medicines Agency for maralixibat for the treatment of cholestatic liver disease in patients with Alagille syndrome.

Mirum's second investigational treatment, volixibat, also an oral IBAT inhibitor, is being evaluated in two registrational studies including the OHANA Phase 2b study for pregnant women with intrahepatic cholestasis of pregnancy and the VISTAS Phase 2b study for adults with primary sclerosing cholangitis. Mirum is planning to launch a Phase 2b study in primary biliary cholangitis later this year.

To augment its pipeline in cholestatic liver disease, Mirum has acquired the exclusive option to develop and commercialize gene therapy programs VTX-803 and VTX-802 for PFIC3 and PFIC2, respectively, from Vivet Therapeutics SAS, following preclinical evaluation and investigational new drug-enabling studies.

Follow Mirum on Twitter, Facebook, LinkedIn and Instagram.

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 commercialization plans and expectations for commercializing LIVMARLI in the United States, estimates of the number of patients impacted by ALGS and who are appropriate for treatment with LIVMARLI, the potential benefits or competitive position of LIVMARLI, the timing of ongoing and planned clinical trials and the regulatory approval process of maralixibat in other indications and jurisdictions and of volixibat. 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 "will," "could," "would," "potential" 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 the COVID-19 pandemic, and the other risks described in Mirum's filings with the Securities and Exchange Commission. 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.

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[1]Danks, et al. Archives of Disease in Childhood 1977
[2]Johns Hopkins Medicine. hopkinsmedicine.org/health/conditions-and-diseases/Alagille-syndrome
[3]Vandriel, et al. GALA EASL 2020; Kamath, et al. Hepatology Communications 2020
[4]Elisofon, et al. Journal of Pediatric Gastroenterology and Nutrition 2010