Hope for Children with Orphan Liver Diseases
Through Bile Acid Modulation

August 2020
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This presentation includes forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include statements, other than historical facts, regarding, among other things: the plans for, or progress, scope, cost, initiation, duration, enrollment, results or timing for availability of results of, development of odevixibat or any of our other product candidates or programs, including regarding expectations regarding the impact of COVID-19 on the United States and our ability to adapt our approach as appropriate; the Phase 3 clinical program for odevixibat in patients with progressive familial intrahepatic cholestasis (PFIC), the pivotal trial for odevixibat in biliary atresia, the planned pivotal trial for odevixibat in Alagille syndrome and a Phase 2 trial for elobixibat being conducted by EA Pharma in Japan; the target indication(s) for development or approval, the size, design, population, location, conduct, cost, objective, enrollment, duration or endpoints of any clinical trial, or the timing for initiation or completion of or availability or reporting of results from any clinical trial, including the Phase 3 PFIC trial for odevixibat, and the long-term open-label extension study, the pivotal trial for odevixibat in biliary atresia, the planned pivotal trial for odevixibat in Alagille syndrome, for submission of any regulatory filing, or for discussions with regulatory authorities; the timing of and our ability to obtain and maintain regulatory approval of any of our product candidates and any related limitations, limitations, or warnings in the label of any approved product candidates; the timing for commercialization of any of our product candidates, if approved; 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 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," "considers," "estimates," "expects," "intends," "may," "will," "should," "could," "estimates," "predicts," "potential," "continue," "guidance," and similar expressions sometimes identify forward-looking statements.

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Albireo: Innovative Science + Deep Pipeline + Well Capitalized

**STRONG BASIC SCIENCE**
- More than a decade of leadership in bile acid modulation
- World’s first regulatory approval for IBATi therapy (elobixibat)

**ORPHAN PEDIATRIC LIVER LEAD ASSET**
- Odevixibat (IBATi) wholly owned, oral QD capsule/sprinkle with MOU patent through 2031/34*, orphan designs., PRIME, PIP, fast track and PRV eligibility
- Adult liver and bile acid malabsorption programs

**SOLID FINANCIAL POSITION**
- Nasdaq listed as ALBO; 15M outstanding shares as of June 30, 2020
- $152M cash and cash equivalents as of June 30, 2020
- Cash into the beginning of 2022; past planned odevixibat approval/launch

*Natural expiry/with potential patent term extension (PTE)
A Robust Pipeline Targeting Liver and GI Diseases/Disorders

**Planned Independent Commercialization**

**Pediatric Liver Diseases**
- Odevixibat
- PFIC
- Biliary Atresia
- Alagille Syndrome
- Other Cholestatic

**Adult Liver Diseases**
- Elobixibat
- Chronic Constipation
- Approved in Japan/Partnered with EA Pharma
- Lead Candidate
- Adult Liver Diseases
- Undisclosed
- Bile Acid Modulators
- Bile Acid Malabsorption
- A3384

**Phases**
- **PRECLINICAL**
- **PHASE 1**
- **PHASE 2**
- **PHASE 3**
- **APPROVED**

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Delivering on Our Plan as a Public Company

2016
- NASDAQ Listing ~$30M
- Elobixibat Milestone Payment ~$8M

2017
- Equity Raise ~$50M
- Legacy Asset Sale ~$4.5M

2018
- Equity Raise ~$100M
- Royalty Monetization ~$45M
- Elobixibat Approval Milestone Payment ~$11M

2019
- ATM Financing ~$21M
- PRV Eligibility Odevixibat

2020
- Equity Raise ~$43M
- Royalty Monetization ~$15M
- Debt Facility $10M

Ph3 Odevixibat Biliary Atresia Pivotal Start
Odevixibat PFIC Ph.3 Last Patient Visit Completed
## Multiple Planned Milestones

<table>
<thead>
<tr>
<th>Milestone</th>
<th>1H’20</th>
<th>2H’20</th>
<th>1H’21</th>
<th>2H’21</th>
<th>2022</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>PFIC PEDFIC 1</strong>: Phase 3 topline data</td>
<td></td>
<td>Mid 2020</td>
<td><em>Last patient, last visit achieved</em></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>PFIC PEDFIC 2</strong> rollover and expanded cohort</td>
<td>Open label</td>
<td></td>
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<td></td>
</tr>
<tr>
<td><strong>Biliary atresia pivotal program</strong></td>
<td>1H’20</td>
<td>Initiation</td>
<td>1H’21</td>
<td></td>
<td>Full site activation</td>
</tr>
<tr>
<td><strong>Alagille syndrome pivotal program</strong></td>
<td>EOY ’20</td>
<td></td>
<td>Initiation</td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>PFIC approval and launch</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Lead Candidate Adult Liver Disease</strong> (MOA undisclosed)</td>
<td>IND-enabling studies</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Novel bile acid modulators</strong></td>
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Odevixibat: Multi-Disease Development Approach
Many Diseases with Cholestasis of the Liver

- Progressive Familial Intrahepatic Cholestasis (PFIC)
- Intrahepatic Cholestasis of Pregnancy
- Primary Biliary Cholangitis
- Drug-Induced Cholestasis
- Primary Sclerosing Cholangitis
- Cystic Fibrosis-Associated Liver Disease
- Biliary Atresia
- AIDS Cholangiopathy
- Malignancy of Bile Ducts
- Alagille Syndrome
- IG4-associated cholangitis
- Malignancy of Bile Ducts
- Low Phospholipid-Associated Cholestasis
- Biliary Strictures
- Cystic Fibrosis-Associated Liver Disease

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Potential Target Indications

~30,000-40,000* patients in the U.S. and EU alone who are lacking an approved pharmacological treatment

- Biliary Atresia (15-20K)
- Pediatric PSC (8-10K)
- PFIC (8-10K)
- Alagille (3-5K)

Potential Target Indications:
- Genetic disorder, paucity of bile ducts
- Genetic disorders with bile acid build-up in liver
- Inflammation and scarring of bile ducts
- Blocked or absent large bile ducts

*Estimate derived from literature, primary market research and modeling. Forecast estimates do not include other regional opportunities, such as Saudi Arabia, Turkey, Asia, LATAM.
## What Is PFIC?

<table>
<thead>
<tr>
<th>Presentation</th>
<th>Genetic Disorder</th>
<th>Disease Progression</th>
<th>Survival</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age ~1-2</td>
<td>Multiple genes, similar symptoms</td>
<td>Inflammation Fibrosis Cirrhosis Death</td>
<td>Almost no patients survive beyond age 20 without surgical diversion or liver transplant*</td>
</tr>
<tr>
<td>Cholestatic/Pruritic</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

*Pawlikowska 2010
Inadequate Treatment Options for PFIC

Off-Label Medications

- UDCA, rifampicin, cholestyramine...

PEBD Surgery

(partial external biliary diversion)\(^1\)

- Bile acid and pruritus reductions
- Undesirable external stoma bag

Liver Transplantation

- Limited timely organ availability
- Need for lifelong immunosuppression
- Morbidity and disease recurrence

\(^1\)Yang, et al. J Pediatr Gastroenterol Nutr 2009
Kennedie’s Story

**Diagnosis**
- Failure to thrive
- Unexplained seizure, brain bleed
- Undetectable levels of Vitamins A, D, E, K
- PFIC 2 diagnosis at 6 months

**Insatiable Pruritis**
“We did what we could. Nothing could comfort her. Nothing helps the insatiable itching.”
-Emily, Kennedie’s mother

**Life Post-Transplant**
- Urgent need for liver transplant
- Lengthy hospitalization
- Various setbacks
- Compromised immune system
- Requires daily immunosuppressive medications

For more patient stories, visit www.pficvoices.com/videos
Odevixibat: A Profile Potentially Suitable for Pediatric Use

- Once-Daily Dosing
- Oral Capsule or Sprinkles
- Minimal Systemic Exposure
- Favorable Tolerability Profile*

*In Phase 2 clinical trial
**NAPPED: Natural Course and Prognosis of PFIC and Effect of Biliary Diversion**

**PFIC2 Native Liver Survival Improvement**

- **PFIC1 Native Liver Survival Improvement**

*Improved native liver survival does not require bile acid normalization*

*Van Wessel et al. 10.1016/j.hep.2020.02.007, Would be 100%, but one patient died due to complications of multiple PEBD surgeries ** Van Wessel Espghan 2019*
Odevixibat: Phase 2 Trial in Pediatric Cholestatic Liver Disease

Odevixibat doses evaluated (µg/kg), 4 weeks

10  30  60  100  200

PFIC, Biliary Atresia, Alagille Syndrome, Intrahepatic Cholestasis Patients

- Open-label, dose-finding trial
- Primary endpoints: TESAEs and serum bile acid change
- Baseline – single test dose – 2-wk washout – 4-wk treatment
- Trial initially designed with a maximum dose of 300 µg/kg
- N=24 (20 unique + 4 retreated)

Oral late breaker EASL’17/Presidential Poster of Distinction AASLD’17
Primary Efficacy Endpoint:
Reduction Demonstrated in Serum Bile Acids

Phase 2 trial was an open-label, dose-finding trial 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 meet inclusion criteria for Phase 3 trial.

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

Baseline μM

<table>
<thead>
<tr>
<th>Patient</th>
<th>Alagille Syndrome</th>
<th>Biliary Atresia</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>260</td>
<td>43</td>
</tr>
<tr>
<td>2</td>
<td>116</td>
<td>136</td>
</tr>
<tr>
<td>3</td>
<td>338</td>
<td>132</td>
</tr>
<tr>
<td>4</td>
<td>26</td>
<td>-58</td>
</tr>
<tr>
<td>5</td>
<td>121</td>
<td>-51</td>
</tr>
<tr>
<td>6</td>
<td>564</td>
<td>-14</td>
</tr>
</tbody>
</table>

Serum Bile Acids % reduction from baseline

10-200 ug/kg dose, 4 weeks of treatment

30 ug/kg, 4 weeks of treatment

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

\[ a_p=0.008, r=0.54, n=23. \]
\[ b_p=0.004, r=0.58, n=23. \]
\[ c_p=0.006, r=0.57, n=22. \]
\[ d_p=0.005, r=0.57, n=22. \]

n<24 reflects missing scores. All tools based on 0–10 scales except Whitington tool (0–4 scale).
Favorable Tolerability Profile in Trial

- All patients completed treatment; no evidence of diarrhea during 4-week treatment period
- 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)
- Decision made not to dose escalate above 200 µg/kg
  - Some transaminase elevations at 200 µg/kg
PEDFIC 1&2: Phase 3 PFIC Program Summary

**Pediatric PFIC (PEDFIC)**

- **62 Subjects**
  - Target 60
  - Oral capsule/sprinkle
  - Once daily

- **24-Week Treatment**
  - Odevixibat 40 µg/kg/day N~20
  - Odevixibat 120 µg/kg/day N~20
  - Placebo N~20

- **Endpoints**
  - **FDA**
    - Assessment of change in pruritus
  - **EMA**
    - Serum bile acid responder rate (reach ≤70 μmol/L or a reduction of 70%)

- **Double-Blind, Randomized, Placebo-Controlled Trial to Demonstrate Efficacy and Safety of Odevixibat in Children with PFIC**

- **LAUNCHED EXPANDED ACCESS PROGRAM U.S., EUROPE, CANADA, AND AUSTRALIA**

- **Key Inclusion Criteria:**
  - Diagnosis of PFIC1 or 2
  - Confirmed BSEP activity
  - Serum bile acids ≥100 μmol/L
  - Pruritus ≥2 on 0-4 scale

- **PEDFIC 2**
  - Rollover cohort extension trial
  - Expanded cohort non-PEDFIC 1 eligible

- **Launched Expanded Access Program**

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1a. How bad was your worst itching since you went to bed last night?

PRO+ObsRO: 0-4 scales

- Each response distinguished by pictures, words, numbers and colors
- Tested with both patients and caregivers
- Multiple interactions with FDA in development of the tool
## Planning For Success

### Manufacturing
- Agreed elements of CMC plan w/FDA
- Planned commercial formulation in Ph3
- Registration batches on stability

### Expand Pt. Population
- Initiated biliary atresia pivotal trial
- Plan to initiate Alagille pivotal trial
- Evaluate additional indications

### Go to Market
- KOL engagement
- Pricing and access planning
- Patient support program build
## Expansion Opportunity: Biliary Atresia

<table>
<thead>
<tr>
<th><strong>Presentation</strong></th>
<th><strong>Cause</strong></th>
<th><strong>Treatment</strong></th>
<th><strong>Disease Progression</strong></th>
</tr>
</thead>
<tbody>
<tr>
<td>Age ~2 wk-3 mos.</td>
<td>Absence of bile ducts</td>
<td>Kasai (HPE)</td>
<td>~50% of patients have liver transplant in first 2 years¹</td>
</tr>
<tr>
<td>Failure to thrive</td>
<td>Acholic stools</td>
<td>Surgery may restore bile flow</td>
<td>Transplant is definitive treatment</td>
</tr>
<tr>
<td>Jaundice</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

### #1 Cause of Pediatric Liver Transplants

Estimated Prevalence 15-20K (U.S./EU)

¹Data on file;²Lykaviers et al. *Hepatology*, 2005
Bile Acids: Significant Impact in Biliary Atresia

**Lower sBA Correlated With Improved NLS**

<table>
<thead>
<tr>
<th>Median Serum Bile Acid Concentration (μmol/L)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Alive Native Liver</td>
</tr>
<tr>
<td>59</td>
</tr>
<tr>
<td>39</td>
</tr>
<tr>
<td>227</td>
</tr>
<tr>
<td>Death or Liver Transplant</td>
</tr>
<tr>
<td>139</td>
</tr>
<tr>
<td>110</td>
</tr>
<tr>
<td>Kasai Surgery</td>
</tr>
<tr>
<td>59</td>
</tr>
<tr>
<td>39</td>
</tr>
<tr>
<td>n= 516</td>
</tr>
</tbody>
</table>

**Improved Liver Markers Correlated With Lower Serum Bile Acids**

- **ALT** (≤40 U/L)
- **GGT** (≤55 U/L)
- **Platelets** (≥150/μL)
- **Spleen** (≤2 cm below costal region)

**sBA Reduction Correlated With Sustained Improvements Post-Kasai (HPE) Over 2 Yrs.**

ALT, alanine aminotransferase; GGT, gamma-glutamyl transferase; HPE, hepatoportoenterostomy; sBA, serum bile acids.

**BOLD: Precedent-Setting Biliary Atresia Pivotal Trial**

*Biliary Atresia and the Use of Odevixibat in Treating Liver Disease (BOLD)*

**24-Month Treatment**

- **Odevixibat**
  - 120 µg/kg/day
  - N= 100

- **Placebo**
  - N= 100

**Primary Endpoint**

Proportion of patients who are alive and have not undergone a liver transplant

**FDA/EMA: Single Pivotal Sufficient to Support Filing**

**~70 global sites will be initiated**

---

**Rollover cohort Extension Trial**

**~200 Subjects Post-Kasai HPE**

- Oral capsule/sprinkle
- Once daily

**Key Inclusion Criteria:**

- Age at Kasai HPE ≤ 90 days
- Randomized within 3 weeks

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Expansion Opportunity: Alagille Syndrome

Presentation

Age
~4-12 Mos.

Multiple Symptoms

Genetic Disorder

Autosomal dominant

Impact

Paucity of bile ducts

Disease Progression

Many patients may need a liver transplant
Disease can stabilize

Initiation of Planned Pivotal Trial by EOY 2020

FDA and EMA Agreement on Protocol Design

- Estimated prevalence 3-5K (U.S./EU)
- Orphan designations received in U.S. and EU
Odevixibat: Expanding Development Across Pediatric CLDs

PFIC
- Pivotal trial initiated H1 2020
- Mid-2020 topline Ph.3 results anticipated

Biliary Atresia
- Planned pivotal trial initiation by EOY 2020

Alagille

Other Rare CLDs
- Other Indications

Pediatric Liver Disease Franchise
Commercialization Strategy

U.S. Launch

Identify Patients

Competitive Profile

Drive Access to Accelerate Uptake

EU Launch

Focused Medical Presence

Strong Market Access

Flexible Commercial Operations

RoW Strategy

Robust Country Prioritization

Strong Local Partners

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Odevixibat Go-to-Market Plan

PFIC Ph3 Results

U.S./EU Launch

2019

Completed Commercial Activities

2020

Doctors

Account Mapping

Hire Field Management

Hire and Train Field Teams

Data Presentations & Publications

Market Access

Value Story and Economic Models

Hire Account Team

Scientific Exchange

Finalize Pricing

Early Access Programs & Distribution Planning

2021

Patients

Develop Patient Support Program

Hire and Train Case Managers

Expand PFIC Awareness Campaign and Ongoing Advocacy

Key Commercial Hires

Physician, Patient & Payer Research

PFIC Voices & Advocacy

Market Access Strategy

Global Market Prioritization

Brand Name

Completed Commercial Activities

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Off-Label Reimbursement

- Clinical trial data
- Managed care negotiations
- Real-world evidence
- Reimbursement strategies

Pediatric and Orphan Populations

- New Use Formulations
- Routes of Administration
- Method of Use Patent Expiration 2034*
  - 3 patents, 10+ claims targeted to PFIC
  - Multiple Orange-Book listable patents for PFIC and CLDs
- Orphan exclusivity in the U.S. (7 yrs.) and EU (10+2yrs.)
- Composition of Matter 2025*

*with PTE and pediatric extensions
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 trial
- **Three Pivotal Programs**: PFIC, biliary atresia, Alagille syndrome
- **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
Multiple Upcoming Milestones Anticipated

<table>
<thead>
<tr>
<th>Milestone</th>
<th>Timeframe</th>
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<tbody>
<tr>
<td>Odevixibat PFIC: PEDFIC 1 Phase 3 topline data</td>
<td>Mid 2020</td>
</tr>
<tr>
<td>Odevixibat Alagille syndrome: Initiate planned pivotal program</td>
<td>EOY 2020</td>
</tr>
<tr>
<td>Elophibat NASH: Japan Phase 2 trial topline data</td>
<td>EOY 2020/1Q’21</td>
</tr>
<tr>
<td>Odevixibat PFIC: Potential approval and launch</td>
<td>2H 2021</td>
</tr>
</tbody>
</table>
Hope for Children with Orphan Liver Diseases
Through Bile Acid Modulation

August 2020