



Mirum Pharmaceuticals Honors Alagille Syndrome Awareness Day with Launch of Disease Awareness Campaign

January 24, 2021

- *The national campaign seeks to emphasize the unbearable burden ALGS has on patients and their families.*

- *Mirum underscores its commitment to continued understanding of the rare liver disease through research and support of ALGS community.*

FOSTER CITY, Calif.--(BUSINESS WIRE)--Jan. 24, 2021-- Mirum Pharmaceuticals, Inc. (Nasdaq: MIRM) today announced the launch of a disease awareness campaign for Alagille syndrome (ALGS), a rare cholestatic liver disease affecting one in every 30,000 births worldwide. The campaign seeks to broaden awareness and education of ALGS so that healthcare providers can more readily identify symptoms and diagnose patients with the goal to improve time to diagnosis and treatment.

Children with ALGS live with severe and unrelenting symptoms that can impact their overall quality of life. Identifying these symptoms early on can lead to a diagnosis and an effective treatment plan.

"Many patients live for months or years before being diagnosed and some patients are misdiagnosed, leading to unnecessary and invasive surgeries that do not address the underlying cause of their liver disease," said Chris Peetz, president and chief executive officer at Mirum. "Our objective is to provide a platform that helps healthcare providers understand the specific signs and symptoms of ALGS so they can be better equipped to identify and diagnose this rare disease and help their patients get the treatment they need."

"This disease awareness campaign has the potential to help healthcare providers recognize Alagille syndrome in their patients in order to properly address this rare disease," said Roberta Smith, president of the Alagille syndrome Alliance and parent of a child with ALGS. "The impacts of Alagille syndrome on families are many. We have only begun to scratch the surface in understanding disease burden and specifically, how different regions in the world are affected differently. As we celebrate International Alagille Syndrome Awareness Day, we will focus on sharing Alagille syndrome family stories that emphasize the critical need for continued research and treatment options for patients worldwide. The patient voice is the Alagille syndrome community's greatest vehicle for change."

Mirum's goal is to advance the understanding and diagnosis of ALGS so that patients and their families will get the medical care and support they need to improve their quality of life. The company and its founders have been dedicated to understanding ALGS through the support of resources like the Global Alagille Alliance (GALA) for continued research, as well as genetic testing platforms that help effectively identify ALGS in patients. These resources assist in advancing research and the healthcare community's understanding of this devastating rare disease.

Visit the Unbearable ALGS [website](#) to learn more about Alagille syndrome and the significant impact it has on patients and their families.

Alagille syndrome Awareness Day – January 24, 2021

Mirum is also proud to champion the Alagille syndrome Alliance and their ongoing work to support ALGS families through education, outreach, and community. This year, in celebration of International ALGS Awareness Day, they are hosting a 24-hour [Aware-a-Thon](#) that will include family stories, messages, expert talks, and educational resources aimed at increasing the public's awareness of ALGS, a crucial element in their mission. To learn more about how to get involved and support the ALGS Alliance and the ALGS community, visit their Awareness Day [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

About Mirum

Mirum Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company focused on the development and commercialization of a late-stage pipeline of novel therapies for debilitating liver diseases. Mirum's lead product candidate, maralixibat, is an investigational oral drug in development for Alagille syndrome (ALGS), progressive familial intrahepatic cholestasis (PFIC), and biliary atresia. Mirum has initiated a rolling NDA submission for maralixibat in the treatment of cholestatic pruritus in patients with ALGS and expects to complete the submission in the first quarter of 2021. Additionally, Mirum's marketing authorization application for the treatment of pediatric patients with PFIC2 has been accepted for review (validated) by the European Medicines Agency.

Mirum is also developing volixibat, also an oral ASBT-inhibitor, in primary sclerosing cholangitis, intrahepatic cholestasis of pregnancy, and primary biliary cholangitis. For more information, visit [MirumPharma.com](#).

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 regulatory approval path for maralixibat in the United States and the European Union, and Mirum’s ALGS disease awareness campaign. 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 “plans,” “goal,” “will,” “may,” “expects,” “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.

References

¹Danks, et al. Archives of Disease in Childhood 1977

²Johns Hopkins Medicine. hopkinsmedicine.org/health/conditions-and-diseases/Alagille-syndrome

³Vandriel, et al. GALA EASL 2020; Kamath, et al. Hepatology Communications 2020

⁴Elisofon, et al. Journal of Pediatric Gastroenterology and Nutrition 2010

View source version on [businesswire.com](https://www.businesswire.com/news/home/20210124005076/en/): <https://www.businesswire.com/news/home/20210124005076/en/>

Mirum Contacts:

Media

Erin Murphy

media@mirumpharma.com

Investors

Ian Clements, Ph.D.

investors@mirumpharma.com

Source: Mirum Pharmaceuticals, Inc.