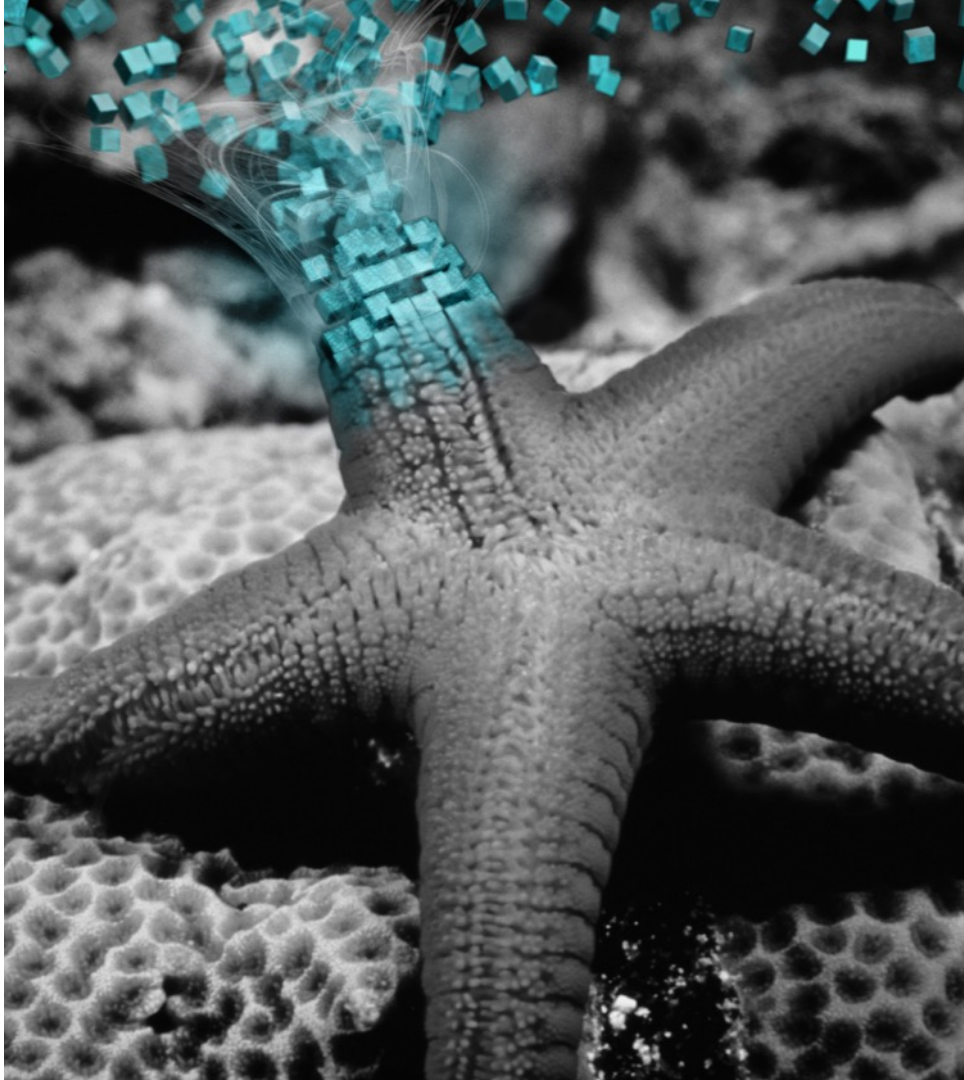
# Intercept Pharmaceuticals

Q4 & FY 2019 Earnings Call Presentation

February 25, 2020

Intercept



# Cautionary Note Regarding Forward-Looking Statements

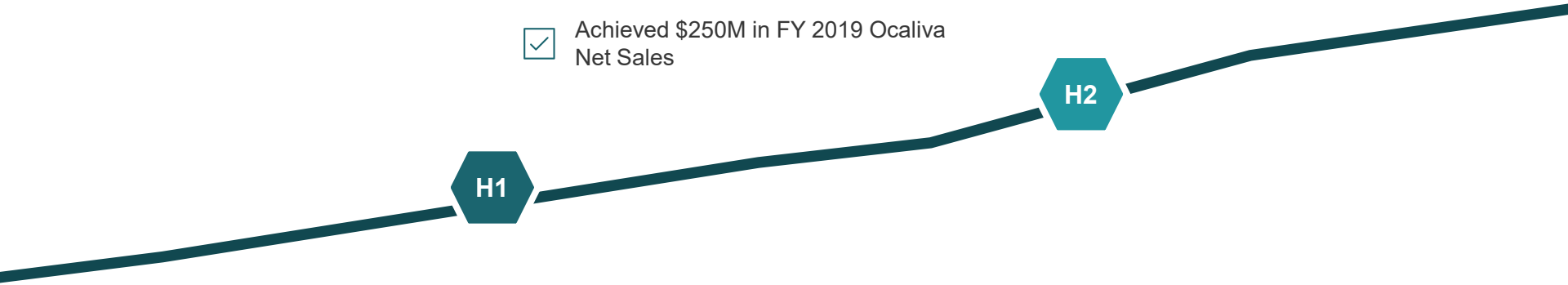
This presentation contains forward-looking statements, including, but not limited to, statements regarding the progress, timing and results of Intercept's clinical trials, including its clinical trials for the treatment of nonalcoholic steatohepatitis ("NASH"), the safety and efficacy of Intercept's approved product, Ocaliva (obeticholic acid or "OCA") for primary biliary cholangitis ("PBC"), and Intercept's product candidates, including OCA for liver fibrosis due to NASH, the timing and acceptance of Intercept's regulatory filings and potential approval of OCA for liver fibrosis due to NASH or any other indications in addition to PBC, the timing and potential commercial success of OCA and any other product candidates Intercept may develop and Intercept's 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 Intercept undertakes no obligation to update any forward-looking statement except as required by law. These forward-looking statements are based on estimates and assumptions by Intercept's 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 Intercept's forward-looking statements: Intercept's ability to successfully commercialize Ocaliva for PBC; Intercept's ability to maintain its regulatory approval of Ocaliva for PBC in the United States, Europe, Canada, Israel, Australia and other jurisdictions in which it has or may receive marketing authorization; the initiation, timing, cost, conduct, progress and results of Intercept's 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 it intends to seek approval or completing and timely reporting the results of its NASH or PBC clinical trials; Intercept's ability to timely and cost-effectively file for and obtain regulatory approval of its product candidates, including the regulatory approval of its New Drug Application for liver fibrosis due to NASH; any advisory committee recommendation that Intercept's product candidates, including OCA for liver fibrosis due to NASH, should not be approved or approved only under certain conditions; any determination that the regulatory applications and subsequent information Intercept submits for its 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 Intercept's marketing approvals for its products and product candidates, such as the need for clinical outcomes data (and not just results based on achievement of a surrogate endpoint), and any related restrictions, limitations and/or warnings contained in the label of any of its products or product candidates; any potential side effects associated with Ocaliva for PBC, OCA for liver fibrosis due to NASH or Intercept's 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; Intercept's ability to establish and maintain relationships with, and the performance of, third-party manufacturers, contract research organizations and other vendors upon whom it is substantially dependent for, among other things, the manufacture and supply of its products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and its clinical trial activities; Intercept's ability to identify, develop and successfully commercialize its products and product candidates, including its ability to timely and successfully launch OCA for liver fibrosis due to NASH, if approved; Intercept's ability to obtain and maintain intellectual property protection for its products and product candidates, including its 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 Intercept's products and product candidates and its 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 Intercept's other product candidates among physicians, patients and healthcare payors; the availability of adequate coverage and reimbursement from governmental and private healthcare payors for Intercept's products, including Ocaliva for PBC and, if approved, OCA for liver fibrosis due to NASH, and its ability to obtain adequate pricing for such products; Intercept's 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; Intercept's ability to prevent system failures, data breaches or violations of data protection laws; costs and outcomes relating to any disputes, governmental inquiries or investigations, legal proceedings or litigation, including any securities, intellectual property, employment, product liability or other litigation; Intercept's collaborators' election to pursue research, development and commercialization activities; Intercept's ability to establish and maintain relationships with collaborators with development, regulatory and commercialization expertise; Intercept's need for and ability to generate or obtain additional financing; Intercept's estimates regarding future expenses, revenues and capital requirements and the accuracy thereof; Intercept's use of cash and short-term investments; Intercept's ability to acquire, license and invest in businesses, technologies, product candidates and products; Intercept's ability to attract and retain key personnel to manage its business effectively; Intercept's ability to manage the growth of its operations, infrastructure, personnel, systems and controls; Intercept's ability to obtain and maintain adequate insurance coverage; 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 Intercept's periodic filings filed with the U.S. Securities and Exchange Commission, including Intercept's Annual Report on Form 10-K for the year ended December 31, 2018.

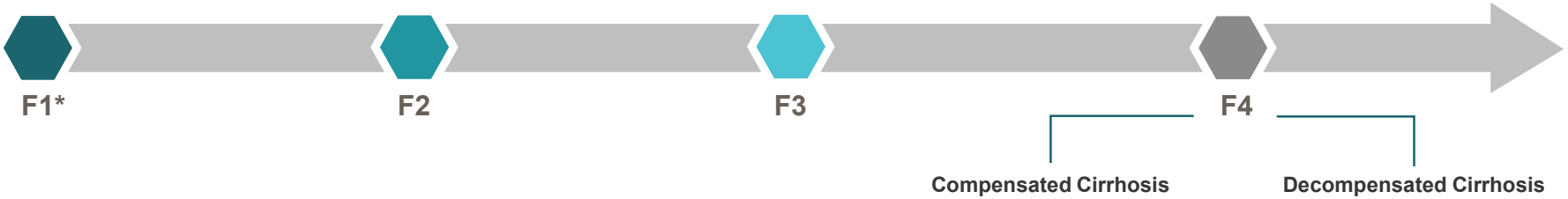
# Significant Milestones Achieved in 2019

- Announced acquisition of license to U.S. rights to bezafibrate
- Positive topline results from interim analysis of pivotal Phase 3 REGENERATE study
- Opening Plenary presentation of REGENERATE topline data at EASL
- Completed $470 million financing
- NDA for OCA for liver fibrosis due to NASH accepted by FDA with Priority Review
- Presented NIT data from REGENERATE during AASLD
- REGENERATE results published in *The Lancet*
- MAA submitted

- Achieved $250M in FY 2019 Ocaliva Net Sales



# Comprehensive Development Program Focused on Advanced Fibrosis




## REGENERATE

- NASH patients with F2, F3 and high-risk F1 fibrosis*
- Interim analysis (931 F2/F3 patients)
- 2,480 patients enrolled for outcomes

## REVERSE

- NASH patients with compensated cirrhosis
- 919 patients enrolled
- 18-month treatment period

**Two ongoing global NASH Phase 3 studies to evaluate OCA**

*Exploratory group of NASH patients with stage 1 liver fibrosis with comorbid risk factors (defined as diabetes, obesity or active liver inflammation (ALT >1.5X ULN)) have been enrolled, but not included in the primary endpoint analyses.

# Significant Milestones Achieved in 2019… And Near Term Milestones Ahead

- ☑ Announced acquisition of license to U.S. rights to bezafibrate
- ☑ Positive topline results from interim analysis of pivotal Phase 3 REGENERATE study
- ☑ Opening Plenary presentation of REGENERATE topline data at EASL
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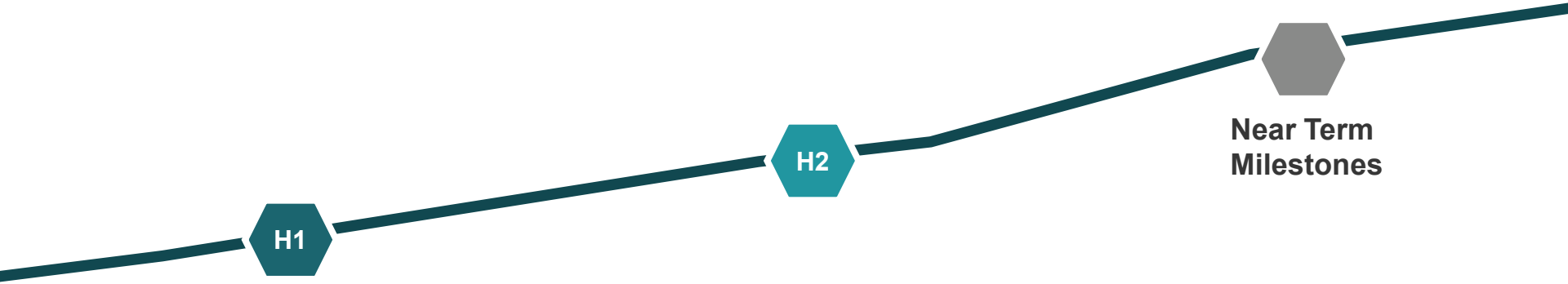
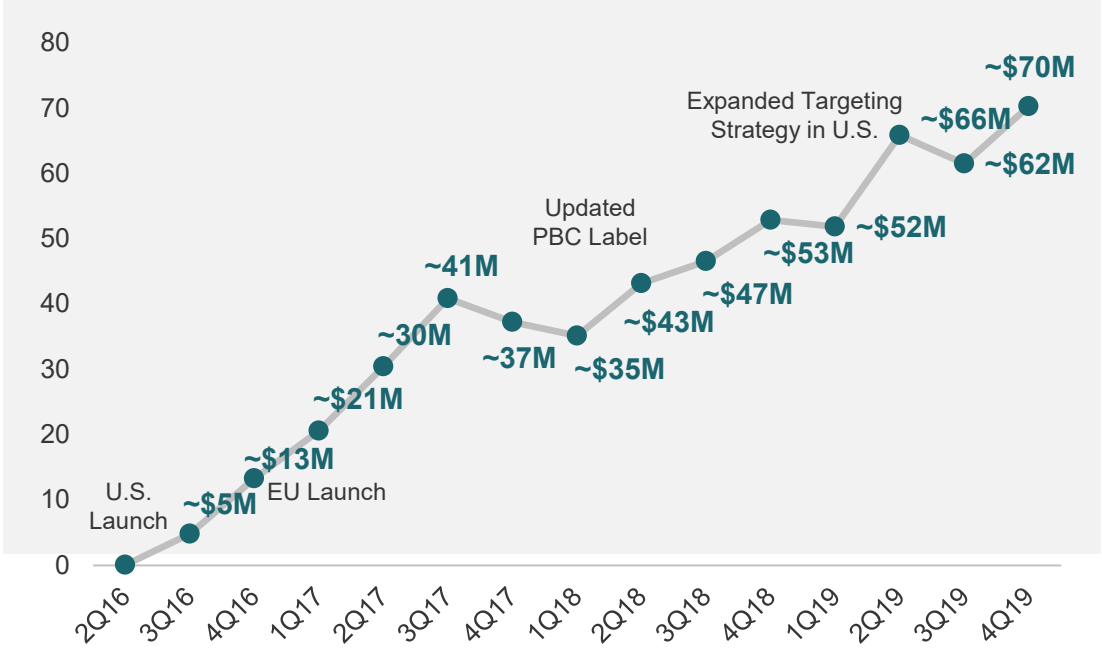
- ☑ NDA for OCA for liver fibrosis due to NASH accepted by FDA with Priority Review
- ☑ Presented NIT data from REGENERATE during AASLD
- ☑ REGENERATE results published in *The Lancet*
- ☑ MAA submitted
- ☑ Achieved $250M in FY 2019 Ocaliva Net Sales
- ☑ REVERSE completed enrollment
- ☐ Strong presence at upcoming medical meetings, including EASL and DDW
- ☐ Advisory committee meeting tentatively scheduled for April 22
- ☐ Anticipated U.S. approval – PDUFA date: June 26
- ☐ Anticipated successful commercial launch



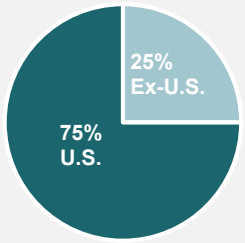
# Continued Strong Commercial Performance for Ocaliva in PBC

## Quarterly Worldwide Net Sales as of 4Q 2019

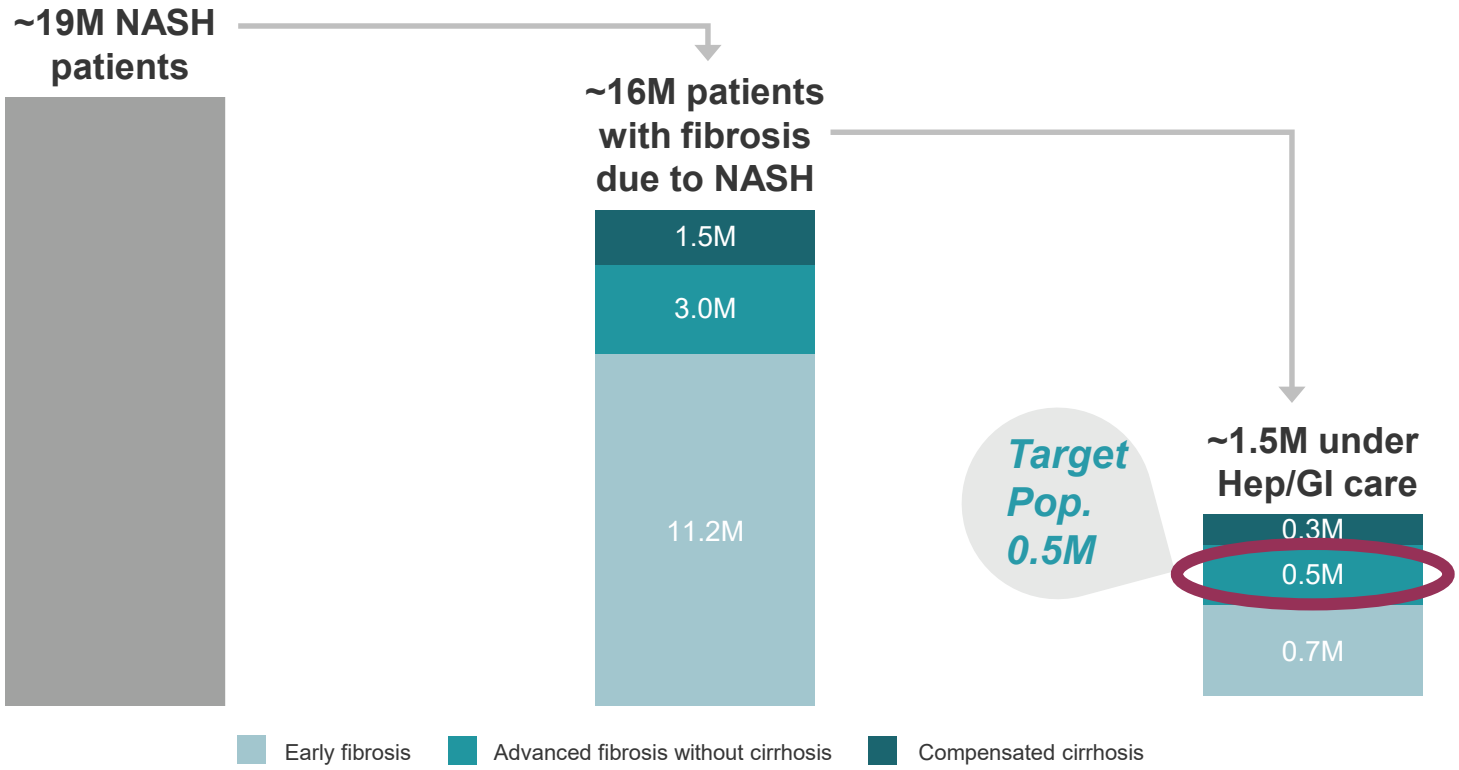



## FY 2019 Worldwide Ocaliva Net Sales

Reported **~$250M globally,** representing 40% growth over the prior year

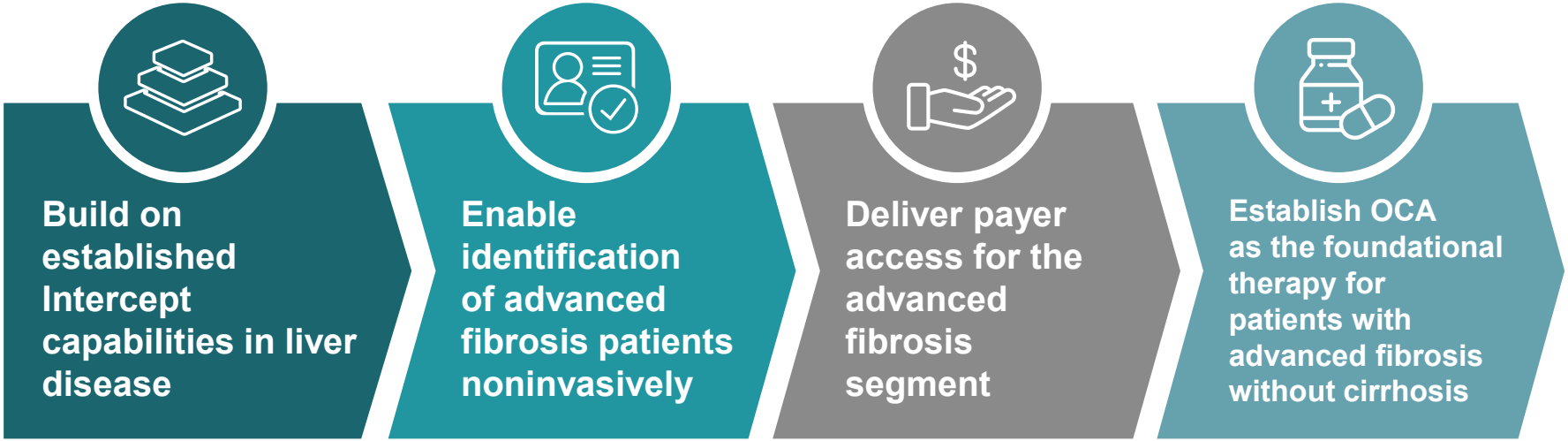


# Our Target Population at Launch Represents a Subset of the Larger NASH Patient Population




**Reference**: Estimates based upon Estes et al. *Hepatology* 2018;67(1)123-133; Adapted learnings from multiple studies and triangulated with medical claims & laboratory report data

# We Have a Focused NASH Launch Plan Based on Four Key Pillars



# Liver Specialists Use Non-Invasive Tests (NITs) to Assess NASH Patients with Fibrosis

**Significant majority of specialists surveyed believe they can reliably assess advanced fibrosis using NITs without conducting a liver biopsy[1]**

## Simple Scores

- NFS (NAFLD fibrosis score)[2]
- FIB-4 (Fibrosis-4)[2]
- APRI (AST / platelet ratio index)[3]

## Proprietary Serum Tests

- FibroSURE® (FibroTest® ex-U.S.)[2]
- ELF™ (Enhanced Liver Fibrosis)[2]

## Imaging Techniques

- VCTE (e.g., FibroScan®)[2]
- MRE (magnetic resonance elastography)[4]

ELF™ is a trademark of Siemens Healthineers and is not commercially available in the U.S., but is used widely outside of the U.S.
FibroScan® is a registered trademark of EchoSens™, Paris
FibroTest® is a registered trademark of BioPredictive S.A.S, Paris; FibroSURE® is distributed by LabCorp in the U.S.
List of NITs provided on slide is not exhaustive, and NIT research and learning continues to evolve

**References: 1.** Internal Market Research on File (Double-Blinded), October 2019, n=115 U.S. Hepatologists/Gastroenterologists. **2.** Alkhouri N, et al. Gastroenterology and Hepatology. 2012;8(10):661–668. **3.** Atay, K. Biomedical Research. 2017;28(2):565–570. **4.** Chalasani N, et al. Hepatology. 2018;67(1):328–357

# The Stage is Set for Intercept to Leverage its Unique Capabilities and Deliver a Strong Launch for Patients with Advanced Fibrosis Due to NASH

**Our target launch population, those with advanced fibrosis due to NASH under the care of a specialist, represents a significant unmet need today**

**Specialists can identify and manage patients with advanced fibrosis noninvasively**

**Payers recognize the urgent need to treat advanced fibrosis**

**OCA is well positioned to become the potential treatment of choice in patients with advanced fibrosis**

**References:** Synthesis of Internal Market Research on File (Double-Blinded), 2018-2019

# Q4 & FY 2019 Financial Highlights

| | Three Months Ended December 31, | | Twelve Months Ended December 31, | |
|---|---|---|---|---|
| | 2019 | 2018 | 2019 | 2018 |
| Total revenue | $ 71.5M | $ 53.3M | $ 252.0M | $ 179.8M |
| Ocaliva net sales – U.S. | 53.5M | 41.1M | 187.5M | 140.8M |
| Ocaliva net sales – ex-U.S. | 16.8M | 11.8M | 62.1M | 37.0M |
| Licensing revenue | 1.2M | 0.4M | 2.4M | 2.0M |
| GAAP operating expenses | 160.8M | 135.3M | 564.4M | 465.3M |
| Non-GAAP adjusted operating expenses | 145.4M | 122.7M | 499.4M | 410.8M |
| Cost of sales | 2.5M | 1.0M | 4.2M | 2.5M |
| SG&A Expenses | 93.7M | 71.0M | 317.4M | 255.5M |
| R&D Expenses | 64.6M | 63.3M | 242.8M | 207.3M |

| | 12/31/19 | 12/31/18 |
|---|---|---|
| Cash, cash equivalents, restricted cash & investment debt securities | $ 657.4M | $ 436.2M |

# Appendix

# Non-GAAP Reconciliation

**Reconciliation of Non-GAAP Adjusted Operating Expenses to Total Operating Expenses**
*(Unaudited)*
*(In thousands)*

| | Three Months Ended December 31, | | Year Ended December 31, | |
|---|---|---|---|---|
| | **2019** | **2018** | **2019** | **2018** |
| Total operating expenses | $ 160,790 | $ 135,251 | $ 564,429 | $ 465,294 |
| Adjustments: | | | | |
| Stock-based compensation | 13,173 | 11,499 | 55,982 | 49,914 |
| Depreciation and non-cash operating lease cost | 2,238 | 1,031 | 9,051 | 4,582 |
| Non-GAAP adjusted operating expenses | $ 145,379 | $ 122,721 | $ 499,396 | $ 410,798 |

# Ocaliva® (obeticholic acid) U.S. Important Safety Information

**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® (obeticholic acid) U.S. Important Safety Information (cont.)

***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.***

Intercept

# About the REGENERATE Study

REGENERATE is a Phase 3, randomized, double-blind, placebo-controlled, multicenter study assessing the safety and efficacy of obeticholic acid (OCA) on clinical outcomes in patients with liver fibrosis due to NASH. A pre-specified 18-month interim analysis was conducted to assess the effect of OCA on liver histology comparing month 18 biopsies with baseline. The intent-to-treat population for the interim analysis included 931 patients with stage 2 and 3 fibrosis (placebo, n=311; OCA 10 mg, n=312; OCA 25 mg, n=308). REGENERATE has completed target enrollment for the clinical outcomes cohort, with 2,480 adult NASH patients randomized at 339 qualified centers worldwide, and will continue through clinical outcomes for verification and description of clinical benefit. The end-of-study analysis will evaluate the effect of OCA on all-cause mortality and liver-related clinical outcomes, as well as its long-term safety.

The safety population of the interim analysis included 1,968 randomized patients who received at least one dose of investigational product (OCA or placebo). Adverse events were generally mild to moderate in severity and the most common were consistent with the known profile of OCA. The frequency of serious adverse events was similar across treatment arms (11% in placebo, 11% in OCA 10 mg and 14% in OCA 25 mg). The most common adverse event reported was dose-related pruritus (placebo, 19%; OCA 10 mg, 28%; OCA 25 mg, 51%). The large majority of pruritus events were mild to moderate, with severe pruritus occurring in a small number of patients.