Hope for Children with Orphan Liver Diseases Through Bile Acid Modulation

Jefferies Healthcare Conference June 4, 2019
This presentation includes forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, known as the PSLRA. Forward-looking statements include, among other things, statements, other than historical facts, regarding: the plans for, or progress, scope, cost, duration or results of, clinical trials and nonclinical studies of odevixibat, elobixibat, A3384 or any of our other product candidates or programs, such as the target indication(s) for development or approval, the size, design, population, conduct, cost, objective or endpoints of any clinical trial, or the timing for initiation or completion of or availability of results from any clinical trial (including our Phase 3 clinical trial of odevixibat in patients with progressive familial Intrahepatic cholestasis (PFIC), our planned pivotal trial of odevixibat in biliary atresia or our planned Phase 2 clinical trial of elobixibat in NASH), for submission of any regulatory filing, or for meeting with regulatory authorities; the timing of and our ability to obtain and maintain regulatory approval of any of our product candidates and any related restrictions, limitations, or warnings in the label of any approved product candidates; the size of the PFIC population, the biliary atresia population or any other disease population for indications that may be targeted by Albireo; the potential benefits or competitive position of odevixibat, elobixibat or any other Albireo product candidate or program or the commercial opportunity in any target indication; the potential benefits of a rare pediatric disease designation, the potential benefits of a fast track designation, the potential benefits of orphan drug designation, the pricing of odevixibat if approved; any action by, or decision of, EA Pharma concerning elobixibat or our business relationship; the duration of our cash runway; our future operations, financial position, revenues, costs, expenses, uses of cash, capital requirements or our need for additional financing; or our strategies, prospects, beliefs, intentions, plans, expectations, forecasts or objectives. Words such as "anticipates," "believes," "plans," "expects," "projects," "future," "intends," "may," "will," "should," "could," "estimates," "predicts," "potential," "continue," "guidance," and similar expressions sometimes identify forward-looking statements. Any forward-looking statement involves known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to differ materially from those expressed or implied by such forward-looking statement, and, therefore, investors are cautioned not to place undue reliance on any forward-looking statement. These factors include, but are not limited to: the designs, endpoints, sizes and durations for trials that will be required to support approval of odevixibat to treat patients with PFIC or any other orphan pediatric liver disease; whether favorable findings from clinical trials of odevixibat to date, including findings in patients with diseases other than PFIC, will be predictive of results from the Phase 3 PFIC clinical program for odevixibat in patients with PFIC; whether either or both of the FDA and EMA will determine that the primary endpoint and duration of the double-blind Phase 3 trial in patients with PFIC is sufficient, even if such primary endpoint is met with statistical significance, to support approval of odevixibat in the United States or the European Union, to treat PFIC, a symptom of PFIC, a specific PFIC subtype(s) or otherwise; the outcome and interpretation by regulatory authorities of the ongoing third-party study supported by us pooling and analyzing long-term PFIC patient data; the timing for initiation or completion of, or for availability of data from, the Phase 3 PFIC clinical program for odevixibat, and the outcomes of the program; and delays or other challenges in the recruitment of patients for the double-blind Phase 3 trial of odevixibat; whether odevixibat will meet the criteria to receive a pediatric priority review voucher when applicable; the competitive environment and commercial opportunity for a potential treatment for PFIC or other orphan pediatric cholestatic liver diseases; the medical benefit that may be derived from odevixibat, elobixibat, A3384 or any of our other product candidates; the extent to which one or both of our agreements for elobixibat with EA Pharma and HCR generate future nondilutive income; the significant control or influence that EA Pharma has over the commercialization of elobixibat in Japan and the development and commercialization of elobixibat in EA Pharma's other licensed territories; our ability to protect and expand our intellectual property; whether findings from nonclinical studies and clinical trials of IBAT inhibitors will be predictive of future clinical success for odevixibat or an Albireo bile acid modulator in the treatment of nonalcoholic steatohepatitis (NASH); and the timing and success of submission, acceptance and approval of regulatory filings. These and other risks and uncertainties that we face are described in our most recent Annual Report on Form 10-K and in other filings that we make or have made with the Securities and Exchange Commission. In addition, market and industry statistics contained in this presentation are based on information available to us that we believe to be reliable but have not independently verified.

All forward-looking statements speak only as of the date this presentation is made and should not be relied upon as representing our views as of any date after this presentation is made. We specifically disclaim any obligation to update any forward-looking statement, except as required by applicable law. "Albireo" is a trademark of Albireo AB. All other trademarks, service marks, service marks, trade names, logos and brand names identified in this presentation are the properties of their respective owners.
Albireo: Innovative Science + Deep Pipeline + Well Capitalized

**STRONG BASIC SCIENCE**
- Leader in development of bile acid modulators
- AstraZeneca spinout 2008

**ORPHAN PEDIATRIC LIVER LEAD ASSET**
- Odevixibat (IBATi) orphan designs, PRIME, PIP, fast track and PRV eligible
- Elohexibat (IBATi) approved for chronic constipation in Japan
- NASH and bile acid malabsorption programs

**SOLID FINANCIAL POSITION**
- NASDAQ: Listed as ALBO as of November 2016, 12M outstanding shares
- $150.3M cash and cash equivalents as of March 31, 2019
A Robust Pipeline Targeting Liver and GI Diseases/Disorders

**Planned Independent Commercialization**

<table>
<thead>
<tr>
<th>Odevixibat</th>
<th>PFIC</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pediatric Liver Diseases</td>
<td>Biliary Atresia</td>
</tr>
<tr>
<td></td>
<td>Alagille Syndrome</td>
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<tr>
<td></td>
<td>Other Cholestatic</td>
</tr>
</tbody>
</table>

**Planned Partner Commercialization**

<table>
<thead>
<tr>
<th>Elobixibat</th>
<th>Chronic Constipation</th>
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<tbody>
<tr>
<td>Elobixibat</td>
<td>NASH</td>
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<tr>
<td>Bile Acid Modulators</td>
<td>NASH</td>
</tr>
<tr>
<td>A3384</td>
<td>Bile Acid Malabsorption</td>
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</tbody>
</table>

Approved in Japan/Partnered with EA Pharma
Delivering on Our Plan as a Public Company

**Patients**

- Ph3 Elobixibat Japan Ph3 Results
- Ph2 Odevixibat Results
- Elobixibat Japan Approved
- Ph3 Odevixibat PFIC Pivotal Start

**Stakeholders**

- NASDAQ Listing $30M
- Equity Raise $50M
- Equity Raise $100M
- Royalty Monetization $45M
- PRV Eligibility Odevixibat
- Elobixibat Approval Milestone Payment $11M

**Timeline**

- 2016
  - Elobixibat Milestone Payment $8M
- 2017
  - Legacy Asset Sale $4.5M
- 2018
  - Ph3 Odevixibat PFIC Target Site Initiation Completed
- 2019
  - Elobixibat Approval Milestone Payment $11M
### Multiple Planned Milestones

<table>
<thead>
<tr>
<th>Milestones</th>
<th>1H'19</th>
<th>2H'19</th>
<th>1H'20</th>
<th>2H'20</th>
<th>1H'21</th>
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<tr>
<td><strong>Odevixibat</strong></td>
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<td>Initiate biliary atresia pivotal program</td>
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<tr>
<td>PFIC PEDFIC 1: Phase 3 topline data readout</td>
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<td>2H'19</td>
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<td>PFIC PEDFIC 2 rollover cohort</td>
<td></td>
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<td>'19/'20</td>
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<tr>
<td>PFIC PEDFIC 2 expanded cohort</td>
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<tr>
<td>Additional pediatric orphan liver diseases</td>
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<tr>
<td>PFIC approval and launch</td>
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<td><strong>NASH/PIPELINE</strong></td>
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<tr>
<td>Elobixibat NASH: Initiate Phase 2 trial</td>
<td>2Q'19</td>
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<tr>
<td>Elobixibat NASH: Phase 2 trial topline readout</td>
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<tr>
<td>Novel bile acid modulators</td>
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<tr>
<td>A3384: Initiate bile acid malabsorption trial*</td>
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*Gated on issuance of pending U.S. patent

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Odevixibat: Multi-Disease Development Approach
Many Diseases with Cholestasis of the Liver

Progressive Familial Intrahepatic Cholestatic (PFIC) & “Benign” Recurrent Intrahepatic Cholestasis (BRIC)

Intrahepatic Cholestasis of Pregnancy

Primary Biliary Cholangitis

Cystic Fibrosis-Associated Liver Disease

Drug-Induced Cholestasis

Malignancy of Bile Ducts

AIDS Cholangiopathy

IG4-associated cholangitis

Biliary Strictures

Low Phospholipid-Associated Cholestasis

The diseases above are cholestatic liver disease or may cause cholestasis (EASL Guidelines, J. Hepatol. 2009). Whether an IBATi could treat the cholestasis in these diseases is unknown.
What Are Our Potential Target Indications?

~30,000-40,000* is the estimated prevalence in the U.S. and EU only...with no approved pharmacological treatment

<table>
<thead>
<tr>
<th>Condition</th>
<th>Estimated Prevalence</th>
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<tbody>
<tr>
<td>Alagille</td>
<td>3-5K</td>
</tr>
<tr>
<td>PFIC &amp; BRIC</td>
<td>8-10K</td>
</tr>
<tr>
<td>Pediatric PSC</td>
<td>8-10K</td>
</tr>
<tr>
<td>Biliary Atresia</td>
<td>15-20K</td>
</tr>
</tbody>
</table>

*Derived from literature, primary market research and modeling. Forecast estimates do not include other regional opportunities, such as Saudi Arabia, Turkey, Asia, LATAM.
“There’s no way of mentally grasping PFIC. It’s like walking into a tornado.”
- Brooke Ramirez, Mother to Trinity (PFIC Patient)
### What Is PFIC?

<table>
<thead>
<tr>
<th>Presentation</th>
<th>Genetic Disorder</th>
<th>Disease Progression</th>
<th>Estimated Median Survival</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>AGE ~1-2</strong></td>
<td>MULTIPLE GENES, SIMILAR SYMPTOMS</td>
<td>INFLAMMATION, FIBROSIS, CIRRHOSIS, DEATH</td>
<td>APPROXIMATELY 15 YEARS*</td>
</tr>
<tr>
<td>Cholestatic/Pruritic</td>
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</tr>
</tbody>
</table>

### What is BRIC?

**Benign recurrent intrahepatic cholestasis (BRIC)**

- ✓ Same genes affected as PFIC, manifests the same symptoms, particularly severe pruritis
- ✗ Episodic with a slower disease onset and progression, estimated median survival ~30 years without transplant*

*Estimates derived from secondary references and primary market research, and may vary greatly based on patient type.
Inadequate Treatment Options for PFIC

**Off-label medications seeking symptomatic relief**
- UDCA, rifampicin, cholestyramine, etc.

**Liver transplantation**
- Limited timely organ availability
- Morbidity and disease recurrence
- Cost/expense

**PEBD surgery (partial external biliary diversion)**
- ~25% treatment failure rate\(^1\)
- Requires undesirable external stoma bag
- Challenging identification of suitable candidates

Odevixibat: A Profile Suitable for Pediatric Use

- Once Daily
- Oral Small Molecule
- Minimal Systemic Exposure
- Tolerability Profile

* also known as apical sodium-dependent bile acid transporter (ASBT)
NAPPED: Natural Course and Prognosis of PFIC and Effect of Biliary Diversion*

**NAPPED** Natural Liver Survival (NLS) PFIC2

- sBA reduced to ≥118 μmol/L; 95%** NLS at 15 yrs
- sBA reduced by >70%; 95%** NLS at 15 yrs
- Improved NLS did not require bile acid normalization

*Van Wessel, EASL 2019

**Would be 100%, but one patient died due to complications of multiple PEBD surgeries.
Odevixibat Ph.2 - Primary Efficacy Endpoint
Mean Reduction in Serum Bile Acids

Phase 2 trial was an open-label, dose-finding study of PFIC, biliary atresia, Alagille syndrome, intrahepatic cholestasis patients for four weeks. Primary endpoints: TESAEs and serum bile acid change
N=24 (20 unique + 4 retreated) in five cohorts
*Excludes PFIC patient with no BSEP function.
**Excludes 17-year-old PFIC patient with low baseline sBA.
Neither would meet inclusion criteria for planned Phase 3 trial.

Excludes PFIC patient with no BSEP function.
Excludes 17-year-old PFIC patient with low baseline sBA.
Neither would meet inclusion criteria for planned Phase 3 trial.
Serum Bile Acids in Alagille Syndrome and Biliary Atresia Patients

**A4250 Ph.2: sBA Reduction ALGS Pts.***

- Patient 1: -92%
- Patient 2: -39%
- Patient 3: -52%
- Patient 4: -57%
- Patient 5: -41%
- Patient 6: -57%

Baseline (uM): 260, 116, 338, 26, 121, 564

* A4250, 10-200 ug/kg, 4 weeks of treatment

**A4250 Ph.2: sBA Reduction BA Pts.*

- Patient 1: -58%
- Patient 2: -58%
- Patient 3: -70%

Baseline (uM): 43 µM, 136 µM, 132 µM

* A4250 dose 30 ug/kg, 4 weeks of treatment

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Statistical Correlation Between Reductions of Serum Bile Acids and Pruritus Reinforces Link

n<24 reflects missing scores. All tools based on 0–10 scales except Whittington tool (0–4 scale).

\( ^a\text{p}=0.008, r=-0.54, n=23. \)
\( ^b\text{p}=0.004, r=-0.58, n=23. \)
\( ^c\text{p}=0.006, r=-0.57, n=22. \)
\( ^d\text{p}=0.005, r=-0.57, n=22. \)
- Odevixibat was well tolerated overall; all patients completed treatment
- No AEs related to treatment during 4-week treatment period
  - Most common AEs: pyrexia, ear infections (12.5%)
- No SAEs designated as treatment related (2 deemed unrelated)
- No evidence of diarrhea during 4-week treatment period
- Decision made not to dose escalate above 200 µg/kg
  - Some transaminase elevations at 200 µg/kg
### PEDFIC 1: Phase 3 PFIC Program Summary

**Double-Blind, Randomized, Placebo-Controlled, Phase 3 Study to Demonstrate Efficacy and Safety of Odevixibat in Children with Progressive Familial Intrahepatic Cholestasis**

| Key Inclusion Criteria | Diagnosis of PFIC1 or 2 confirmed by genotyping  
Serum bile acids ≥100 µmol/L  
Pruritus ≥2 on 5-point scale |
<table>
<thead>
<tr>
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<tbody>
<tr>
<td>Size (n)</td>
<td>~60 (20 per group)</td>
</tr>
<tr>
<td>Dosage (µg/kg/day)</td>
<td>40, 120</td>
</tr>
<tr>
<td>Treatment Duration</td>
<td>24 weeks</td>
</tr>
<tr>
<td>Primary Endpoint – FDA</td>
<td>Assessment of change in pruritus</td>
</tr>
<tr>
<td>Primary Endpoint – EMA</td>
<td>Serum bile acid responder rate (reach ≤70 µmol/L or a reduction of 70%)</td>
</tr>
<tr>
<td>Follow-Up – PEDFIC 2</td>
<td>Opportunity to enroll in long-term extension study</td>
</tr>
</tbody>
</table>

In addition to the Phase 3 program, support provided for NAPPED natural history study (*Natural Course and Prognosis of PFIC and Effect of Biliary Diversion*)
Odevixibat: Expanding Across Multiple Pediatric CLDs

**Estimated U.S./EU populations**

- **PFIC**
  - Late 2019/early 2020 topline Ph.3 PEDFIC 1 results

- **Biliary Atresia**
  - H2 2019 planned pivotal initiation

- **Other Rare CLDs**
  - Alagille
  - PSC
  - Others
  - 2020 plan to expand development

**Pediatric Liver Disease Franchise**
**What Is Biliary Atresia?**

<table>
<thead>
<tr>
<th>Presentation</th>
<th>Cause/Impact</th>
<th>Treatment</th>
<th>Disease Progression</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age ~2-6 weeks</td>
<td>Absence of bile ducts</td>
<td>Kasai surgery may restore bile flow</td>
<td>~50% of patients have liver transplant in first 2 years(^1) (~80% in first 2 decades(^2))</td>
</tr>
<tr>
<td>Acholic stools, jaundice, hepatomegaly, failure to thrive</td>
<td>Bile flow to gut</td>
<td>Transplant is definitive treatment</td>
<td>#1 cause of pediatric liver transplants</td>
</tr>
<tr>
<td>Few pts. pruritic</td>
<td>Hepatic bile acid levels</td>
<td></td>
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</tr>
</tbody>
</table>

**H2 2019 Plan to Initiate Pivotal Trial**
- Orphan designations in U.S. and EU
- Working with regulators to finalize trial design

\(^1\)Data on file  
\(^2\)Lykavieris et al. Hepatology, 2005
Odevixibat: Filing and Albireo Launch Readiness

**Manufacturing**
- Establishing supply chain—on track
- Registration batches—on track

**Regulatory**
- Long-term cancer tox studies—on track
- Repro tox studies—on track
- Supportive Ph.1 program—on track

**Commercial**
- Hired Commercial and Medical Affairs Personnel
- Developed Brand Strategy, Commercialization and Advocacy Plans
- Received FDA conditional approval of proprietary brand name
High Unmet Need and Compelling Opportunity

- **Pediatric Cholestasis:** orphan indications with no approved drug
- **PEBD:** strong clinical rationale for potential benefit of IBAT inhibition
- **Odevixibat:** serum bile acids, pruritus, low diarrhea in pediatric Ph.2 study
- **Exclusivity Position:** orphan drug designations (U.S.-7/EU-12* years); COM 2022/25; MOU for specified cholestatic liver diseases, 2031/34**
- **Attractive P&L:** modest commercial infrastructure required, few target Rx’ers

*Assumes execution of agreed PIP  **Natural expiry/with potential PTE
NASH Program
NASH and Cardiovascular Disease (CVD) Are Associated

- NASH is hepatic manifestation of metabolic syndrome
- Most NAFLD patients die of CVD before reaching end-stage liver disease
- NASH is largely asymptomatic disease

Bile Acids Elevated in NASH Patients

Elevated Total Primary BA\(^1\)  Elevated Total Conjugated Primary BA\(^1\)

In addition, involved in\(^{1,2}\):
- cholesterol levels
- insulin sensitivity
- liver inflammation
- fibrosis

\(^1\)Puri et al. *Hepatology* 2017
\(^2\)Current Diabetes Reports 2011
Albireo’s Two-Pronged Development Approach In NASH

Rationale
- Efficacy—bile acids, cholesterol, glucose, liver inflammation and fibrosis
- Oral 1x/day, minimal systemic exposure, low potential for off-target effects

Elobixibat
Phase 2 Initiate Q2’19
- Locally-active IBATi
- Clinical LDL and GLP1 data
- >1,500 exposures
- Long-term safety data

Novel Bile Acid Modulators
Preclinical
- New mechanism of action
- New chemical structures
- TPP*: high efficacy, low diarrhea
- Accelerating development

*Aspirational target product profile
Deep Biotech and Pharma Experienced Management Team

Ron Cooper  
President and CEO  
Bristol-Myers Squibb  
(President of Europe)

Jan Mattsson, PhD  
Chief Scientific Officer  
(Co-Founder)  
AstraZeneca

Pat Horn, MD PhD  
Chief Medical Officer  
Orphan Technologies, Dyax, Tetraphase, Abbott

Simon Harford  
Chief Financial Officer  
Parexel, GlaxoSmithKline, Eli Lilly

Pamela Stephenson  
Chief Commercial Officer  
Vertex, Pfizer

Martha Carter  
Chief Regulatory Officer  
Aegerion, Proteon, Trine

Jason Duncan  
General Counsel  
Stallergenes Greer, Sobi, EMD Serono

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<table>
<thead>
<tr>
<th>Development Project</th>
<th>Timeline</th>
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<tbody>
<tr>
<td>Elobixibat NASH: Initiate Phase 2 trial</td>
<td>2Q 2019</td>
</tr>
<tr>
<td>Odevixibat biliary atresia: Initiate pivotal program</td>
<td>2H 2019</td>
</tr>
<tr>
<td>Odevixibat PFIC: Initiate PEDFIC 2 second cohort (PEDFIC 1 non-qualifiers)</td>
<td>2H 2019</td>
</tr>
<tr>
<td>Odevixibat PFIC: PEDFIC 1 Phase 3 topline data readout</td>
<td>End of 2019/early '20</td>
</tr>
<tr>
<td>Elobixibat NASH: Phase 2 trial topline data readout</td>
<td>Mid-2020</td>
</tr>
<tr>
<td>Odevixibat: Expand development into additional pediatric orphan liver diseases</td>
<td>2020</td>
</tr>
<tr>
<td>A3384 BAM: Initiate bile acid malabsorption trial*</td>
<td>2020</td>
</tr>
<tr>
<td>Odevixibat PFIC: Approval and launch</td>
<td>1H '21</td>
</tr>
</tbody>
</table>

*Gated on issuance of pending U.S. patent
Hope for Children with Orphan Liver Diseases
Through Bile Acid Modulation

Jefferies Healthcare Conference
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