



## Mirum Pharmaceuticals to Present Phase 2 Clinical Results of Maralixibat in Alagille Syndrome and Progressive Familial Intrahepatic Cholestasis at the International Liver Congress

March 27, 2019

*Maralixibat shows reductions in pruritus and serum bile acids in 48 week analysis of ICONIC Phase 2b study in Alagille syndrome patients*

*Long-term analysis of INDIGO study of maralixibat shows sustained response and growth improvements in progressive familial intrahepatic cholestasis type two (PFIC2)*

FOSTER CITY, Calif., March 27, 2019 /PRNewswire/ -- Mirum Pharmaceuticals announced today that data from three of its programs will be presented at the International Liver Congress™ 2019, occurring in Vienna, Austria, from April 10-14, 2019, at the Reed Messe Wien Exhibition & Congress Center.

### Abstracts of Interest:

**Title:** Phase 2 open-label study with a placebo-controlled drug withdrawal period of the apical sodium-dependent bile acid transporter inhibitor maralixibat in children with Alagille Syndrome: 48-week interim efficacy analysis.

**Date and Time:** April 13, 2019; 8:15 a.m. – 8:30 a.m. CET

**Session:** Clinical developments in rare liver disease

**Abstract Number:** PS-193 – Oral presentation

**Title:** Growth analysis in children with progressive familial intrahepatic cholestasis treated with the apical sodium-dependent bile acid transporter inhibitor, maralixibat.

**Date and Time:** April 13, 2019; 5:45 p.m. – 6:00 p.m. CET

**Session:** Late Breaker

**Abstract Number:** LB-08 – Oral presentation

**Title:** Safety, tolerability and efficacy of volixibat, an apical sodium-dependent bile acid transporter inhibitor, in adults with non-alcoholic steatohepatitis (NASH): 24-week interim analysis results from a Phase 2 study.

**Abstract Number:** LBP-24 – Poster presentation

### About Maralixibat

Maralixibat is an orally administered investigational drug being evaluated in several rare cholestatic liver diseases for both pediatric and adult populations. Maralixibat inhibits the apical sodium dependent bile acid transporter (ASBT), thereby preventing bile acids from accumulating in the liver. Maralixibat is being developed as an investigational treatment for rare pediatric liver disorders such as Alagille syndrome (ALGS) and progressive familial intrahepatic cholestasis (PFIC). In the ICONIC study of maralixibat in ALGS, patients taking maralixibat had reductions in pruritus and serum bile acids compared to placebo. Results from the Phase 2 INDIGO study supported maralixibat's Breakthrough Therapy designation for PFIC from the U.S. Food and Drug Administration. Maralixibat has been generally well tolerated throughout clinical studies. The most frequent adverse events were diarrhea, abdominal pain and vomiting.

### About Volixibat

Volixibat is an orally administered investigational drug. Volixibat inhibits the apical sodium dependent bile acid transporter (ASBT), thereby preventing bile acids from accumulating in the liver. Development of volixibat in NASH was terminated by Shire prior to the drug's in-licensing by Mirum. Mirum is exploring adult cholestasis indications where volixibat may have potential.

### About Mirum Pharmaceuticals

Mirum Pharmaceuticals Inc. is a clinical-stage therapeutics company developing therapies for orphan 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 being evaluated in 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](http://MirumPharma.com).

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