Mirum Pharmaceuticals Secures $120 Million in Series A Financing to Develop Phase 3-Ready Maralixibat for Rare Liver Diseases

Maralixibat Phase 2b study interim analysis suggests proof of concept in Alagille syndromeCombined leadership from former Lumena and Tobira teams to pursue registration in Alagille syndrome, progressive familial intrahepatic cholestasis and other liver diseases

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Mirum Pharmaceuticals Inc. →
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SAN DIEGO, Nov. 7, 2018 /PRNewswire/ -- Mirum Pharmaceuticals today announced that it has secured $120 million in Series A financing to support development of its lead drug candidate maralixibat for rare cholestatic liver diseases. The financing was led by New Enterprise Associates (NEA) with participation from Deerfield Management, Frazier Healthcare Partners, Novo Holdings A/S, Pappas Capital, RiverVest Venture Partners and Rock Springs Capital. The following have been appointed to Mirum's board of directors in conjunction with the financing: Ed Mathers, partner NEA; Patrick Heron, managing general partner, Frazier Healthcare Partners; Jonathan Leff, partner, Deerfield; Tiba Aynechi, Ph.D., partner, Novo Ventures; and Niall O'Donnell, Ph.D., managing director, RiverVest.

Mirum also announced it has entered into an agreement with Shire for the exclusive global rights to develop and market maralixibat, an oral inhibitor of the apical sodium dependent bile acid transporter (ASBT). Shire will receive an upfront payment, an equity position in Mirum and potential future milestone payments and royalties. Maralixibat is being developed for Alagille syndrome (ALGS) and progressive familial intrahepatic cholestasis (PFIC).

Maralixibat is under investigation to evaluate its ability to prevent accumulation of excess bile acids and control extreme itching associated with cholestatic liver diseases such as ALGS and PFIC. Maralixibat could be a first-in-class oral drug for these conditions, pending regulatory authority approval. While Mirum plans to initially focus its efforts in these two pediatric indications, there is also potential to develop it for additional pediatric and adult cholestatic liver disease indications. Maralixibat has been administered to more than 230 cholestatic patients to date, providing an extensive safety data set.

"The interim data we are announcing today from the Phase 2b ICONIC study in ALGS conducted by Shire underscores my continued confidence in maralixibat and its potential to help patients with these severely debilitating liver diseases," said Mike Grey, chairman and CEO of Mirum. "The study leveraged an improved trial design and in its interim analysis, patients taking maralixibat had reductions in bile acids and pruritus compared to placebo. Additionally, in a single-arm, open-label Phase 2 study, a subset of patients with PFIC2 responded to maralixibat, with a sustained (>2 years for some) reduction or normalization of serum bile acids and reduction of pruritus. These results led to the FDA's Breakthrough Therapy designation for patients with PFIC2. These clinical studies demonstrate the potential of maralixibat to significantly impact patients' lives."

Mirum is announcing the successful outcome of the Phase 2b ICONIC study in subjects with ALGS based on the 48-week interim analysis, and plans to present the data at a liver diseases meeting next year.

As part of its strategic transaction with Shire, Mirum also acquired exclusive global rights to develop and market volixibat, also an ASBT inhibitor.

Mr. Grey has assembled a world-class leadership team at Mirum, bringing expertise in drug development in the liver disease space from former companies Lumena and Tobira.

- Chris Peetz is appointed as president of Mirum and a member of the company's board of directors
- Pamela Vig, Ph.D., joins as chief scientific officer
- Lara Longpre serves as the company's chief development officer
- Shelly Xiong, Ph.D., is appointed senior vice president of regulatory

Also supporting Mirum's launch are co-founders and advisors from the former Lumena team: Alex Dorenbaum, M.D., Susan Dubé, Ciara Kennedy, Ph.D., and Niall O'Donnell, Ph.D.

"The substantial investment we have made in Mirum and the outstanding syndicate of savvy biotech investors underscores the value we ascribe to maralixibat, as well as our confidence in the company's management team to execute on an aggressive development plan," said Ed Mathers, partner, NEA. "Mike and his team are seasoned biotech veterans with a deep knowledge of the drug, the disease biology and the patient populations they hope to help."

In an effort to bring maralixibat to patients as quickly as possible, Mirum plans to initiate Phase 3 confirmatory studies in patients with ALGS and PFIC in 2019.

About ALGS

Alagille syndrome is a rare genetic disorder that can affect the liver, heart, skeleton, eyes and kidneys. Symptoms and severity of disease can vary greatly from one person to another; some individuals may have mild forms of the disorder, while others may have more serious forms. Common symptoms, which often develop during the first three months of life, include blockage of the flow of a digestive fluid called bile from the liver (cholestasis), yellowing of the skin and mucous membranes (jaundice), poor weight gain and growth, severe itching (pruritus) and pale, loose stools.

About PFIC

Progressive familial intrahepatic cholestasis (PFIC) causes progressive liver disease, which typically leads to liver failure. In people with PFIC, liver cells are less able to secrete bile. The buildup of bile in liver cells causes liver disease in affected individuals. Signs and symptoms of PFIC typically begin in infancy. Patients experience severe itching, jaundice, failure to gain weight and grow at the expected rate (failure to thrive), high blood pressure in the vein that supplies blood to the liver (portal hypertension), and an enlarged liver and spleen (hepatosplenomegaly).

About Maralixibat

Maralixibat is an orally administered investigational drug being evaluated in several rare cholestatic liver diseases for both pediatric and adult populations. Preclinical models suggest maralixibat inhibits the apical sodium dependent bile acid transporter (ASBT), thereby preventing bile acids from accumulating in the liver. More than 230 patients have received

maralixibat as an investigational treatment for Alagille syndrome (ALGS) and progressive familial intrahepatic cholestasis (PFIC). In an interim analysis, ALGS patients taking maralixibat had reductions in bile acids and pruritus compared to placebo. In a Phase 2b PFIC study, a subset of patients responded to maralixibat, which led to maralixibat's Breakthrough Therapy designation from the U.S. Food and Drug Administration. Maralixibat was generally well tolerated throughout the study. The most frequent adverse events were diarrhea, abdominal pain and vomiting.

About Volixibat

Volixibat is an investigational oral inhibitor of ASBT, a protein that is primarily responsible for recycling bile acids from the intestine to the liver. Volixibat was under investigation for the treatment of adults with nonalcoholic steatohepatitis (NASH), a serious, chronic liver disease for which no approved drugs currently exist.

About Mirum Pharmaceuticals

Mirum Pharmaceuticals Inc. is a clinical-stage therapeutics company developing a novel approach for treating cholestatic liver diseases, with an immediate focus on rare pediatric conditions. The company's lead product candidate, maralixibat, is a Phase 3-ready investigational oral drug with an established safety profile and efficacy data in several indications, including Alagille syndrome (ALGS) and progressive familial intrahepatic cholestasis (PFIC). Backed by investors including NEA, Deerfield Management, Frazier Healthcare Partners, Novo Holdings A/S, Pappas Capital, RiverVest Venture Partners and Rock Springs Capital, Mirum is dedicated to bringing innovation to patients as quickly and efficiently as possible. For more information, visit MirumPharma.com.

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