

# Q4/FY 2022 Earnings Call Presentation

Disclosed March 2, 2023

# Cautionary Note Regarding Forward-Looking Statements ("FLS")

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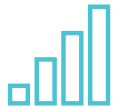
This document contains FLS, including regarding: financial guidance and sales and expense expectations; timing and results of our R&D, clinical trials, regulatory submissions, and new product initiatives; drug efficacy, safety, and tolerability; trends in prescriber and patient behavior and adoption of Ocaliva; our plans regarding launch of OCA in NASH; timing of FDA review of our NDA; and prospects for FDA approval of our NDA.

Important factors could cause actual results to differ materially from the FLS, including: the FDA could take longer than expected to review our NDA; OCA in NASH could not receive FDA approval in a timely manner or at all; the FDA could require us to provide additional information that is not timely or economical to provide; we could be unable to address to the satisfaction of the FDA the issues raised in its complete response letter of June 2020 responding to our earlier submission; there could be efficacy, safety, or tolerability concerns about OCA in NASH; OCA in NASH could have less commercial potential than anticipated or could be superseded by a competing product; if approved, we could be less effective than anticipated in launching OCA in NASH commercially; we may not be able to obtain or maintain regulatory approvals; we may not be able to satisfy post-marketing requirements, including using real-world evidence; the initiation, timing, cost, conduct, progress and results of our R&D activities, preclinical studies, and clinical trials, including regarding safety and efficacy; adverse medical, clinical, efficacy, quality, safety, or pharmacovigilance events or results from clinical trials; potential side effects associated with our products or product candidates; the outcomes of interactions with regulators including the FDA regarding clinical trials, safety and efficacy, products and product candidates, and regulatory approvals; marketing conditions, limitations, or warnings required by regulators; the degree of market acceptance of our products among physicians, patients, and healthcare payors; competition from new or existing drugs; the impact of the sale of our international business; our ability to manage successfully our commercial and operational performance; our ability to attract and retain key personnel; our estimates of future financial needs and results; and other factors discussed in the FLS and Risk Factors sections of our Form 10-Q and Form 10-K filings, and in our Form 8-K reporting our quarterly earnings.

# Key Business Updates



**U.S. Ocaliva® net sales of \$77.2 million and \$285.7 million for the fourth quarter and full year 2022; 13% and 10% growth over the prior year**



**Worldwide Ocaliva non-GAAP adjusted net sales of \$343.8 million for the full-year 2022**



**Fully resolved patent infringement case that was scheduled for trial on February 27, 2023; settlements protect Ocaliva market exclusivity into the 2030s**



**New drug application for OCA for the treatment of pre-cirrhotic liver fibrosis due to NASH accepted; PDUFA target action date set for June 22, 2023**



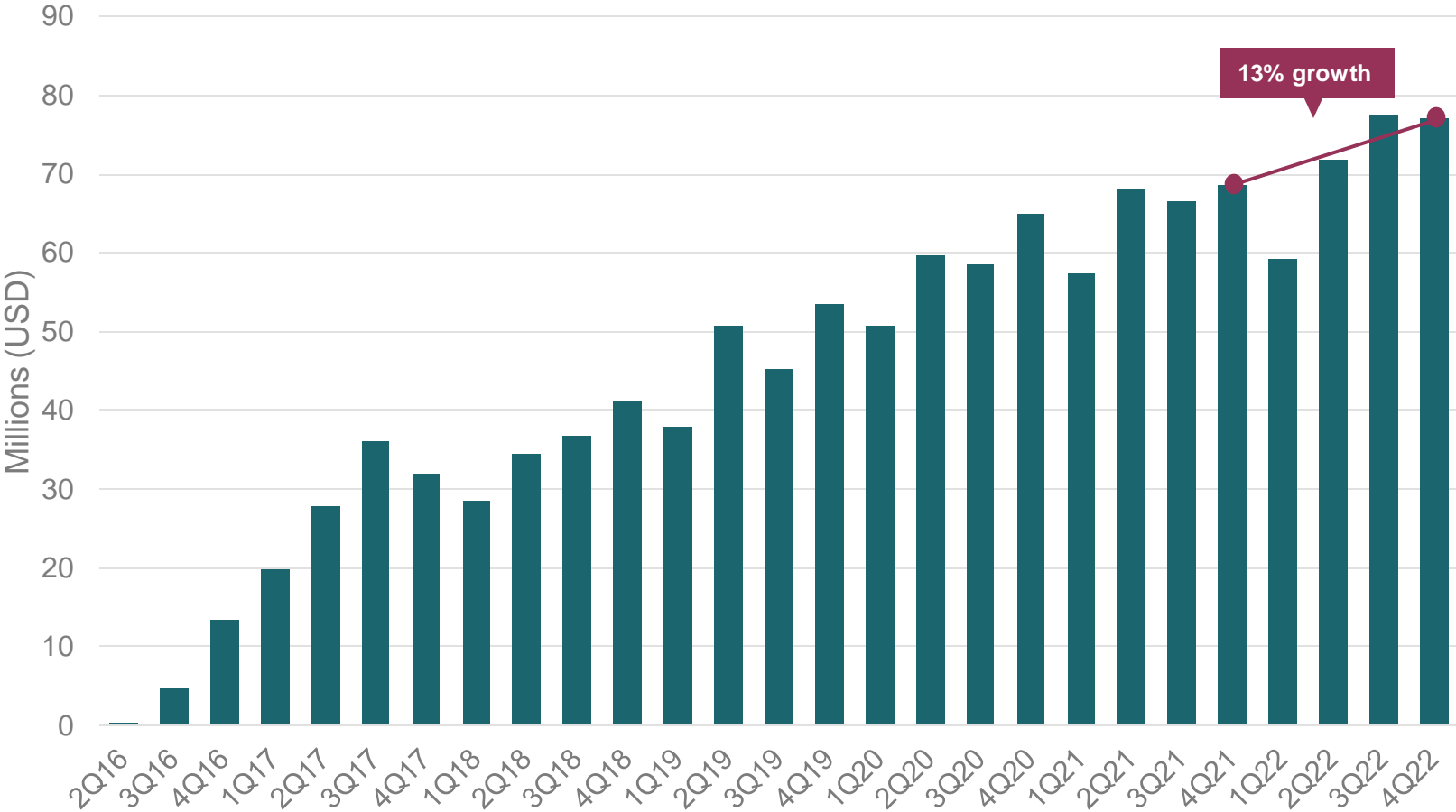
**Phase 2 OCA/bezafibrate fixed-dose combination studies progressing; announced lead indication in severe alcohol-associated hepatitis for next-generation FXR agonist, INT-787 and initiated Phase 2a FRESH study**



**Cash, cash equivalents, restricted cash, and investment debt securities available for sale of \$490.9 million as of December 31, 2022**

# Accelerated, Double-Digit Growth in Ocaliva with 13% Increase in U.S. Net Sales in 4Q22 vs. 4Q21

U.S. Ocaliva Net Sales



**U.S. Ocaliva net sales of \$77.2 million in 4Q22, bringing U.S. net sales to \$285.7 million for FY22**

# Long Runway for Growing PBC Business

## Market growth of Ocaliva

- 30% growth of new-to-brand prescriptions in 2H22
- 42% increase in first-time writers in 2H22 vs. 2H21
- 8-day improvement in specialty pharmacy time-to-fill in 2022
- 95% of business is driven by existing patients
- New HCP and patient marketing campaigns

## Long-term data to educate on Ocaliva and support fulfilling post-marketing requirements

- Preparing data from COBALT and supplementary real-world evidence from large datasets in the U.S., UK and Europe to include in regulatory submission to FDA in 2023
- Highlighting data from *Gastroenterology* showing OCA demonstrated 70% lower risk of death or liver transplant compared to control patients

## Fixed-dose OCA + bezafibrate combination for PBC

- One Phase 2 study is now fully enrolled; accelerating recruitment of patients into second Phase 2 study
- Synergistic MOAs with a potential to further lower key biochemical measures that predict long-term outcomes in PBC, while also improving tolerability

**Added certainty to runway of our life cycle with resolution of our patent infringement case for Ocaliva; settlements protect Ocaliva market exclusivity into the 2030s**

# Unique Opportunity with OCA to Help Patients With Pre-Cirrhotic Fibrosis due to NASH

Risk of liver-related mortality increases exponentially with increasing fibrosis stage<sup>1</sup> and patients with advanced fibrosis are at the greatest risk

No NASH medications are currently approved

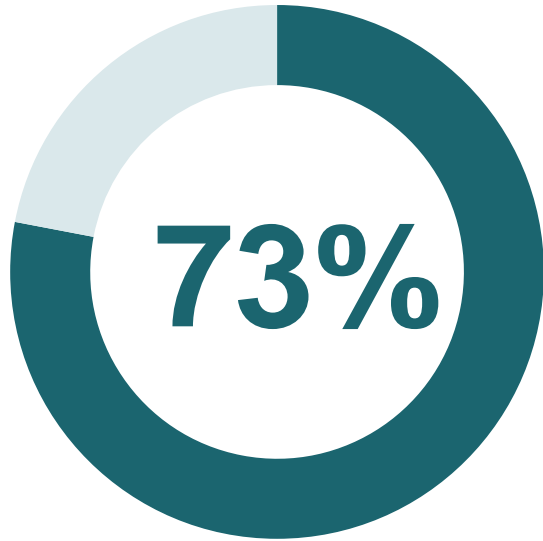
OCA has demonstrated a **strong and confirmed antifibrotic effect** in two independent histologic evaluation methodologies

**PDUFA target action date of June 22, 2023; FDA advisory committee meeting anticipated**

References: 1. Dulai PS et al. *Hepatology* 2017;65(5):1557–1565.

# Poised To Deliver OCA in NASH Upon Anticipated Approval

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**of highest potential  
Heps/GIs are within  
our existing PBC  
customer base**

## **Established U.S. field presence with broad geographic footprint**

- 55 Territory Business Managers in 7 regions with all states covered
  - Key account coverage and relationships
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## **Preparing go-to-market commercial strategy**

- Existing medical affairs team that targets top specialists
  - Engaging with all stakeholders, including payers
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## **Recognized industry leadership**

- Ongoing engagement with patients and key advocacy organizations
- Significant presence at major liver and gastroenterology congresses

# Advancing INT-787 in Lead Indication: Severe Alcohol-Associated Hepatitis

Alcohol-related liver disease as a cause of chronic liver disease is on the rise and currently the leading indication for liver transplant in the U.S., with a marked increase in patients with sAH needing liver transplant

INT-787 is a next-generation farnesoid X receptor (FXR) agonist



Phase 1 and proof-of-concept Phase 2a study underway

- Initiated the FRESH study, a Phase 2a trial evaluating the safety, tolerability, efficacy and pharmacokinetics of INT-787 in patients with sAH
  - Randomized, double-blind, dose-escalation study
  - Expected to enroll approximately 50 patients with sAH across multiple clinical sites in the U.S., UK and France
- Phase 1 trial of INT-787 demonstrated a favorable safety and tolerability profile
- As of February 2023, we have completed recruitment for our Phase 1 study



# Q4/FY 2022 Financial Highlights

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2022	2021	2022	2021
Total revenue	\$77.2M	\$68.6M	\$285.7M	\$260.7M
ex-U.S. revenue (discontinued operations)	-	-	58.1M	52.8M
Total non-GAAP net sales	\$77.2M	\$68.6M	\$343.8M	\$313.5M
GAAP operating expenses	96.1M	97.8M	353.9M	361.2M
Non-GAAP adjusted operating expenses (1)	90.9M	104.4M	355.2M	382.3M
Cost of sales	0.03M	0.4M	1.0M	1.2M
SG&A Expenses	55.4M	46.3M	176.3M	177.5M
R&D Expenses	40.7M	51.1M	176.6M	182.7M

(1) Refer to the following slides for a reconciliation of non-GAAP adjusted operating expenses to total operating expenses

	12/31/22	12/31/21
Cash, cash equivalents, restricted cash & investment debt securities available for sale	\$490.9M	\$427.8M

# Note Regarding Non-GAAP Financial Measures

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This presentation refers to non-GAAP adjusted net sales and non-GAAP adjusted operating expenses on a historical and projected basis.

For the periods presented, non-GAAP adjusted net sales include in total revenue, as calculated and presented in GAAP, the effect of one item: total revenue from discontinued operations. For the periods presented, non-GAAP adjusted operating expenses exclude from total operating expenses, as calculated and presented in accordance with GAAP, the effects of two non-cash items: stock-based compensation and depreciation and one item for discontinued operations.

These are non-GAAP financial measures and are not necessarily consistently defined across companies. Investors should consider them in addition to, but not instead of, the GAAP measures. Our management uses these measures for budgeting, operational goals, and managerial purposes. We believe that presentation of these non-GAAP measures is helpful supplemental information for investors and management regarding operating performance and trends.

For reconciliation tables, please refer to the prior slide and the next slide. For non-GAAP adjusted operating expenses, regarding future, projected periods, a quantitative reconciliation would not be available without unreasonable effort, due to the difficulty of predicting with reasonable certainty future amounts of stock-based compensation expense.

# Reconciliation of Non-GAAP Adjusted Net Sales to Total Revenue

## Reconciliation of Non-GAAP Adjusted Net Sales to Total Revenue

(Unaudited)

(In thousands)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2022	2021	2022	2021
Total revenue	\$ 77,219	\$ 68,633	\$ 285,710	\$ 260,750
Adjustment:				
ex-U.S. revenue (discontinued operations)	-	-	58,065	52,760
Non-GAAP adjusted net sales	<u>\$ 77,219</u>	<u>\$ 68,633</u>	<u>\$ 343,775</u>	<u>\$ 313,510</u>

# Reconciliation of Non-GAAP Adjusted Operating Expenses to Total Operating Expenses

## Reconciliation of Non-GAAP Adjusted Operating Expenses to Total Operating Expenses

(Unaudited)

(In thousands)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2022	2021	2022	2021
Total operating expenses	\$ 96,128	\$ 97,809	\$ 353,926	\$ 361,156
Adjustments:				
Add: ex-U.S. operating expenses (discontinued operations)	227	15,461	30,683	57,985
Less: Stock-based compensation	5,338	8,405	26,390	33,888
Depreciation	92	421	3,038	2,978
Non-GAAP adjusted operating expenses	<u>\$ 90,925</u>	<u>\$ 104,444</u>	<u>\$ 355,181</u>	<u>\$ 382,275</u>