Mirum Pharmaceuticals:

Transforming Lives in Rare Disease

July 2023



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Leader in Rare Liver Disease: 2023 Goals

INDUSTRY LEADING TEAM, DELIVERING REMARKABLE TREATMENTS TO PATIENTS IN NEED WORLDWIDE

Build on US LIVMARLI ALGS Commercial Success

Establish LIVMARLI in Additional Pediatric Cholestatic Settings



Launch LIVMARLI Across International Markets

Leverage IBAT Inhibition Expertise in Adult Cholestatic Liver Disease

Expand Pipeline to Leverage Rare Disease and Commercial Expertise

Potential Acquisition of Travere's Bile Acid Product Portfolio

DEAL RATIONALE:

- Solidifies Mirum's leadership on pediatric hepatology/GI
  - Accelerates LIVMARLI growth
  - Bile Acid portfolio addressing multiple high need settings
  - Branded diagnostic: Mirum as a key partner from the start of patient journey

- Building size and scale to drive future growth in rare disease
  - Combined Q1 2023 run rate revenue ~$220M
  - Significant cash flow generation to fuel pipeline, expansion in rare disease
  - Leverage strength of Mirum commercial capabilities

- Financed with a concurrent oversubscribed private placement of $210M



Significant Revenue & Cash Flow Expected

$103 Million

2022 NET REVENUE

Cholbam (cholic acid) capsules CHENODAL (Chenodiol Tablets 250 mg)



Deal Terms

Travere Bile Acid Portfolio

$210 Million

UPFRONT

Plus up to $235 million sales milestones, tiered from $125 to $500 million annual sales



Concurrent Financing

$210 Million

Committed PIPE

Mirum Proven Execution Track Record, Delivering Value

EFFICIENT BUSINESS MODEL GENERATING TOPLINE GROWTH WITH MULTIPLE NEAR-TERM VALUE CATALYSTS



LIVMARLI – approved for cholestatic pruritus in Alagille Syndrome (ALGS) with Potential Indication Expansions



Robust Pipeline Opportunity with a Validated Scientific Rationale



Strong Foundation for Growth with a Record of Commercial Excellence

$31.6M[1]

TOTAL REVENUE Q1 2023

- ✓ 2022 total revenue $77.1M
- ✓ US business positioned for 50% year over year growth in 2023
- ✓ International markets launching

4 Late-Stage

INDICATIONS

- ✓ Several catalysts in 2023
- ✓ PFIC approvals anticipated year end
- ✓ IBATi effective across multiple liver settings

~$232M[1]

STRONG BALANCE SHEET

- ✓ Commercial business cash flow positive
- ✓ Continue to build operating leverage as revenue grows
- ✓ Strengthened balance sheet and strategic flexibility[2]

DELIVERING LIFE-CHANGING RARE DISEASE MEDICINES

[1] Cash, cash equivalents, restricted cash equivalents and investments; Estimate as of March 31, 2023.
[2] Closed upsized offering of $316.3 million aggregate principal amount of 4.00% convertible senior notes due 2029 , including exercise of the initial purchasers' option in full. Repurchased 9.75% royalty obligation, removed restricted cash provision on balance sheet.

Commercial-Stage with Pipeline of Growth Opportunities

3 APPROVED RARE DISEASE PRODUCTS, 5 ADDITIONAL INDICATIONS IN DEVELOPMENT IN HIGH-NEED ORPHAN INDICATIONS

Livmarli® (maralixibat) oral solution

*Alagille syndrome**

Cholbam® (cholic acid) capsules

CHENODAL® (Chenodiol Tablets 250 mg)

Bile acid portfolio

		Preclinical	Phase 1	Phase 2b/Phase 3	Approved
LIVMARLI (maralixibat) Oral Solution	Progressive Familial Intrahepatic Cholestasis	MARCH-PFIC submitted for label expansion		FDA PDUFA Dec 13, 2023 EMA Q1 2024	
	Biliary Atresia	EMBARK, H2 2023 data			
Volixibat	Primary Sclerosing Cholangitis	VISTAS, interim data H2 2023			
	Primary Biliary Cholangitis	VANTAGE, interim data H2 2023			
Chenodal	Cerebrotendinous Xanthomatosis (CTX)	RESTORE, Phase 3 data H2 2023			

*Received U.S. FDA approval for cholestatic pruritus in patients with ALGS 3 months of age and older.
European Commission has granted marketing authorization for LIVMARLI® (maralixibat) oral solution for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS) two months of age and older.

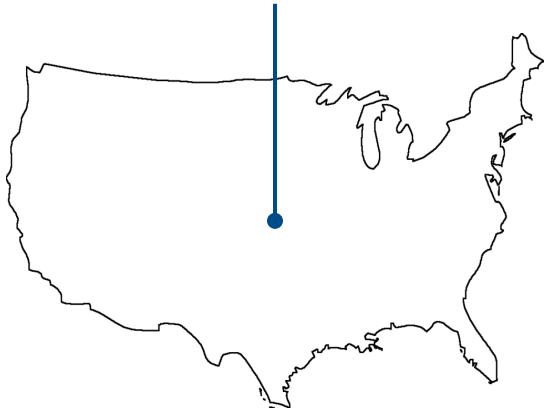
Establishing Leadership on the Rare Pediatric Liver Call Point

mirum

US Pediatric Liver Prescriber Landscape

50 Major Accounts

180 Total Account Universe



Livmarli® (maralixibat) oral solution

>300

ALGS PATIENTS TREATED (US-COMMERCIAL)
SUBSTANTIALLY ALL KEY ACCOUNTS HAVE PRESCRIBED

Cholbam® (cholic acid) capsules

CHENODAL® (Chenodiol Tablets 250 mg)

Standard of Care for Bile Acid Synthesis Disorders and CTX

PREVENTION GENETICS

Cholestasis Panel (77-gene)

Branded Cholestasis Diagnostic Platform

Mirum Positioned as a Partner from the Start of the Patient Journey



Patient Identification & Diagnosis

Free, Mirum sponsored & branded cholestasis genetic testing panel

mirum access PLUS
Navigating Together

Access & Awareness

A Customer-Focused Approach to Drive Uptake & Retention

CHENODAL®
(Chenodiol Tablets 250 mg)
Livmarli®
(maralixibat) oral solution
Cholbam®
(cholic acid) capsules

Treatment Options

Broad portfolio addressing spectrum of genetic metabolic cholestatic diseases

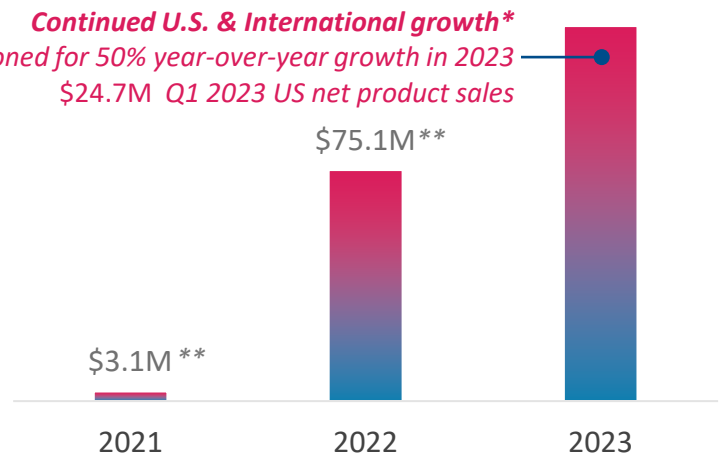
Building Leadership to Accelerate Livmarli Performance

Significant headroom remains to grow Livmarli in ALGS and new indications in US

Livmarli® (maralixibat) oral solution

Total Net Product Sales

Continued U.S. & International growth*
US business positioned for 50% year-over-year growth in 2023
$24.7M Q1 2023 US net product sales



Rare Disease Commercial Excellence

Best in class white glove patient services program

Rapid order to fill; >95% reimbursement

High persistence, low discontinuation rate

>$1 Billion

LIVMARLI US Opportunity
Strengthened by Bile Acid Product Acquisition

ALGS
- 2k– 2.5k US prevalent patients + newly diagnosed
- LIVMARLI Rx'd to 20%+ of addressable patients
- Expanded presence strengthens share of voice

PFIC
- 500+ prevalent patients + newly diagnosed
- PDUFA date December 2023

BA
- ~350 newly diagnosed patients per year
- Data expected H2 2023

*Based on management's current estimates and beliefs
**2021 Net Product Sales comprise US sales only; 2022 Net product sales comprised of US and International sales

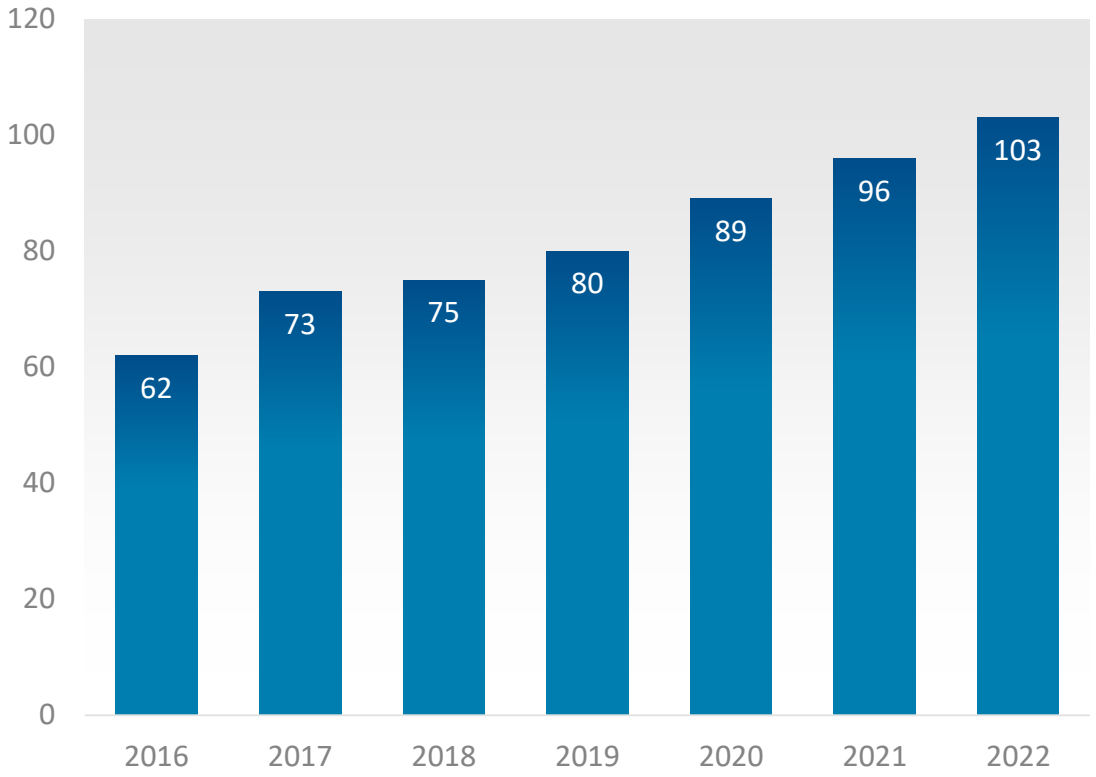
A Mature, Profitable Business With Strong Commercial Synergies

Steady Growth in Bile Acid Portfolio

Net Product Sales

Cholbam® (cholic acid) capsules

CHENODAL® (Chenodiol Tablets 250 mg)



Source: Travere 10K

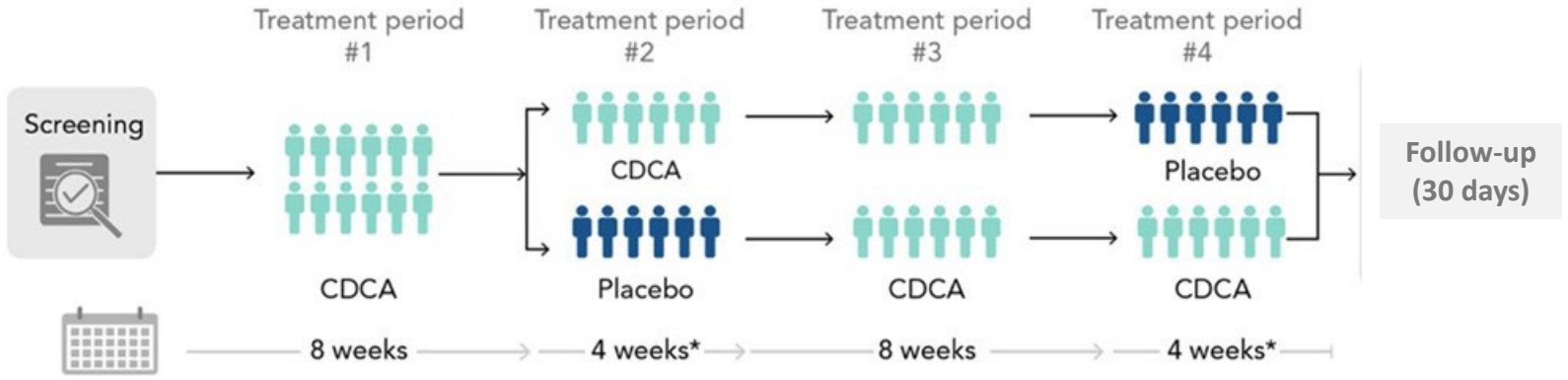
Bile Acid Portfolio Indications

	US Prevalence	Overview
Bile Acid Synthesis Disorders (BASD)	~200-300	Group of genetic disorders with abnormal bile acid production
Zellweger spectrum disorders (ZSD)		Genetic, multisystem condition caused by the loss of peroxisome function, affecting bile acid production
Cerebrotendinous Xanthomatosis (CTX)	~1-2k	Genetic disorder with toxic cholestanol & bile alcohol buildup FDA medical necessity status Opportunity for label expansion & orphan exclusivity ~10% diagnosis rate

RESTORE Phase 3: Potentially NDA enabling for CTX, Orphan Exclusivity

Top-line data in H2:2023

Adult Study (age 16+), n=12*



https://ctxrestore.com/#:~:text=What%20is%20the%20RESTORE%20study,participating%20in%20a%20research%20study

Primary Endpoint

- Change from baseline in Log-transformed urine total 23S-pentol at the end of each double-blind (DB) treatment period

Secondary Endpoints

- Proportion of patients requiring rescue medication during the DB periods
- % change plasma cholestanol (end of DB periods)
- % change plasma 7αC4 (end of DB periods)

High confidence for success on primary and key secondary biomarker endpoints

- Historical CTX studies with chenodiol** support a large effect on:
  - Serum cholestanol (70-75% reduction, stat sig)
  - Urinary bile alcohols (79-100% of patients improved, stat sig)

*Rescue CDCA available as needed if symptoms occur or lab values are out of the expected range for patients received CDCA or PBO. Separate pediatric study (1 month – 15 years, n=3) running concurrently
**Verrips, et al. "The Safety and Effectiveness of Chenodeoxycholic Acid Treatment in Patients with Cerebrotendinous Xanthomatosis: Two Retrospective Cohort Studies." *Neurological Sciences* 41, no. 4 (April 2020): 943–49. Chenodeoxycholic EPAR 2017

A Compelling Transaction to Reinforce Mirum's Rare Disease Leadership

New programs elevate and advance Mirum's strategy

- Adds commercialized medicines for pediatric hepatology
- Enhances the growth of LIVMARLI
- Expands operating and financial scale
- ~$220M combined run rate revenue, expect cash flow break even

Livmarli® (maralixibat) oral solution

CHENODAL® (Chenodiol Tablets 250 mg)

Cholbam® (cholic acid) capsules



Thank you