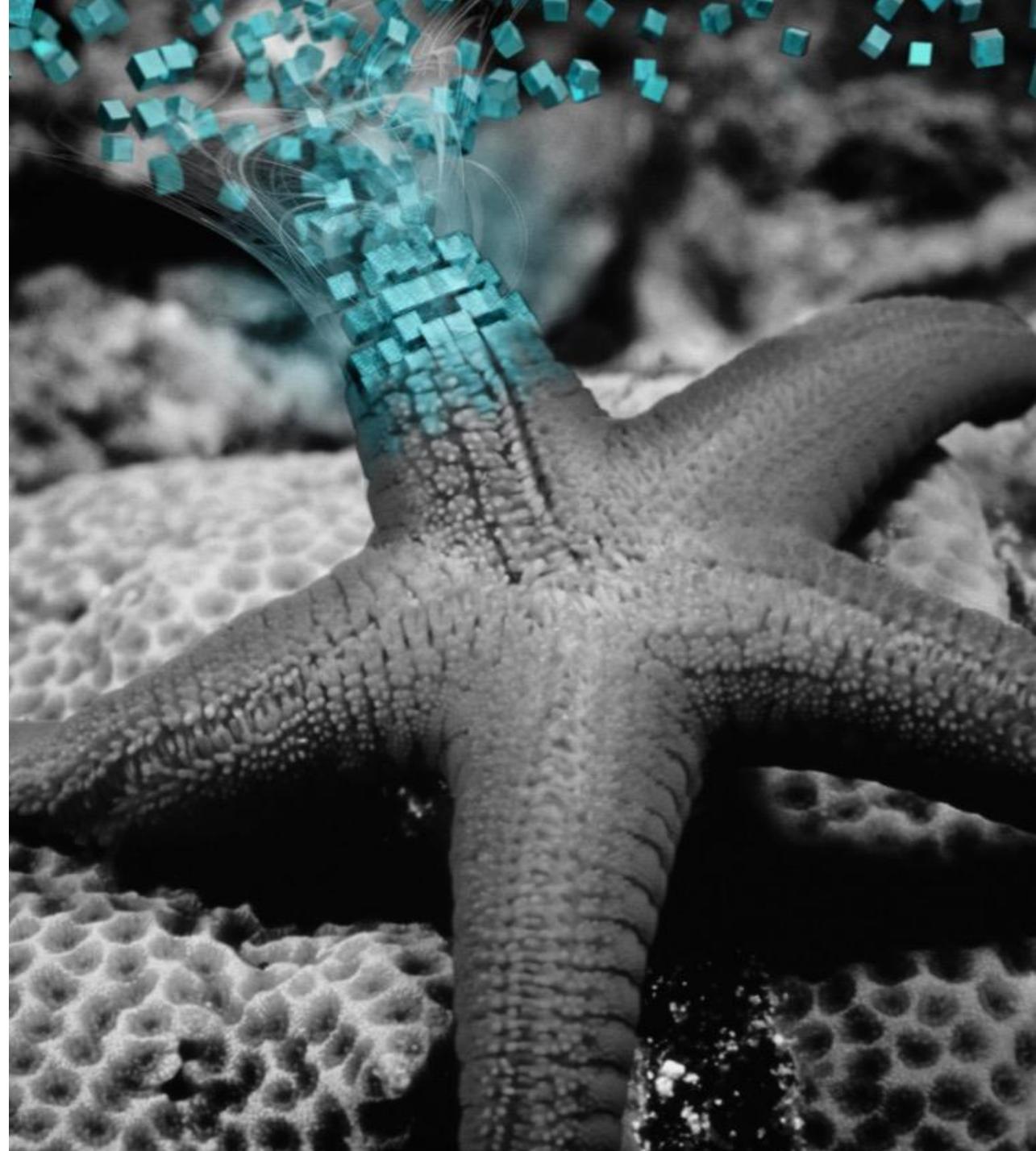


# Intercept Pharmaceuticals

Q3 2020 Earnings Call Presentation

November 9, 2020



# Cautionary Note Regarding Forward-Looking Statements and Non-GAAP Financial Measures

This presentation contains forward-looking statements, including, but not limited to, statements regarding the progress, timing and results of our clinical trials, including our clinical trials for the treatment of nonalcoholic steatohepatitis (“NASH”), the safety and efficacy of our approved product, Ocaliva (obeticholic acid or “OCA”) for primary biliary cholangitis (“PBC”), and our product candidates, including OCA for liver fibrosis due to NASH, the timing and acceptance of our regulatory filings and the potential approval of OCA for liver fibrosis due to NASH, the review of our New Drug Application for OCA for the treatment of liver fibrosis due to NASH by the U.S. Food and Drug Administration (FDA), our intent to work with the FDA to address the issues raised in the complete response letter (CRL), the potential commercial success of OCA, as well as our strategy, future operations, future financial position, future revenue, projected costs, financial guidance, prospects, plans and objectives.

These statements constitute forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. The words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “target,” “potential,” “will,” “would,” “could,” “should,” “possible,” “continue” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Readers are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date of this presentation, and we undertake no obligation to update any forward-looking statement except as required by law. These forward-looking statements are based on estimates and assumptions by our management that, although believed to be reasonable, are inherently uncertain and subject to a number of risks. The following represent some, but not necessarily all, of the factors that could cause actual results to differ materially from historical results or those anticipated or predicted by our forward-looking statements: our ability to successfully commercialize Ocaliva for PBC; our ability to maintain our regulatory approval of Ocaliva for PBC in the United States, Europe, Canada, Israel, Australia and other jurisdictions in which we have or may receive marketing authorization; our ability to timely and cost-effectively file for and obtain regulatory approval of our product candidates on an accelerated basis or at all, including OCA for liver fibrosis due to NASH following the issuance of the CRL by the FDA; any advisory committee recommendation or dispute resolution determination that our product candidates, including OCA for liver fibrosis due to NASH, should not be approved or approved only under certain conditions; any future determination that the regulatory applications and subsequent information we submit for our product candidates, including OCA for liver fibrosis due to NASH, do not contain adequate clinical or other data or meet applicable regulatory requirements for approval; conditions that may be imposed by regulatory authorities on our marketing approvals for our products and product candidates, including OCA for liver fibrosis due to NASH, such as the need for clinical outcomes data (and not just results based on achievement of a surrogate endpoint), any risk mitigation programs such as a REMS, and any related restrictions, limitations and/or warnings contained in the label of any of our products or product candidates; any potential side effects associated with Ocaliva for PBC, OCA for liver fibrosis due to NASH or our other product candidates that could delay or prevent approval, require that an approved product be taken off the market, require the inclusion of safety warnings or precautions, or otherwise limit the sale of such product or product candidate; the initiation, timing, cost, conduct, progress and results of our research and development activities, preclinical studies and clinical trials, including any issues, delays or failures in identifying patients, enrolling patients, treating patients, retaining patients, meeting specific endpoints in the jurisdictions in which we intend to seek approval or completing and timely reporting the results of our NASH or PBC clinical trials; our ability to establish and maintain relationships with, and the performance of, third-party manufacturers, contract research organizations and other vendors upon whom we are substantially dependent for, among other things, the manufacture and supply of our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and our clinical trial activities; our ability to identify, develop and successfully commercialize our products and product candidates, including our ability to successfully launch OCA for liver fibrosis due to NASH, if approved; our ability to obtain and maintain intellectual property protection for our products and product candidates, including our ability to cost-effectively file, prosecute, defend and enforce any patent claims or other intellectual property rights; the size and growth of the markets for our products and product candidates and our ability to serve those markets; the degree of market acceptance of Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH or our other product candidates among physicians, patients and healthcare payors; the availability of adequate coverage and reimbursement from governmental and private healthcare payors for our products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and our ability to obtain adequate pricing for such products; our ability to establish and maintain effective sales, marketing and distribution capabilities, either directly or through collaborations with third parties; competition from existing drugs or new drugs that become available; our ability to prevent system failures, data breaches or violations of data protection laws; costs and outcomes relating to any disputes, governmental inquiries or investigations, regulatory proceedings, legal proceedings or litigation, including any securities, intellectual property, employment, product liability or other litigation; our collaborators’ election to pursue research, development and commercialization activities; our ability to establish and maintain relationships with collaborators with development, regulatory and commercialization expertise; our need for and ability to generate or obtain additional financing; our estimates regarding future expenses, revenues and capital requirements and the accuracy thereof; our use of cash and short-term investments; our ability to acquire, license and invest in businesses, technologies, product candidates and products; our ability to attract and retain key personnel to manage our business effectively; our ability to manage the growth of our operations, infrastructure, personnel, systems and controls; our ability to obtain and maintain adequate insurance coverage; the impact of COVID-19, including any impact on our results of operations or financial position, related quarantines and government actions, delays relating to our regulatory applications, disruptions relating to our ongoing clinical trials or involving our contract research organizations, study sites or other clinical partners, disruptions relating to our supply chain or involving our third-party manufacturers, distributors or other distribution partners, facility closures or other restrictions, and the extent and duration thereof; the impact of general U.S. and foreign economic, industry, market, regulatory or political conditions, including the potential impact of Brexit; and the other risks and uncertainties identified in our periodic filings filed with the U.S. Securities and Exchange Commission, including our Annual Report on Form 10-K for the year ended December 31, 2019 and our Quarterly Report on Form 10-Q for the quarter ended June 30, 2020.

This presentation also presents non-GAAP adjusted operating expenses on a historical and projected basis. 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. Non-GAAP adjusted operating expenses is a financial measure that has not been prepared in accordance with GAAP. Accordingly, investors should consider non-GAAP adjusted operating expenses in addition to, but not as a substitute for, total operating expenses that we calculate and present in accordance with GAAP. Among other things, our management uses non-GAAP adjusted operating expenses to establish budgets and operational goals and to manage our business. Other companies may define or use this measure in different ways. We believe that the presentation of non-GAAP adjusted operating expenses provides investors and management with helpful supplemental information relating to operating performance and trends. A table reconciling non-GAAP adjusted operating expenses to total operating expenses for all historical periods presented is included in the Appendix hereto under the heading “Reconciliation of Non-GAAP Adjusted Operating Expenses to Total Operating Expenses”. A quantitative reconciliation of projected non-GAAP adjusted operating expenses to total operating expenses is not available without unreasonable effort primarily due to our inability to predict with reasonable certainty the amount of future stock-based compensation expense.

# Key Business Updates



**PBC business reported strongest quarter to date, with net sales of \$79.5 million, representing 29% growth over prior year quarter**



**Full Year 2020 guidance narrows supporting strong financial foundation**

- **Net sales now anticipated between \$310 million to \$320 million**
- **Non-GAAP adjusted operating expenses now anticipated between \$460 million to \$480 million**



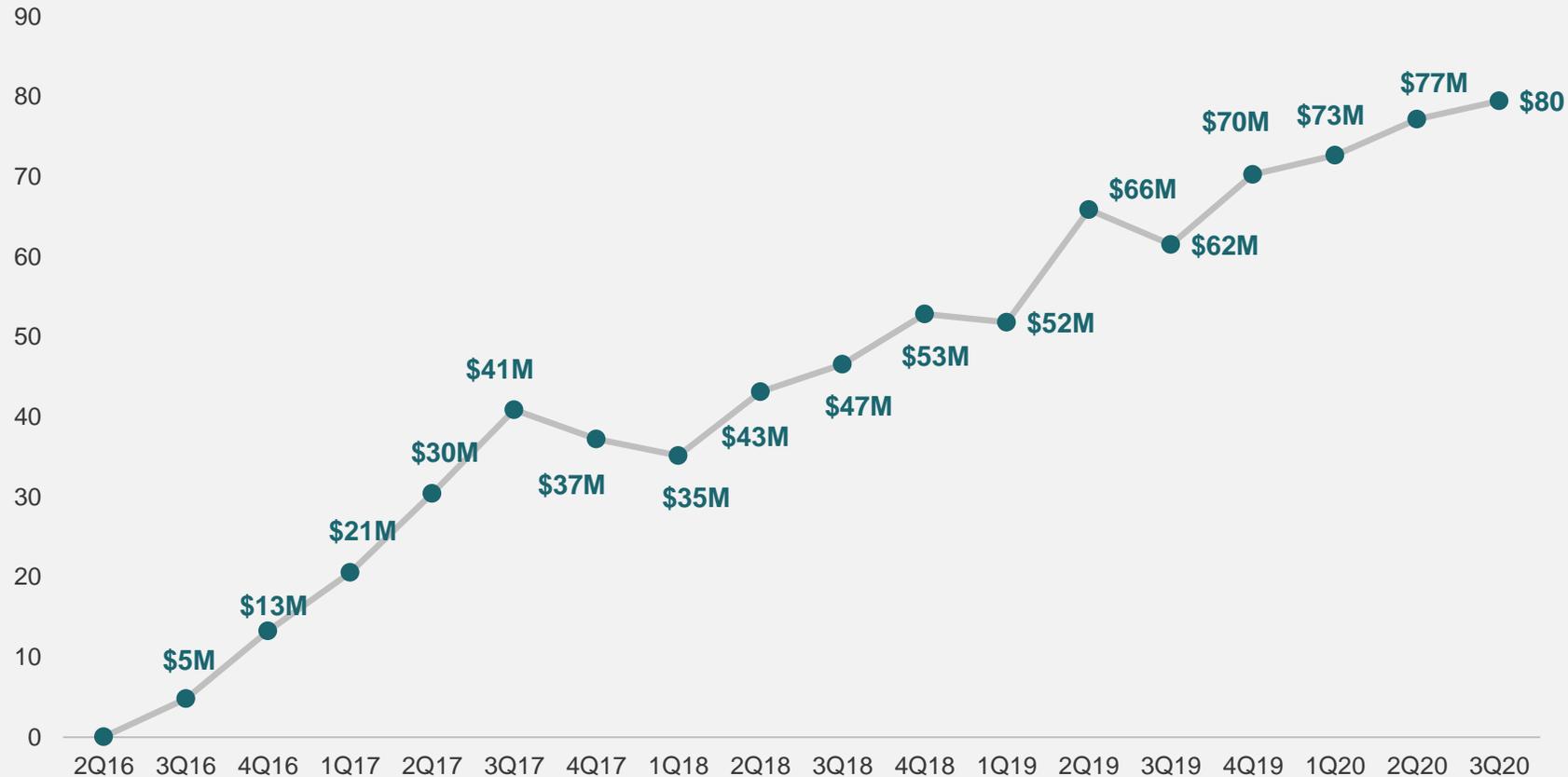
**Constructive Type A end of review meeting with FDA regarding our NDA for OCA in NASH fibrosis. Additional work underway based on feedback.**



**Phase 3 REGENERATE fully enrolled and remains ongoing to outcomes**  
**Phase 3 REVERSE fully enrolled and expected to read out by end of next year**

# Continued Strong Commercial Performance for Ocaliva in PBC

## Quarterly Worldwide Net Sales as of Q3 2020



## Q3 2020 Net Sales Overview

**\$58.6M in U.S.**  
net sales due to strong commercial performance

**\$20.9M in ex-U.S.**  
net sales driven by continued strong performance in key international markets

# Refocusing on Growth of Core PBC Business

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## Sales Growth Continues Despite Ongoing COVID Impact

- Ocaliva resilient through the pandemic, with 3Q net sales growth of 29% versus prior year quarter
- Opportunity for continued market expansion in PBC

## Executing Plan to Reduce Operating Expenses

- Right-sized organization to maximize growth of core PBC business
- Restructured US commercial and medical affairs organizations

## Refocusing on Commercial PBC Efforts

- Redeployed field teams to focus on PBC
- Team has deep experience in hepatology and GI
- Ready to execute to support our objectives for the future

# Q3 2020 Financial Highlights

## Three Months Ended September 30

	2020	2019
Total revenue	\$ 79.5M	\$ 61.9M
Ocaliva net sales – U.S.	58.6M	45.2M
Ocaliva net sales – ex-U.S.	20.9M	16.3M
Licensing revenue	--	0.4M
GAAP operating expenses	134.7M	137.5M
Non-GAAP adjusted operating expenses (1)	118.1M	123.4M
Cost of sales	1.8M	0.5M
SG&A Expenses	70.6M	76.8M
R&D Expenses	48.9M	60.2M

(1) Refer to slide 9 for a reconciliation of non-GAAP adjusted operating expenses to total operating expenses

	9/30/20	12/31/19
Cash, cash equivalents, restricted cash & investment debt securities available for sale	\$ 496.8M	\$ 657.3M

# Updated 2020 Financial Guidance

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## Updated Ocaliva Net Sales Guidance

Expect full year 2020 Ocaliva net sales of **\$310M to 320M**

## Updated Non-GAAP Adjusted Operating Expenses Guidance

Non-GAAP adjusted operating expenses range narrowed to **\$460M to 480M**

- Leveraged flexibility in commercial structure
- Postponed our NASH launch preparation activities and identified additional cost savings

# Appendix

# 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 September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Total operating expenses	\$ 134,684	\$ 137,483	\$ 420,060	\$ 403,639
Adjustments:				
Stock-based compensation	15,825	13,130	44,381	42,809
Depreciation	710	914	2,282	2,839
Non-GAAP adjusted operating expenses	<u>\$ 118,149</u>	<u>\$ 123,439</u>	<u>\$ 373,397</u>	<u>\$ 357,991</u>

# Ocaliva<sup>®</sup> (obeticholic acid) U.S. Important Safety Information

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## **WARNING: HEPATIC DECOMPENSATION AND FAILURE IN INCORRECTLY DOSED PBC PATIENTS WITH CHILD-PUGH CLASS B OR C OR DECOMPENSATED CIRRHOSIS**

- In postmarketing reports, hepatic decompensation and failure, in some cases fatal, have been reported in patients with Primary Biliary Cholangitis (PBC) with decompensated cirrhosis or Child-Pugh Class B or C hepatic impairment when OCALIVA was dosed more frequently than recommended.
- The recommended starting dosage of OCALIVA is 5 mg once weekly for patients with Child-Pugh Class B or C hepatic impairment or a prior decompensation event.

## **Indication**

OCALIVA is indicated for the treatment of primary biliary cholangitis (PBC) in combination with ursodeoxycholic acid (UDCA) in adults with an inadequate response to UDCA, or as monotherapy in adults unable to tolerate UDCA.

This indication is approved under accelerated approval based on a reduction in alkaline phosphatase (ALP). An improvement in survival or disease-related symptoms has not been established. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

## **Contraindications**

OCALIVA is contraindicated in patients with complete biliary obstruction.

## **Warnings and Precautions**

### ***Hepatic Decompensation and Failure in Incorrectly-Dosed PBC Patients with Child-Pugh Class B or C or Decompensated Cirrhosis***

In postmarketing reports, hepatic decompensation and failure, in some cases fatal, have been reported in patients with decompensated cirrhosis or Child-Pugh B or C hepatic impairment when OCALIVA was dosed more frequently than the recommended starting dosage of 5 mg once weekly. Reported cases typically occurred within 2 to 5 weeks after starting OCALIVA and were characterized by an acute increase in total bilirubin and/or ALP concentrations in association with clinical signs and symptoms of hepatic decompensation (e.g., ascites, jaundice, gastrointestinal bleeding, worsening of hepatic encephalopathy). Patients who died due to liver-related complications generally had decompensated cirrhosis prior to treatment and were started on OCALIVA 5mg once daily, which is 7-fold greater than the once-weekly starting regimen in this population.

Routinely monitor patients for progression of PBC disease, including liver-related complications, with laboratory and clinical assessments. Dosage adjustment, interruption or discontinuation may be required. Close monitoring is recommended for patients at an increased risk of hepatic decompensation. Severe intercurrent illnesses that may worsen renal function or cause dehydration (e.g., gastroenteritis), may exacerbate the risk of hepatic decompensation. Interrupt treatment with OCALIVA in patients with laboratory or clinical evidence of worsening liver function indicating risk of decompensation, and monitor the patient's liver function. Consider discontinuing OCALIVA in patients who have experienced clinically significant liver-related adverse reactions. Discontinue OCALIVA in patients who develop complete biliary obstruction.

### ***Liver-Related Adverse Reactions***

Dose-related, liver-related adverse reactions including jaundice, worsening ascites and primary biliary cholangitis flare have been observed in clinical trials, as early as one month after starting treatment with OCALIVA 10 mg once daily up to 50 mg once daily (up to 5-times the highest recommended dosage). Monitor patients during treatment with OCALIVA for elevations in liver biochemical tests and for the development of liver-related adverse reactions.

# Ocaliva<sup>®</sup> (obeticholic acid) U.S. Important Safety Information (cont.)

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## ***Severe Pruritus***

Severe pruritus was reported in 23% of patients in the OCALIVA 10 mg arm, 19% of patients in the OCALIVA titration arm, and 7% of patients in the placebo arm in a 12-month double-blind randomized controlled trial of 216 patients. Severe pruritus was defined as intense or widespread itching, interfering with activities of daily living, or causing severe sleep disturbance, or intolerable discomfort, and typically requiring medical interventions. Consider clinical evaluation of patients with new onset or worsening severe pruritus. Management strategies include the addition of bile acid resins or antihistamines, OCALIVA dosage reduction, and/or temporary interruption of OCALIVA dosing.

## ***Reduction in HDL-C***

Patients with PBC generally exhibit hyperlipidemia characterized by a significant elevation in total cholesterol primarily due to increased levels of high-density lipoprotein-cholesterol (HDL-C). Dose-dependent reductions from baseline in mean HDL-C levels were observed at 2 weeks in OCALIVA-treated patients, 20% and 9% in the 10 mg and titration arms, respectively, compared to 2% in the placebo arm. Monitor patients for changes in serum lipid levels during treatment. For patients who do not respond to OCALIVA after 1 year at the highest recommended dosage that can be tolerated (maximum of 10 mg once daily), and who experience a reduction in HDL-C, weigh the potential risks against the benefits of continuing treatment.

## ***Adverse Reactions***

The most common adverse reactions occurring in ≥5% of subjects taking OCALIVA were pruritus, fatigue, abdominal pain and discomfort, rash, oropharyngeal pain, dizziness, constipation, arthralgia, thyroid function abnormality, and eczema.

## **Drug Interactions**

### ***Bile Acid Binding Resins***

Bile acid binding resins such as cholestyramine, colestipol, or colesevelam adsorb and reduce bile acid absorption and may reduce the absorption, systemic exposure, and efficacy of OCALIVA. If taking a bile acid binding resin, take OCALIVA at least 4 hours before or 4 hours after taking the bile acid binding resin, or at as great an interval as possible.

### ***Warfarin***

The International Normalized Ratio (INR) decreased following coadministration of warfarin and OCALIVA. Monitor INR and adjust the dose of warfarin, as needed, to maintain the target INR range when coadministering OCALIVA and warfarin.

### ***CYP1A2 Substrates with Narrow Therapeutic Index***

Obeticholic acid, the active ingredient in OCALIVA, may increase the exposure to concomitant drugs that are CYP1A2 substrates. Therapeutic monitoring of CYP1A2 substrates with a narrow therapeutic index (e.g. theophylline and tizanidine) is recommended when coadministered with OCALIVA.

### ***Inhibitors of Bile Salt Efflux Pump***

Avoid concomitant use of inhibitors of the bile salt efflux pump (BSEP) such as cyclosporine. Concomitant medications that inhibit canalicular membrane bile acid transporters such as the BSEP may exacerbate accumulation of conjugated bile salts including taurine conjugate of obeticholic acid in the liver and result in clinical symptoms. If concomitant use is deemed necessary, monitor serum transaminases and bilirubin.

Please see **Full Prescribing Information, including Boxed WARNING** and **Medication Guide** for OCALIVA.

**To report SUSPECTED ADVERSE REACTIONS, contact Intercept Pharmaceuticals, Inc. at 1-844-782-ICPT or FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).**