# Corporate Presentation

February 2021

# Forward-Looking Statements

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# Leading the Way in Rare Liver Disease

## Bringing Life-Changing Medicines to Patients in Need

Experienced team, multiple commercializations

NDA submitted for ALGS

MAA validated for PFIC2

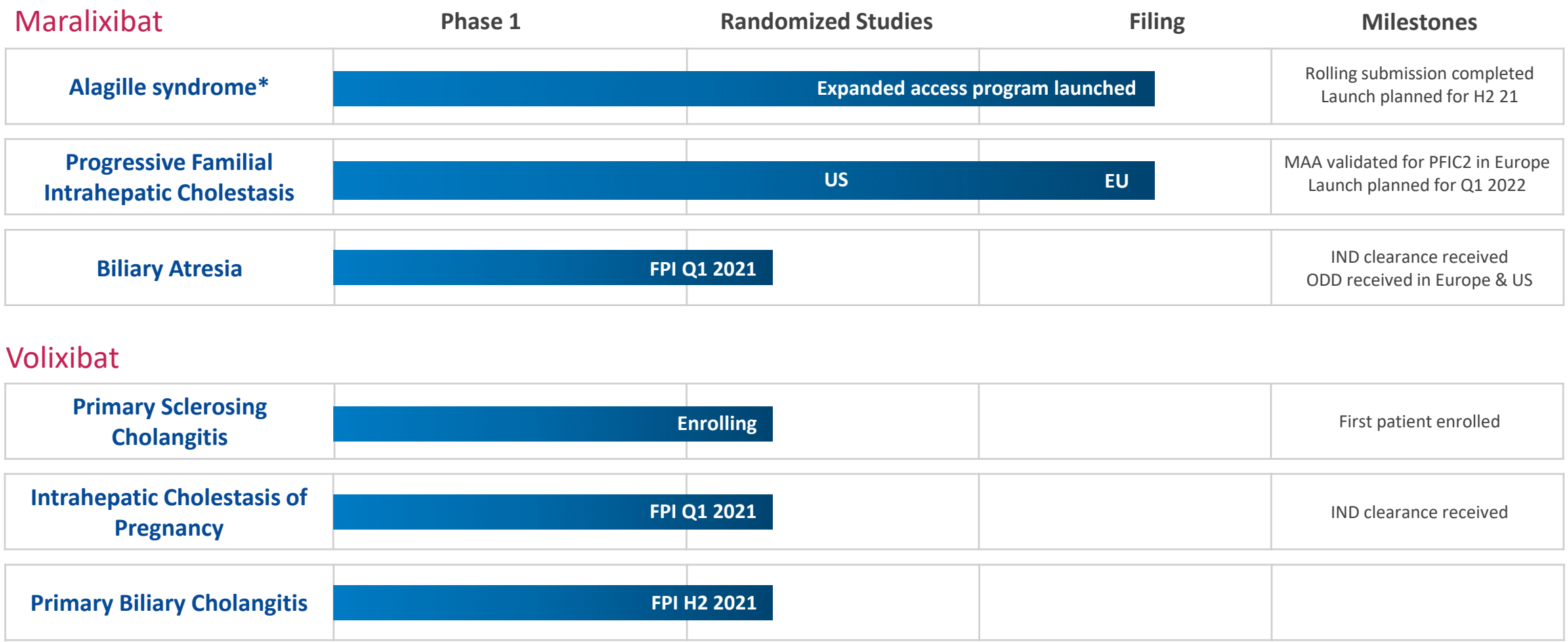
Pipeline of indications directly targeting cholestasis: ICP, PSC, PBC, BA

Near-term launch potential, multiple indications, total market potential $1B+

Abbreviations: BTD – Breakthrough Therapy Designation (FDA); RPDD – Rare Pediatric Disease Designation (FDA); ALGS – Alagille syndrome; PFIC – Progressive Familial Intrahepatic Cholestasis; BSEP – Bile Salt Export Pump; nt-PFIC2 – non-truncating PFIC2; EMA – European Medicines Agency; MAA – Marketing Authorization Application; Note that Marketing Application for PFIC2 is under review by EMA and rolling NDA submission for cholestatic pruritus associated with ALGS complete with FDA

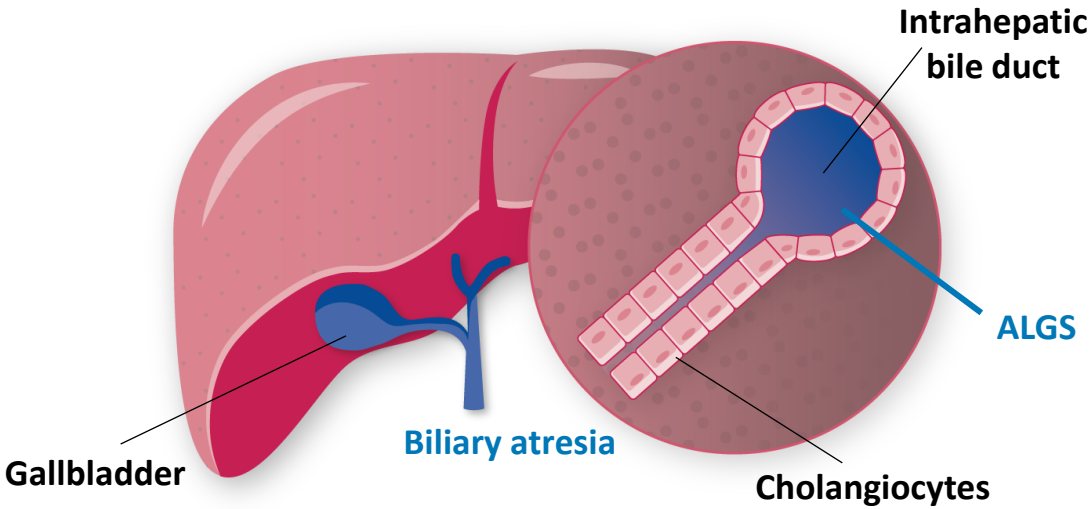
# Pipeline of Growth Opportunities

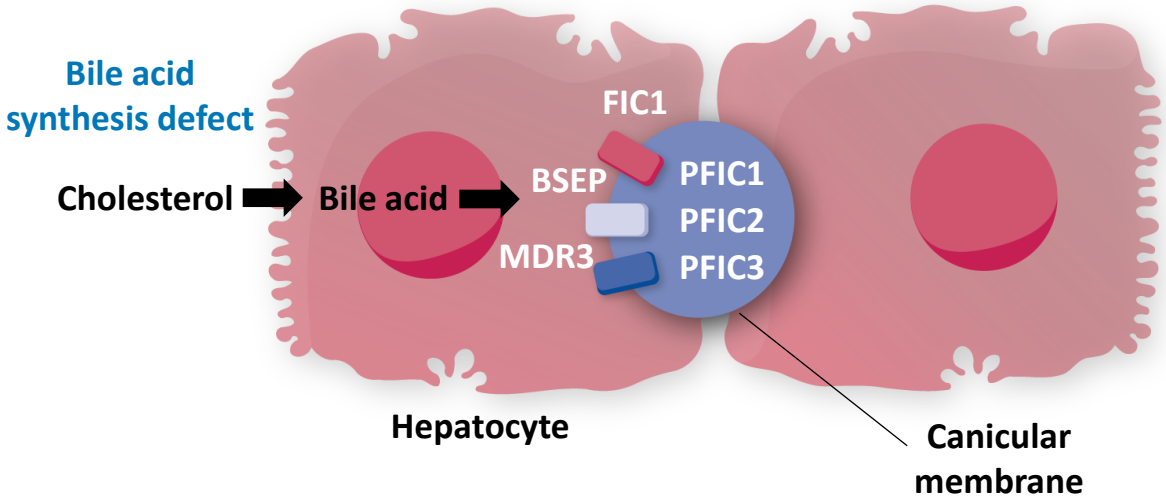
## Planning for ALGS Launch in U.S., PFIC2 Launch in Europe




Proposed indication: Cholestatic pruritus associated with ALGS

# Understanding Cholestasis

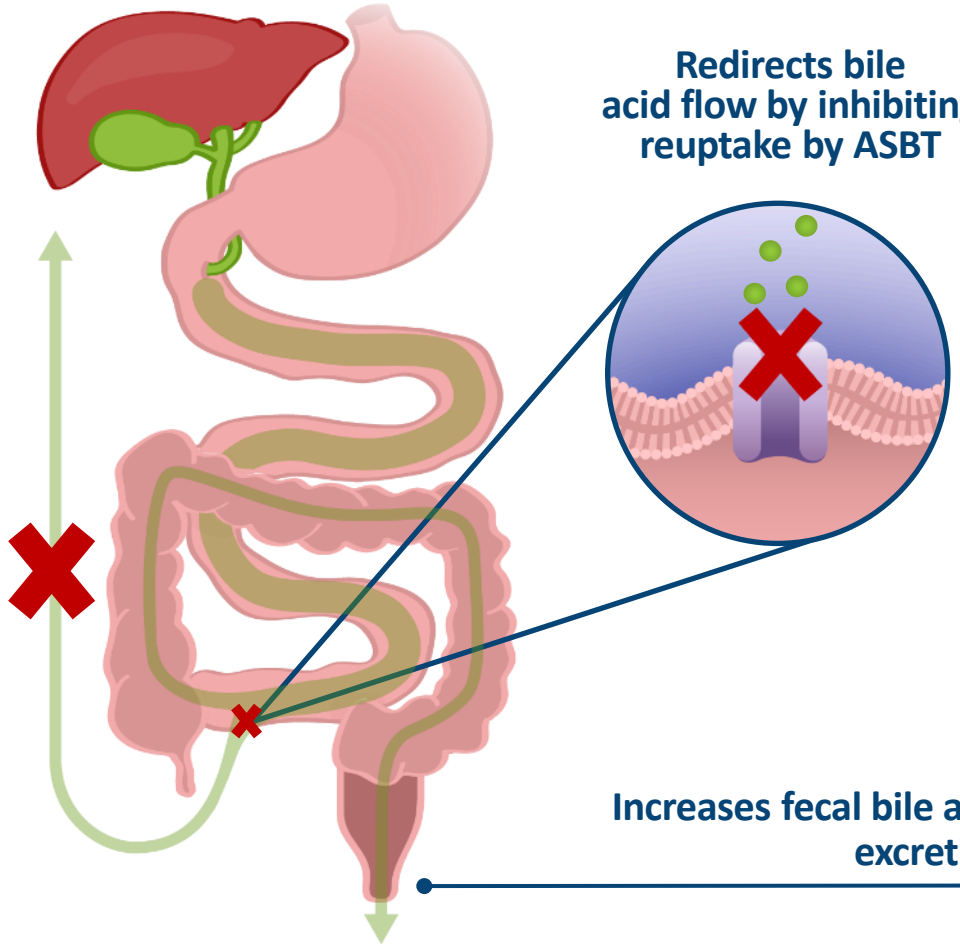



**Cholestasis = disruption of bile flow leading to clinical symptoms and serum laboratory abnormalities**

ALGS, alagille syndrome; BSEP, bile salt export pump; FIC1, familial intrahepatic cholestasis protein; MDR3, multidrug resistance protein; PFIC, progressive familial intrahepatic cholestasis.

# ASBT Inhibitors Interrupt Bile Acid Recirculation




## Clinical effects seen in cholestasis

**ASBTi clinical studies show:**

- ✓ **Reductions in pruritus**
- ✓ **Reduction in sBA**
- ✓ **Impact on markers of bile acid synthesis: cholesterol, 7αC4, FGF19**

Abbreviations: sBA: serum bile acid; ASBT: apical sodium-dependent bile acid transporter

# Maralixibat for ALGS

Significant effects on pruritus, xanthomas and growth

# ALGS: A Debilitating Disease with No Approved Treatment Option

## Alagille Syndrome

- Genetic disease leading to cholestasis and multi-system effects
- Severe pruritus, no approved therapies
- 24% transplant-free survival at 18.5 years
- Majority of transplants driven by symptoms (pruritus, xanthomas)
- Birth incidence approximately 1:30,000

## Signs of cholestasis include

Severe itch, AKA pruritus

Yellow skin or eyes, AKA jaundice

Stunted growth

Hard, yellow skin bumps, AKA xanthomas

## Organs effected by ALGS

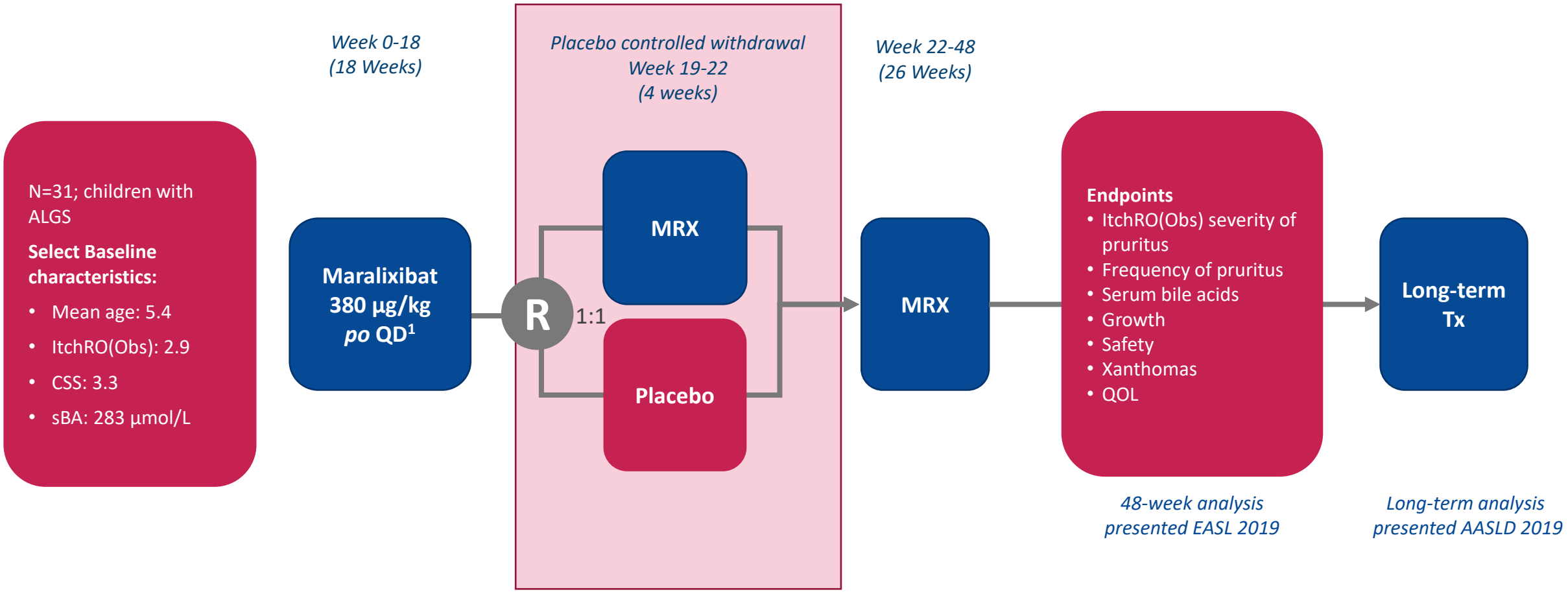
Liver

Heart

Kidneys

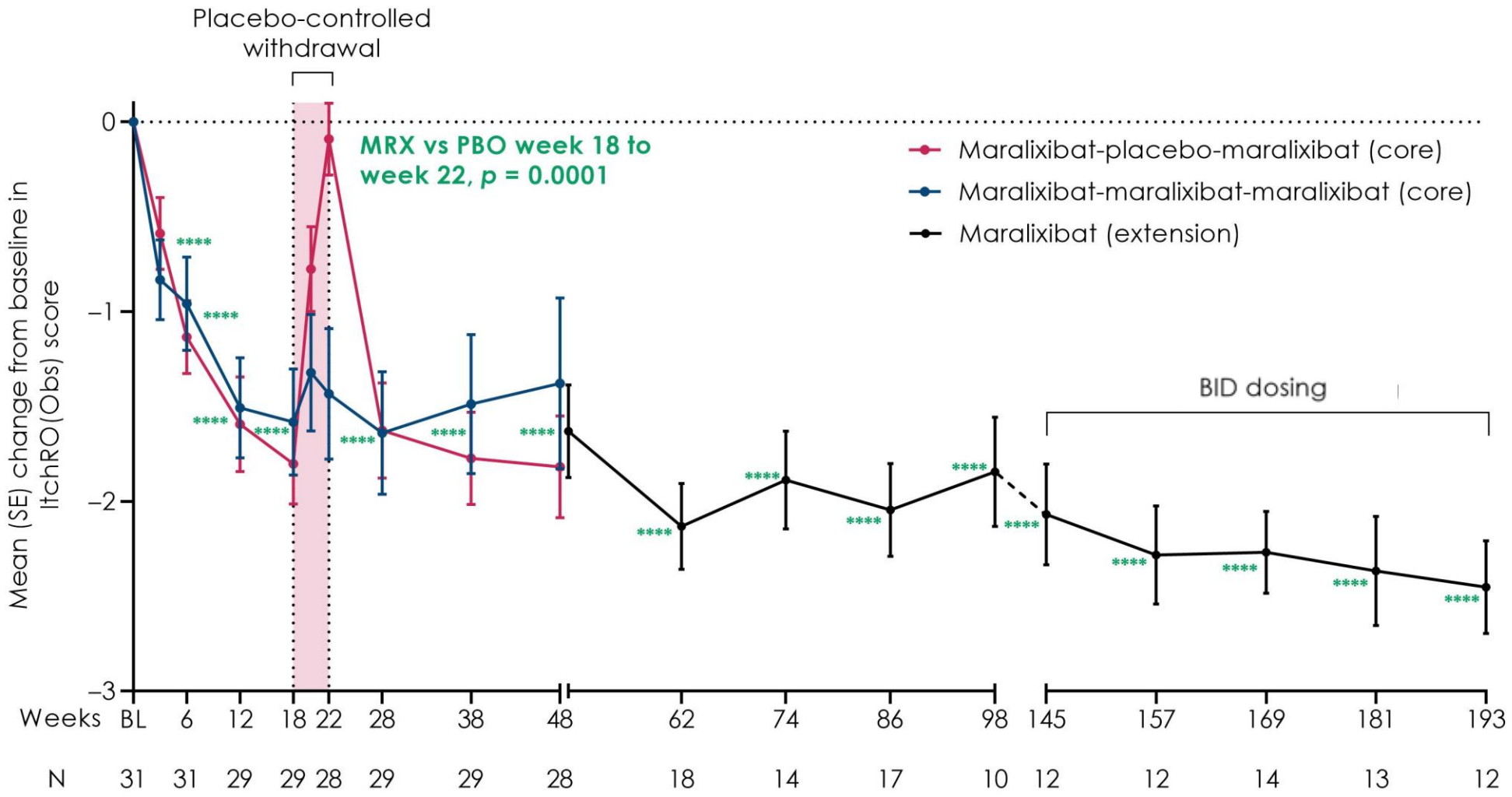
Central Nervous System

# ICONIC: Phase 2b Placebo-controlled Drug-withdrawal Clinical Trial




[1]Patients received doses of 400 µg /kg/day of maralixibat chloride (equivalent to 380 µg /kg/day of maralixibat)
MRX: Maralixibat; BID: twice daily; QD: once daily; R: randomization.

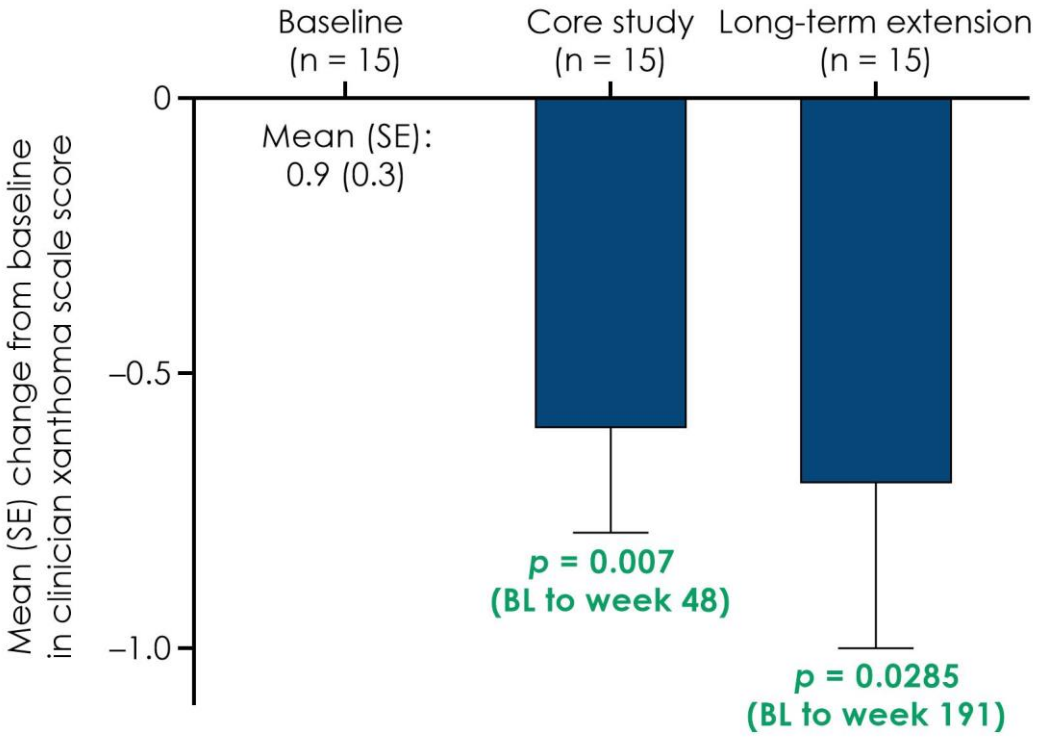
# Significant and Sustained Improvements in Itch




Change from baseline, **** $p \leq 0.0001$ (overall population)

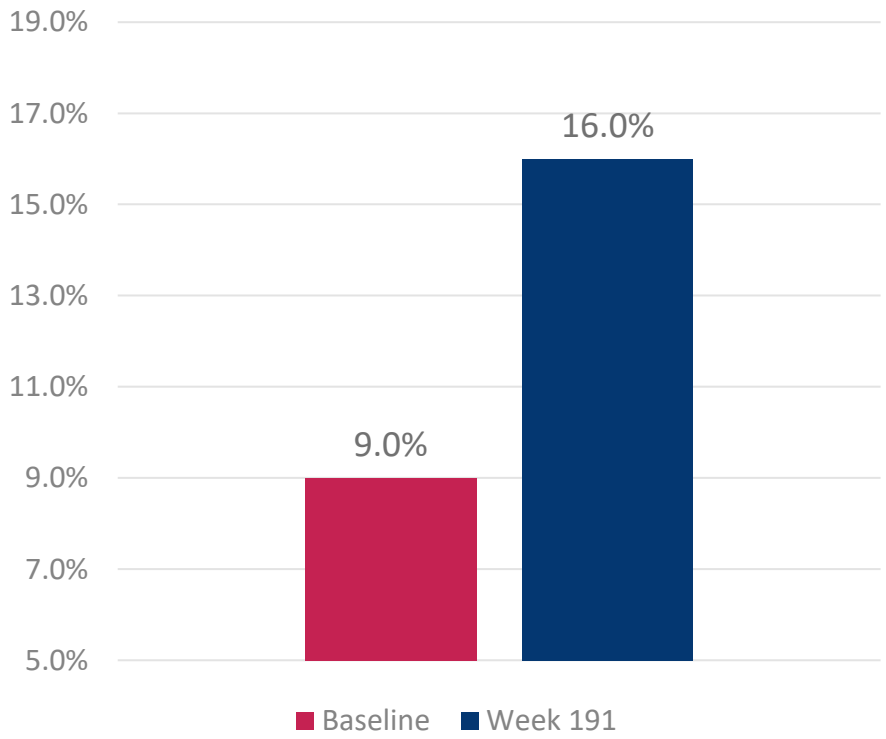
# ICONIC: Long-Term Xanthoma & Growth Changes

## Change from BL in clinician xanthoma scale score (0–4)




## Height Percentile

(n=15)




Gonzales et al., AASLD 2019

# Maralixibat Well Tolerated; Comparable GI Events to Placebo

## Safety summary of ICONIC and placebo-controlled summary of ITCH & IMAGO studies

| | ICONIC | | | | | ITCH & IMAGO | |
|---|---|---|---|---|---|---|---|
| | | Week 19-22 | | | | Baseline-week 13 | |
| Number of participants, n (%) | Week 0–18 (N = 31) | Maralixibat (n = 13) | Placebo (n = 16) | Week 23–48 (N = 29) | Week 49+ (N = 29) | Maralixibat (n = 39) | Placebo (n = 18) |
| **Any TEAE, n (%)** | 30 (97) | 7 (54) | 12 (75) | 25 (86) | 23 (79) | 35 (90) | 16 (89) |
| **Grade 3 or 4 TEAE** | 6 (19) | 0 | 1 (6) | 2 (7) | 6 (21) | 2 (5) | 2 (11) |
| **Serious TEAE (all unrelated to maralixibat)** | 4 (13) | 1 (8) | 1 (6) | 5 (17) | 5 (17) | 1 (3) | 0 |
| **TEAE leading to study drug discontinuation** | 2 (7) | 0 | 0 | 1 (3) | 3 (10) | 1 (3) | 1 (6) |
| **TEAE potentially related to study drug** | 12 (39) | 1 (8) | 3 (19) | 1 (3) | 7 (24) | 27 (69) | 11 (61) |
| **Gastrointestinal disorders** | 22 (71) | 2 (15) | 3 (19) | 14 (48) | 16 (55) | 26 (67) | 11 (61) |
| **Diarrhea** | 13 (42) | 1 (8) | 1 (6) | 5 (17) | 8 (28) | 17 (**44**) | 8 (**44**) |

# Mirum: Launch Ready!

## ALGS & PFIC Care is Concentrated Enabling Focused Commercialization

### Planning for US launch H2 2021

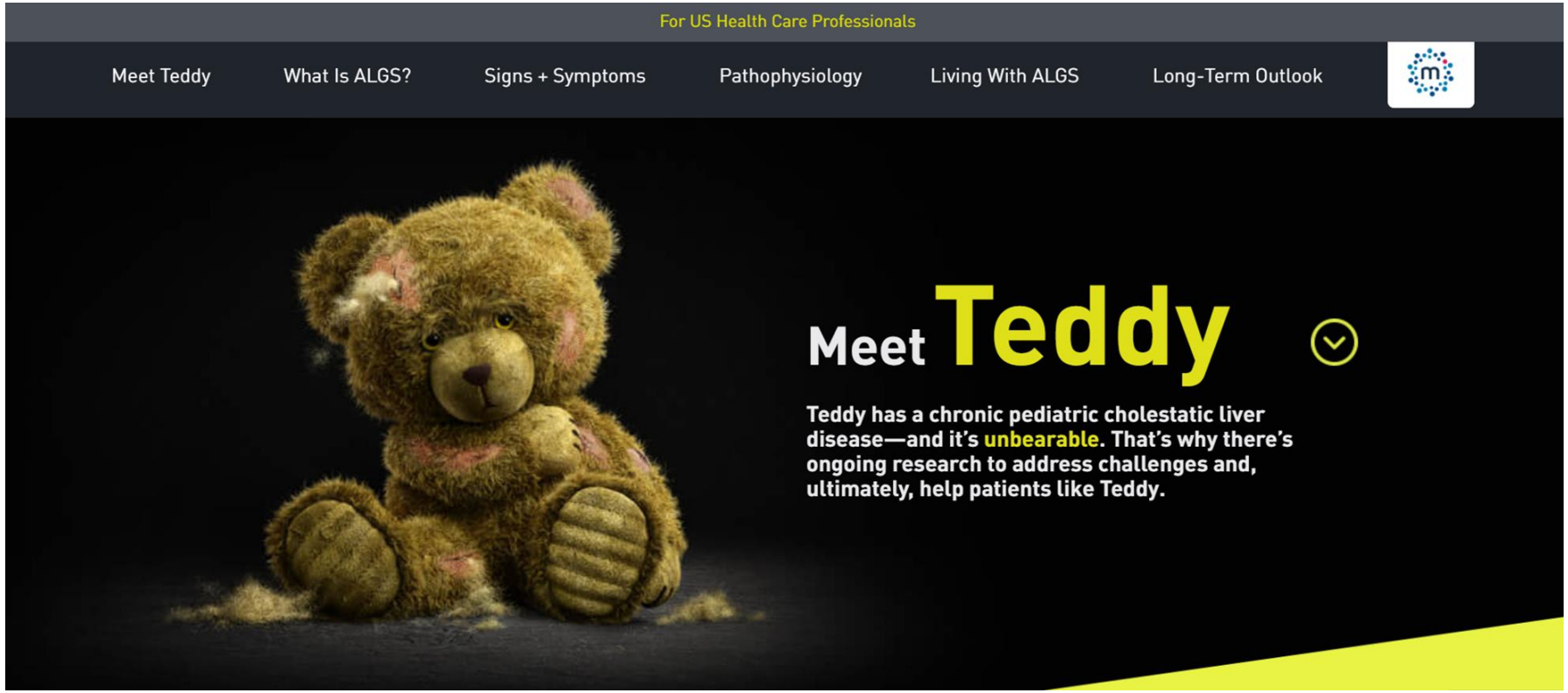
**Maralixibat commercialization**

**Potential alternative to liver transplant for cholestatic pruritus in ALGS**

- **Experienced team: 50+ commercializations**
- **Focused footprint: 125 accounts = 80% of cases**
- **Mirum leadership in place for medical, sales & payer field team**
- **Patient hub, call center, distribution providers contracted (Eversana)**

# Increasing Awareness of ALGS in GI/Hep Community

**Campaign launched on Alagille Awareness Day (January 24, 2021)**



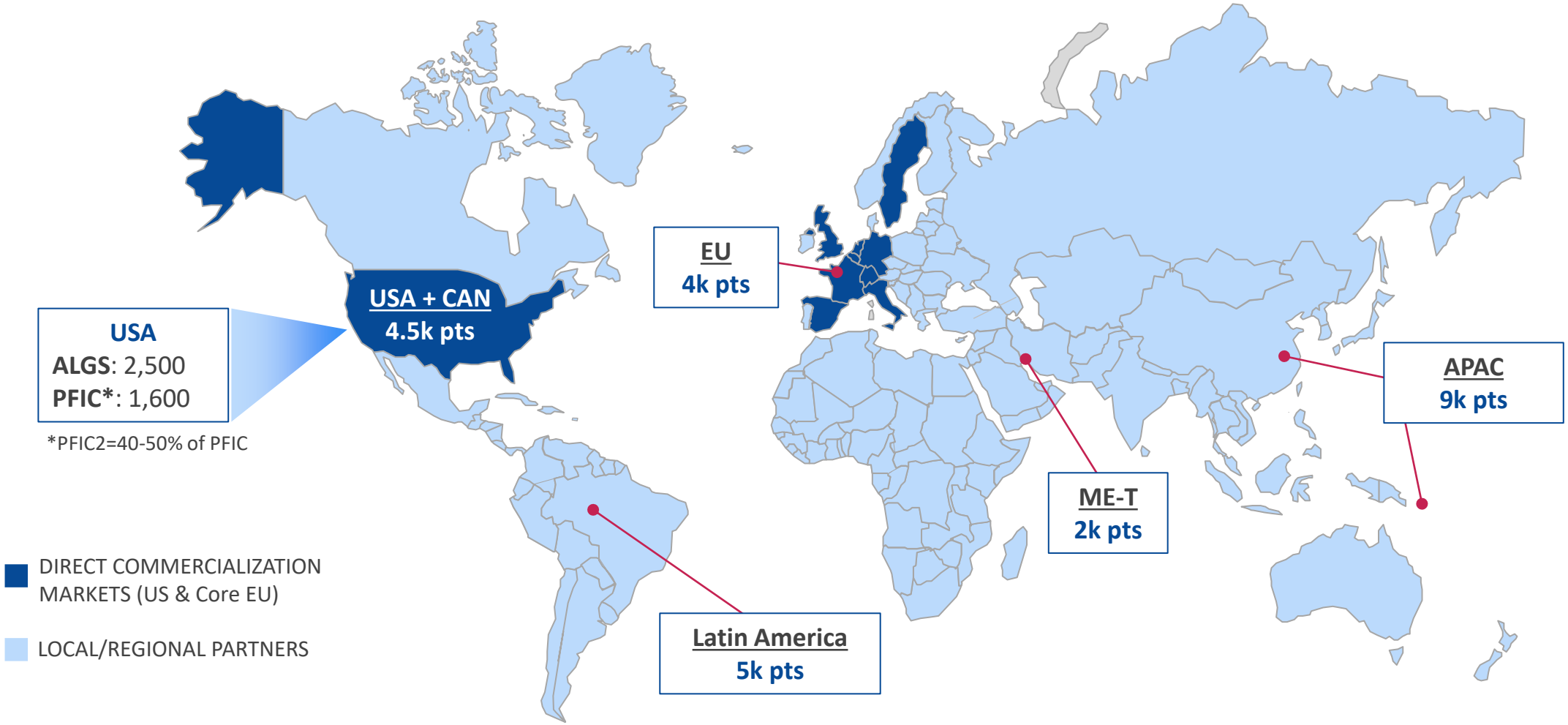

**https://unbearableALGS.com/**

**Goal**: Drive awareness of ALGS

- Improve time to diagnosis
- Educate on pathophysiology, including link between bile acids and clinical manifestations
- Appreciation of full burden of pruritus

# Worldwide Commercial Rollout Planned Through Direct Mirum Presence in U.S. & Core EU Countries, and Partnerships in Other Regions

## Estimated Worldwide Pediatric ALGS and PFIC Prevalence: >20K




Sources: Population based on UN Population and Vital Statistics Report – US 323m; South America Estimates derived from primary market research, literature epidemiology & local partner estimates

# Maralixibat for PFIC

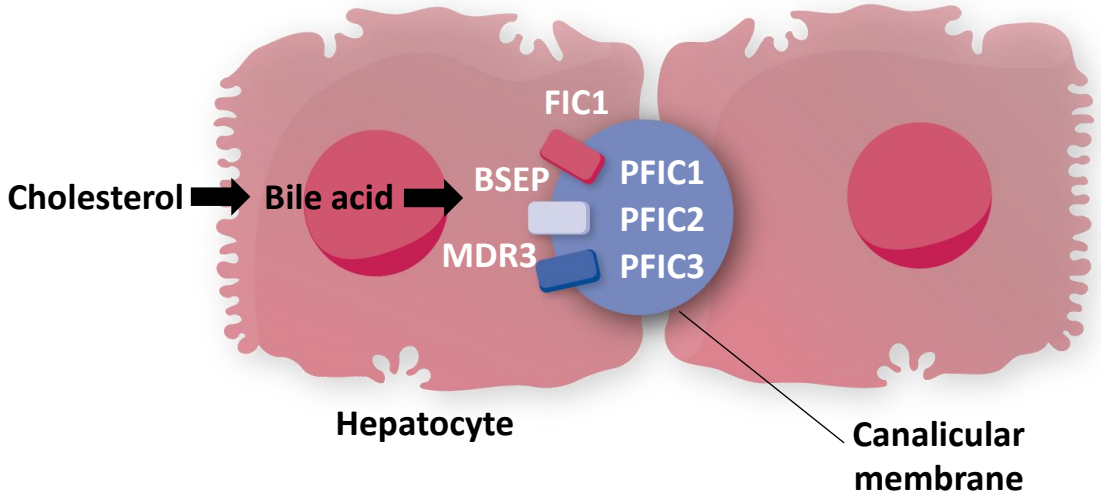
Dramatic and durable response in nt-PFIC2 patients

# PFIC: Genetic Diseases of Bile-Related Transporters

## PFIC2 (BSEP deficiency)

- Genetic disease caused by mutations in proteins that control bile flow, resulting in cholestasis
- Sever pruritus common, no approved medications to treat PFIC
- Signs of PFIC occur during infancy
- ~20% transplant-free survival at 18 years age
- Incidence of 1:50,000 to 1:75,000 births

PFIC sub-types impact different transporters. PFIC2 is the most common.




## Signs of cholestasis include

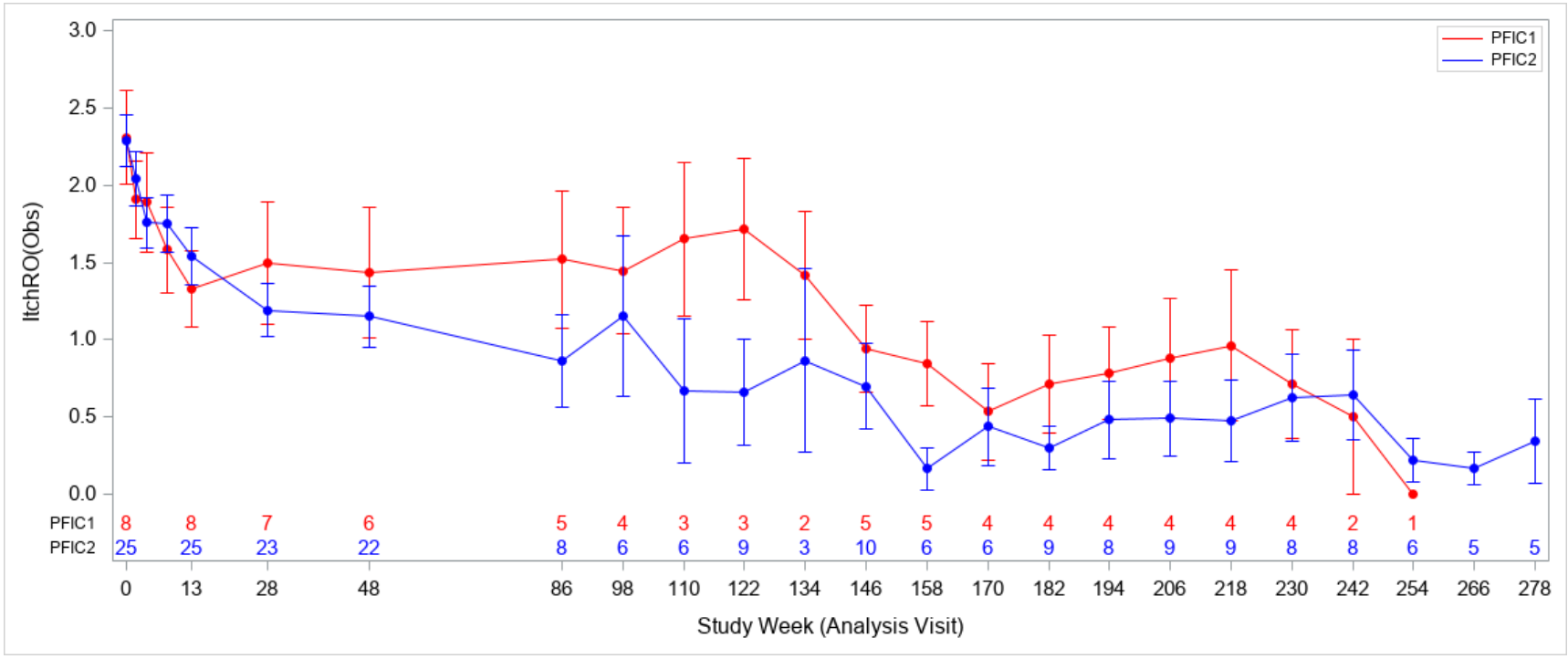
- Severe itch, AKA pruritus
- Yellow skin or eyes, AKA jaundice
- Stunted growth
- Portal hypertension
- Enlarged liver and spleen

# INDIGO: Phase 2 Open-Label Study in Children With PFIC

**INDIGO PHASE 2**

- **N = 33 children with PFIC**
  - (8) PFIC1
  - (25) PFIC2
    - 19 nt-PFIC2 *target indication*
- **Patients received MRX:**
  - 266 µg/kg QD[1] – years 1 & 2
  - 266 µg/kg QD or BID – years 3-6
- **Mean baseline characteristics**
  - Age: 4.2 years
  - sBA: 352 µmol/L
  - ItchRO(Obs): 2.3

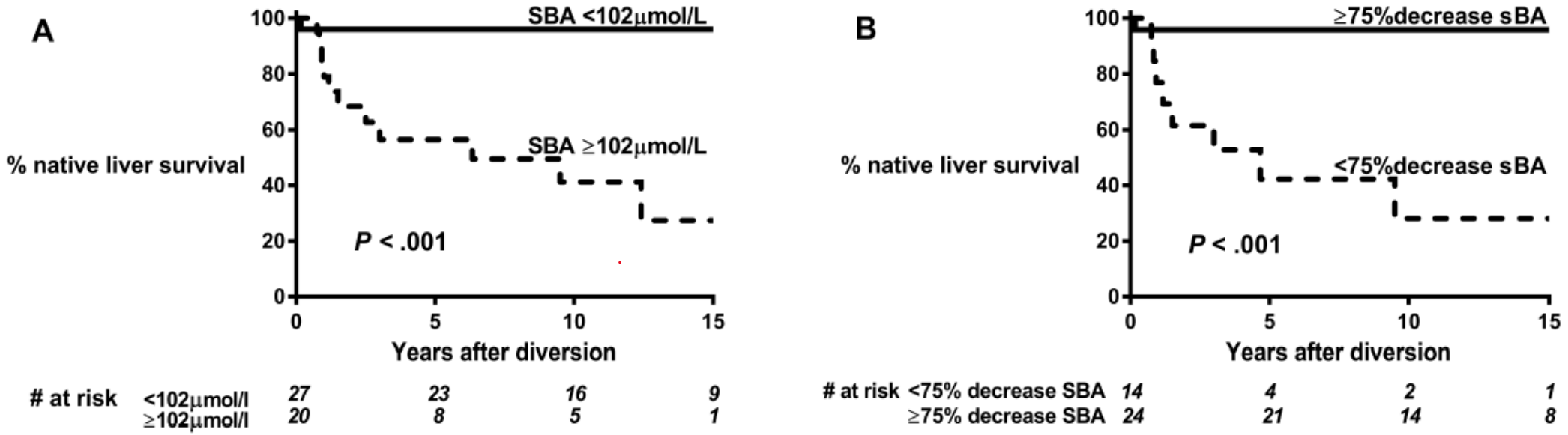
## ItchRO(Obs) Reported Pruritus Scores Over Time – PFIC 1 and 2




[1]Patients received doses of 280 µg/kg/day of maralixibat chloride (equivalent to 266 µg /kg/day of maralixibat)
Abbreviations: MRX: Maralixibat; BID: twice daily; QD: once daily

# sBA Control is Associated with Transplant Free Survival

Patients who achieved sBA threshold of <102 µmol/L or a 75% reduction, experienced NLS to 15 years, post-surgical biliary diversion (data from the NAPPED consortium)

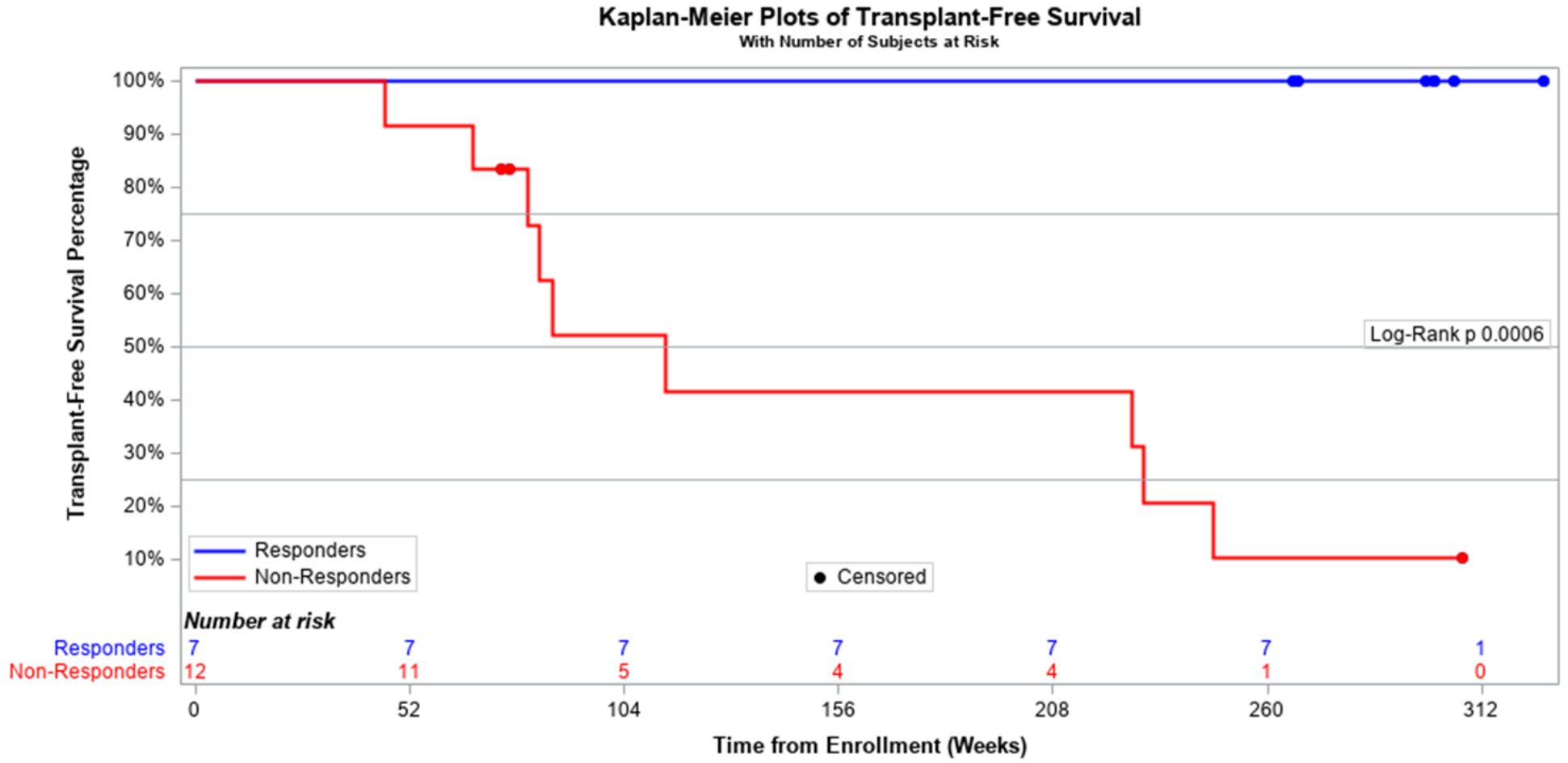



Van Wessel et al., 2020; NLS: Native Liver Survival (Transplant Free Survival)

# INDIGO: sBA Response Associated with Transplant-Free Survival

## 100% maralixibat sBA responders remain transplant free after >5 years of treatment

Improvement or normalization of liver parameters, growth, quality of life



# Five-Year Transplant-Free Survival Incorporated in MAA

## PFIC Registration Strategy

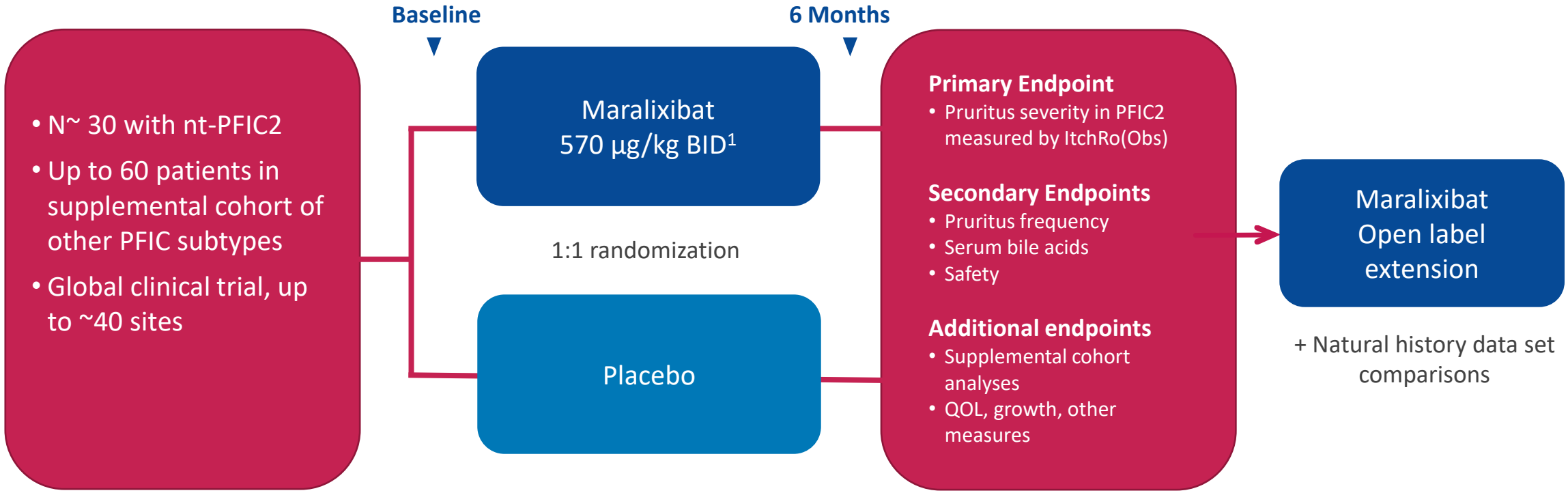
**EU**

- MAA validated for treatment of PFIC2
- Significant responder transplant-free survival
- Event-free survival advantage vs. natural history cohort

**US**

- Parallel approach
  - MARCH-PFIC Phase 3 ongoing
  - Gaining feedback on transplant-free survival data potential earlier filing or broader label

# MARCH-PFIC: Phase 3 Clinical Trial Enrolling




Estimated Enrollment Completion Q2 2021

[1]Patients received doses of 600 µg/kg/day of maralixibat chloride (equivalent to 570 µg /kg/day of maralixibat)
Abbreviations: BID: twice daily; QoL: Quality of LIfe

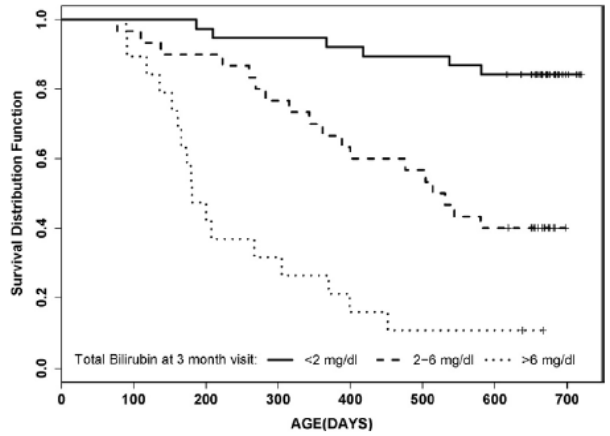
# Biliary Atresia

# Biliary Atresia Is an Inflammatory Cholangiopathy of Infancy

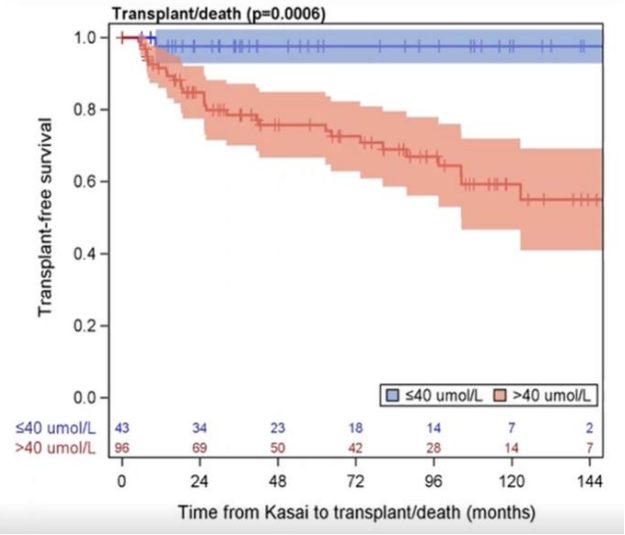
- Necro-inflammatory destruction of the bile ducts involving intra- and extrahepatic bile ducts
- Bile accumulation in the liver, progressive cholestasis and liver damage
- Fatale without Kasai procedure (HPE), standard of care within the first weeks of life
- 0.5 to 0.8 per 10,000 live births[1], #1 cause of pediatric liver transplant
- Post-Kasai, steroids, antibiotics, immunoglobulins used with no efficacy

**Risk of progression to transplant post-HPE associated with:**

Bilirubin[2]



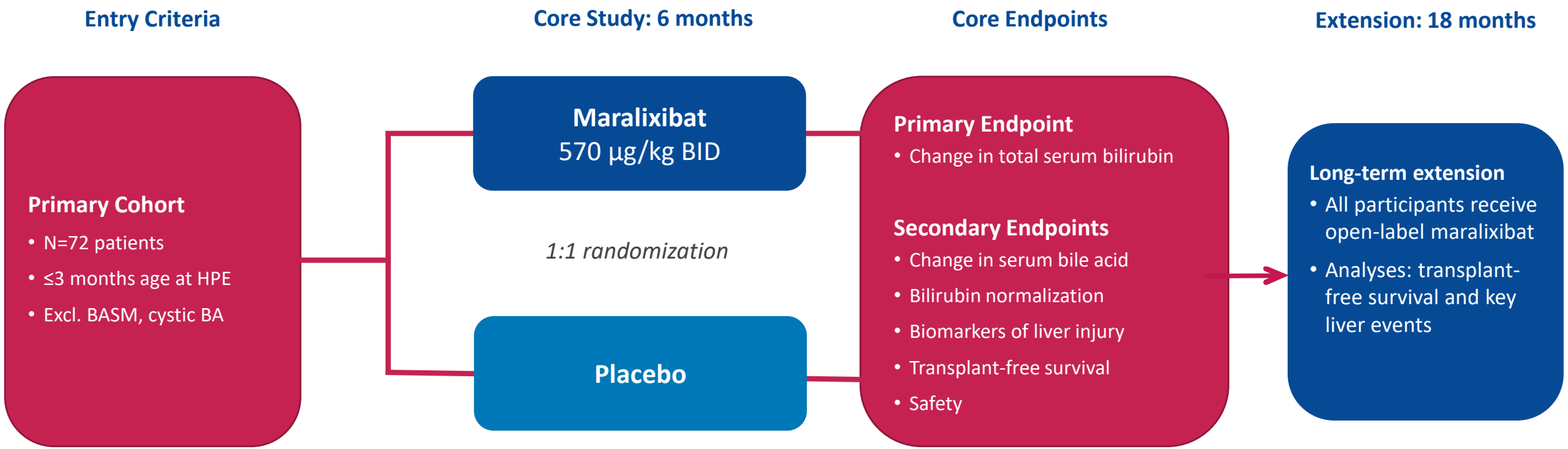

sBA[3]




1. Sanchez-Valle, A et al. Biliary Atresia Epidemiology, Genetics, Clinical Update, and Public Health Perspective, Advances in Pediatrics 64 (2017), [2]Shneider BL, Brown MB, Haber B, et al. A multicenter study of the outcome of biliary atresia in the United States, 1997 to 2000. J Pediatr. 2006;148(4):467–74; [3]Harpavat et al, AASLD 2020)

# MRX-701 The EMBARK Study Design

## Analyzing 6-month bilirubin biomarker before long-term transplant-free survival




**FPI expected in Q1 2021**

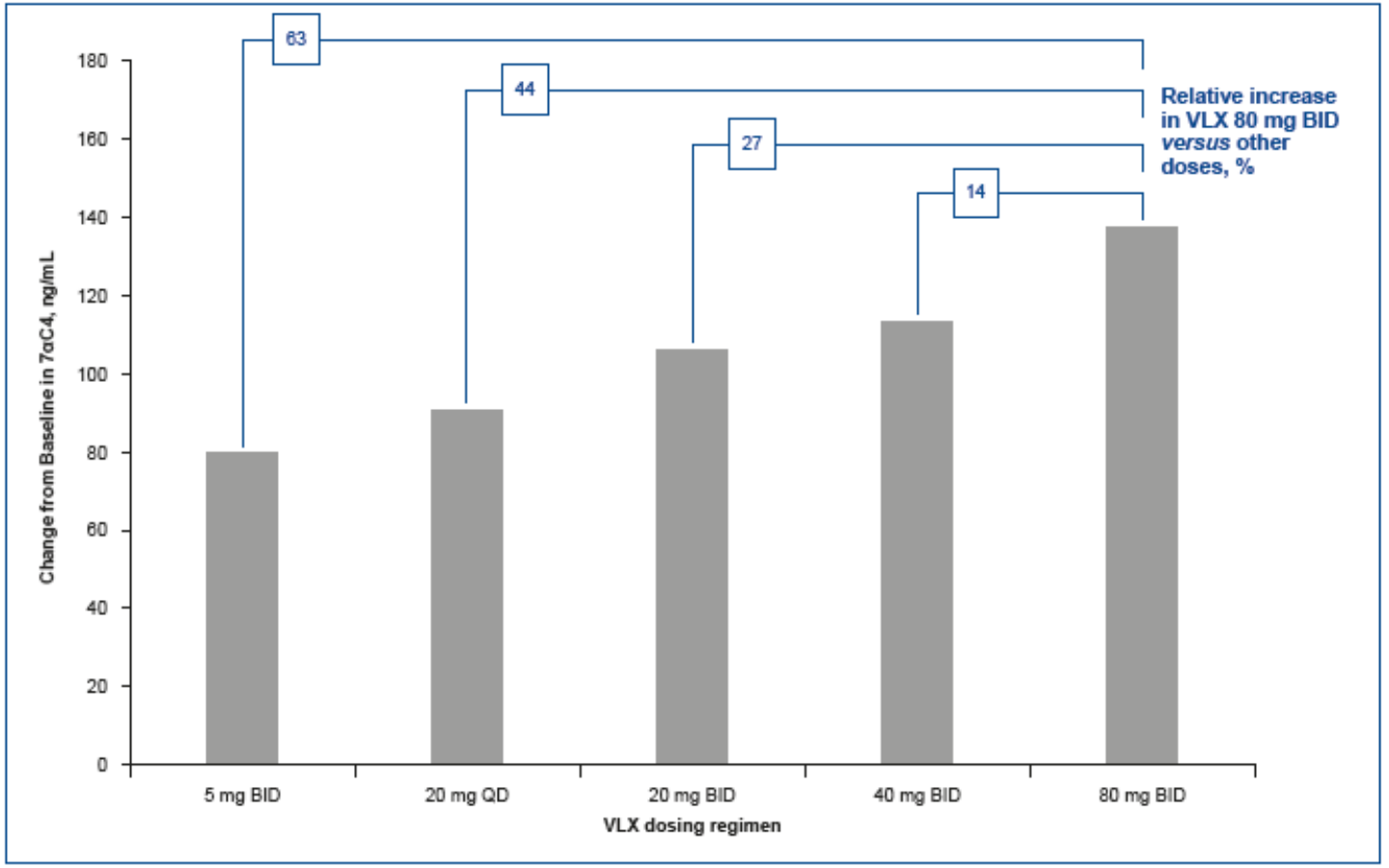
# Volixibat

## ASBTi for Cholestasis in Adults

# Volixibat Highly Active on Increasing Bile Acid Excretion

## Broad Phase 1 and Phase 2 data set informs dose selection for registrational program

- Dose ranging shows highly active on fecal bile acid excretion
- Resultant dose-related increase in 7αC4
- Supported by 48-week safety data in prior studies at lower doses




[a]Baseline for fBA was defined as Day 5. Thus, the summary represents the data at 7 days after Baseline
7αC4, 7 alpha-hydroxy-4-cholesten-3-one; BID, twice daily; fBA, fecal bile acid; QD, once daily; VLX, volixibat

Company data on file

# Treatment of Adult Patients with Rare Liver Diseases

## Intrahepatic Cholestasis of Pregnancy (ICP), Primary Sclerosing Cholangitis (PSC) and Primary Biliary Cholangitis (PBC)

### High Unmet Need

### Incidence

**ICP:**
**~1% of pregnancies**
***Up to ~5% in specific populations***

**PSC:**
**~29,000 in US**

**PBC:**
**~130,000 US**

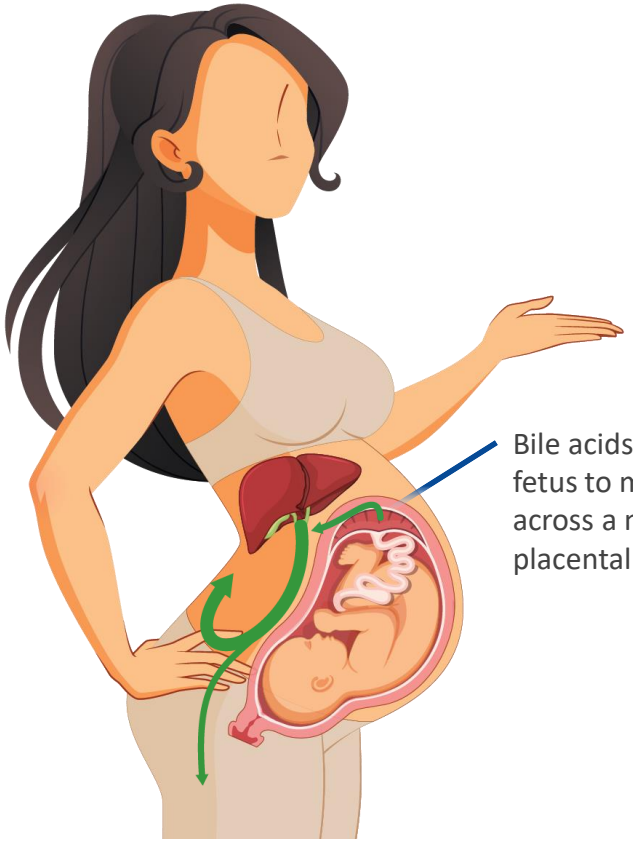
### Limited Tx Options

**ICP, PSC - No Approved Therapies**
**PBC – Treatments Sub-optimal**

Byron D, Minuk GY, Hepatology 1996; Bambha et al, Gastroenterology 2003; Geenes V, Williamson C, World Journal of Gastroenterology 2009; Lee et al, J Perinatol. 2006; Jones, 2019

# Pathophysiology of ICP

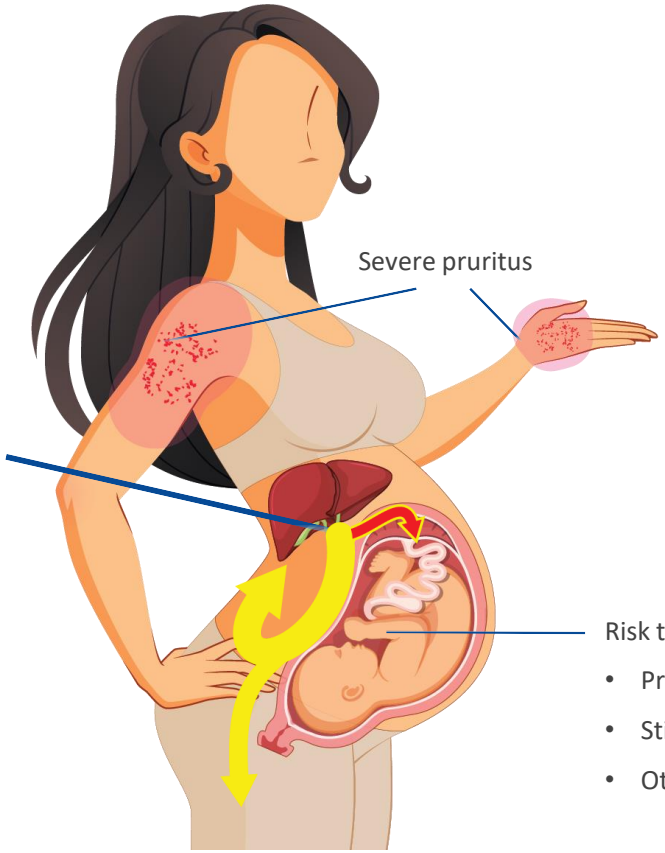
## Normal Pregnancy



Bile acids flow from fetus to mother across a natural placental gradient

## ICP Pregnancy

Maternal cholestasis; Accumulation of bile acids reverses natural bile acid gradient, exposing fetus to toxic bile acids




Risk to Fetus:

- Preterm labor
- Stillbirth
- Other complications

Monte et al, 1995; Shaw et al, 1982

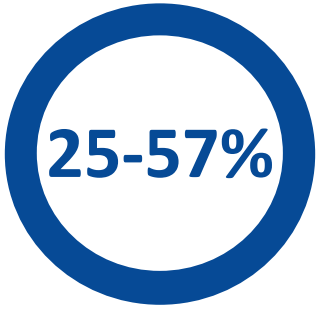
# Accumulation of Maternal BAs Drives Fetal Morbidity/Mortality

**Fetal morbidity and mortality:**

- High incidence of preterm birth
- Higher rates of asphyxia events, intrapartum meconium passage
- Increased risk of stillbirth and neonatal mortality

**Fetal morbidity and mortality associated with maternal serum bile acid levels:**

- For each additional µmol/L of sBA, probability of preterm delivery, asphyxia events, and intrapartum meconium passage increases by 1-2%
- A doubling in sBA levels doubles risk of stillbirth




ICP babies are born preterm

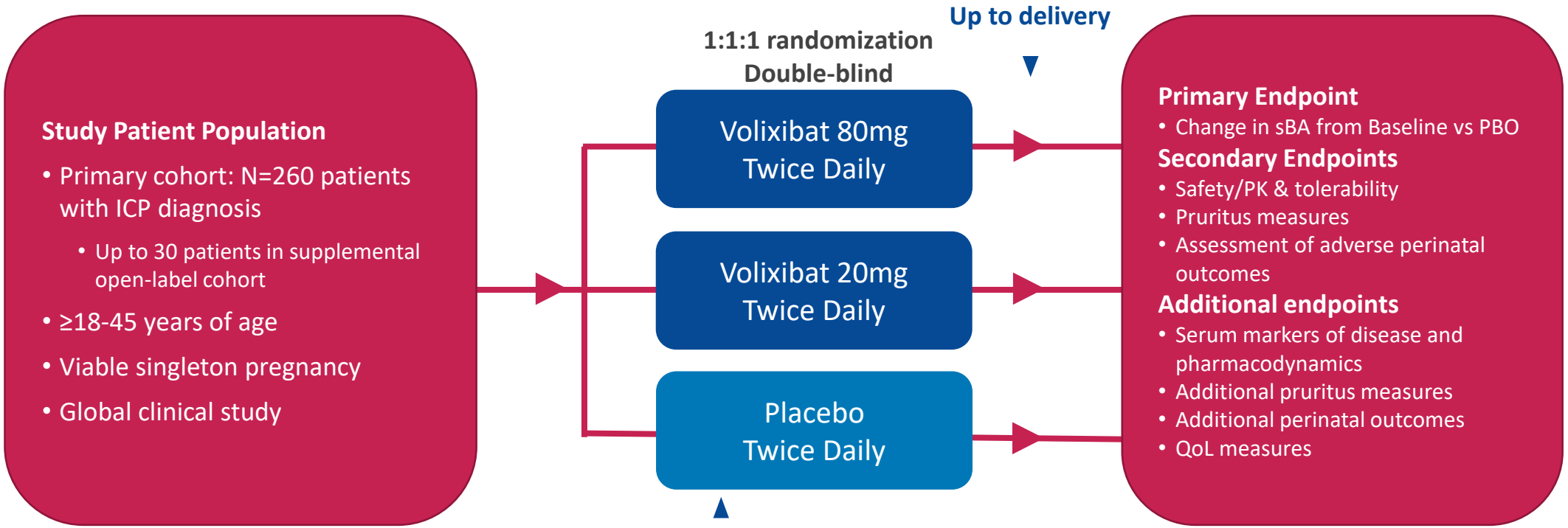

Average cost of preterm birth


Annual U.S. medical costs

Glantz et al, Hepatology 2004; Geenes et al, Hepatology 2014; Ovadia et al, The Lancet 2019; 2019 March of Dimes Report Card

# OHANA Study Design: FPI Q1 2021

## Evaluating reductions in sBA, pruritus and perinatal outcomes in ICP



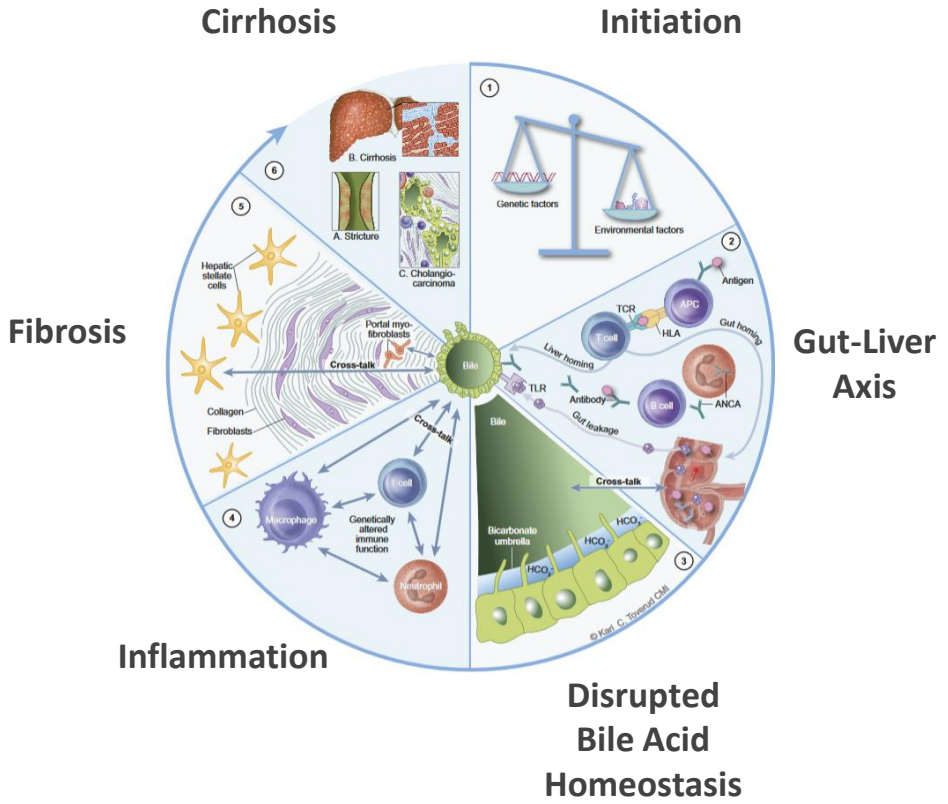

**Interim Analysis (IA) after N=60 complete study for**

- **Efficacy**
- **Safety/PK and tolerability**
- **Dose selection**

**Post-IA, N~200 for confirmatory phase with 1:1 selected dose : placebo**

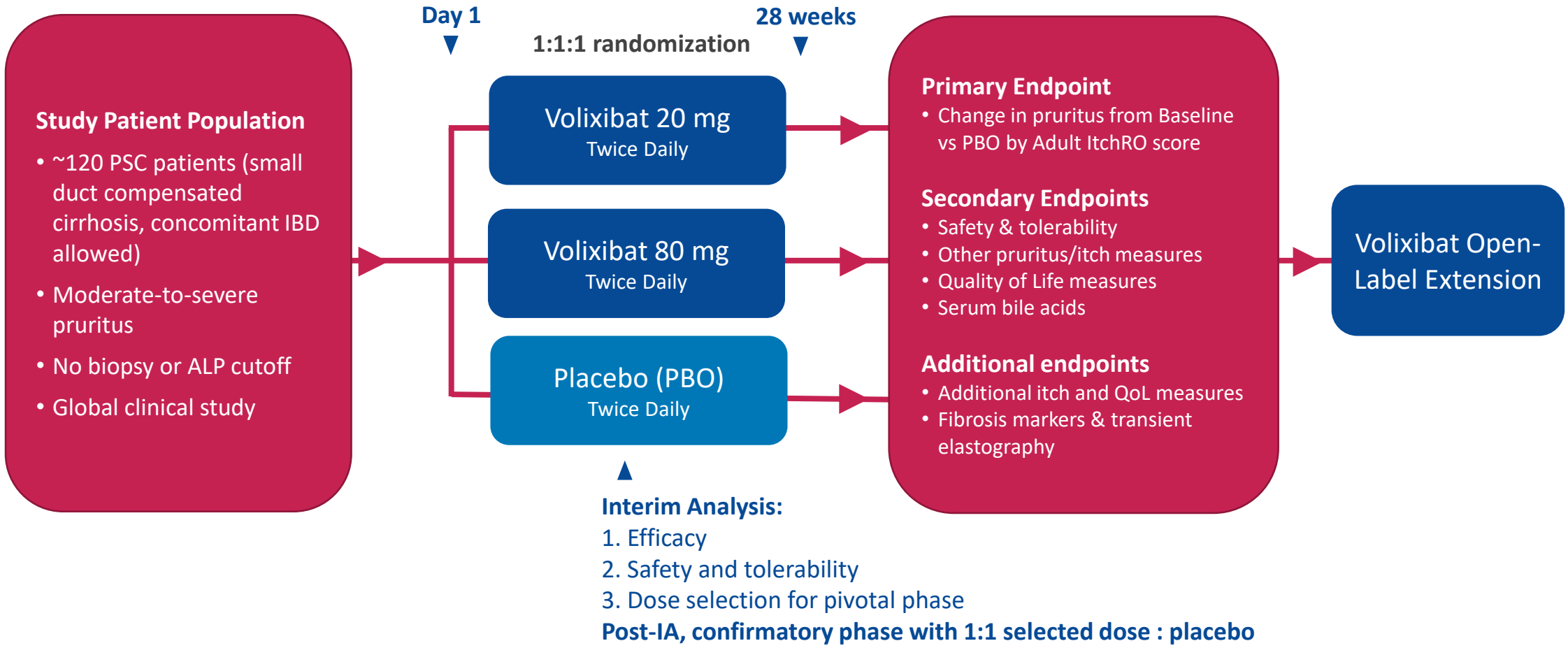
# PSC: A Chronic, Debilitating Cholestatic Disease

- **PSC is a chronic cholestatic disorder characterized by disrupted bile-acid homeostasis.**
  - ↑ sBA, ↓ 7αC4, ↑ FGF19
  - Altered expression levels and pattern of ABCB11 and ABCB4
- **Results in inflammation and fibrosis of the bile ducts**
  - May result in cirrhosis, liver failure, and/or liver cancer
- **Pruritus associated with PSC can lead to significant reductions in QoL**
  - Prevalence of pruritus is 70-80% during the course of disease
  - Refractory pruritus is an indication for liver transplant
- **Estimated incidence is 29,000 cases in the U.S. and 50,000 cases in Europe**



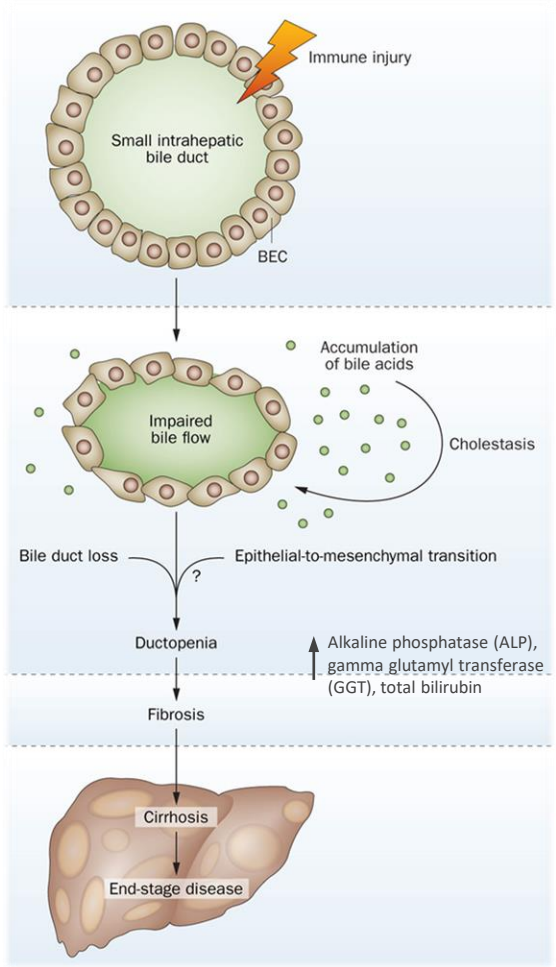
# VISTAS – Volixibat PSC Phase 2b

## Assessing pruritus reductions in PSC, setting with no approved therapies



# Volixibat in Primary Biliary Cholangitis (PBC)

**PBC: Rare cholestatic progressive liver disease**



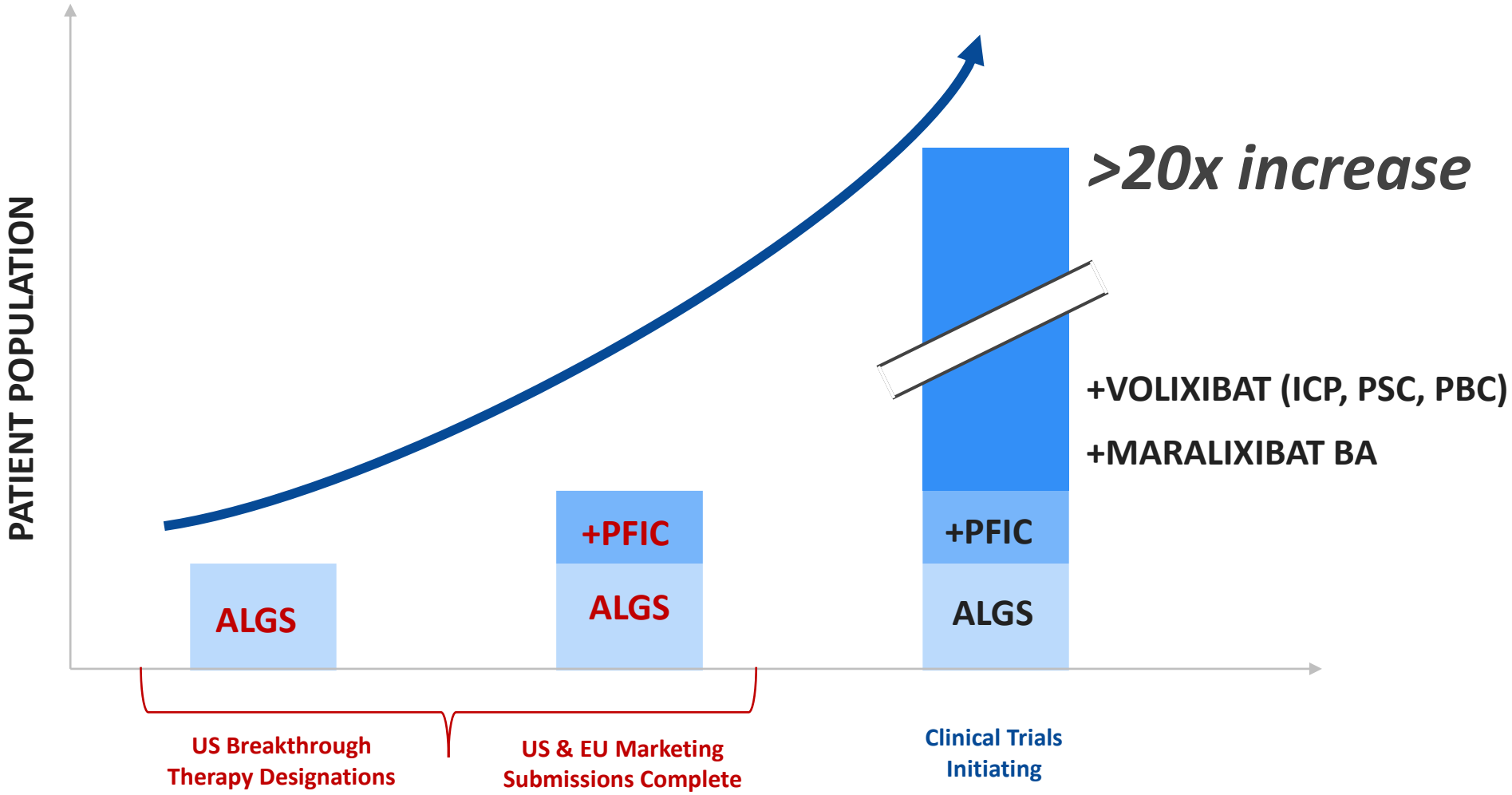

- Estimated prevalence ~130k (U.S.); 10:1 female-to-male distribution
- Current treatment options:
  - 1st line treatment with Ursodeoxycholic Acid (UDCA)
  - 2nd line (~30% of patients ALP $\geq$ 1.67 ULN) Ocaliva
- Pruritus (itching) and fatigue are common and not correlated with ALP
  - Up to 70% of patients report itching
  - No approved therapies for pruritus
- Volixibat program focused on cholestatic pruritus in first or second line
  - Pruritus provides near-term clinical outcome
  - Phase 2b study planned to start 2H 2021

Jones, 2019; Hegade et al 2019; Kuiper et al, 2009; Poupon et al 1997; Dyson et al, 2015
Abbreviations: ALP: alkaline phosphatase; ULN: upper limit of normal

# Mirum

Leading the Way in Rare Liver Disease

# Opportunity to Significantly Expand in Cholestasis




Note that Marketing Application for PFIC2 is under review by EMA and rolling NDA submission for cholestatic pruritus associated with ALGS complete with FDA

# Strong Balance Sheet: Projected 3+ Years Runway

## Strategically financed for growth

- Cash, cash equivalents, investments: $231.8 as of Dec 31, 2020
- $75 million financing completed December ‘20
- $210 million structured financing with Oberland Capital
  - $60 million received December ‘20
  - Up to $150 million based on maralixibat milestones and new product acquisition
  - Repaid with capped royalty on maralixibat
- Eligible for priority review voucher if maralixibat approved for ALGS or PFIC

| SELECTED BALANCE SHEET DATA | Sept. 30, 2020 | Dec 31, 2019 |
|---|---|---|
| Cash, cash equivalents and investments | $ 133.7 | 140.0 |
| Total Assets | 141.9 | 146.7 |
| Total stockholders’ equity (deficit) | 120.3 | 130.3 |

| | Three Months Ended | |
|---|---|---|
| SELECTED STATEMENTS OF OPERATIONS DATA | Sept. 30, 2020 | Sept. 30, 2019 |
| Operating expenses: | | |
| Research and development | 16.0 | 12.2 |
| General and administrative | 5.7 | 3.7 |
| Total operating expenses | 21.7 | 15.9 |
| Net loss | (21.5) | (15.1) |

*$ in millions*

# Where We Are Going: Path to Value Creation

## 2021

- ✓ Q1: Maralixibat rolling NDA submission completion
- Q1: Study initiations
  - Phase 2 biliary atresia (maralixibat)
  - Phase 2 ICP (volixibat)
  - ✓ Phase 2 PSC (volixibat)
- Q2: MARCH complete enrollment
- H2: Maralixibat FDA approval in ALGS
- H2: Initiate Phase 2 PBC study (volixibat)
- Year-end: MARCH-PFIC topline data

## 2022

- PFIC2 approval in Europe with maralixibat
- ICP interim data
- PFIC U.S. filing
- ALGS EU filing

*Anticipate milestones subject to those risks and uncertainties described in our filings with the Securities and Exchange Commission, including those risks and uncertainties arising out of the COVID-19 pandemic.

# Leading the Way in Rare Liver Disease

## Bringing Life-Changing Medicines to Patients in Need

Experienced team, multiple commercializations

NDA submitted for ALGS

MAA validated for PFIC2

Pipeline of indications directly targeting cholestasis: ICP, PSC, PBC, BA

Near-term launch potential, multiple indications, total market potential $1B+

Abbreviations: BTD – Breakthrough Therapy Designation (FDA); RPDD – Rare Pediatric Disease Designation (FDA); ALGS – Alagille syndrome; PFIC – Progressive Familial Intrahepatic Cholestasis; BSEP – Bile Salt Export Pump; nt-PFIC2 – non-truncating PFIC2; EMA – European Medicines Agency; MAA – Marketing Authorization Application; Note that Marketing Application for PFIC2 is under review by EMA and rolling NDA submission for cholestatic pruritus associated with ALGS complete with FDA

# Thank you!

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