# Post-AASLD Business Update

November 16, 2021

(Nasdaq: ALBO)

Albireo

For Media & Investors Only

# Forward Looking Statements

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 statements of historical fact, regarding, among other things: Albireo’s commercialization plans and expectations for commercializing Bylvay in the U.S. and Europe; the plans for, or progress, scope, cost, initiation, duration, enrollment, results or timing for availability of results of, development of Bylvay, A3907, A2342 or any other Albireo product candidate or program; the pivotal trial for Bylvay in biliary atresia (BOLD); the pivotal trial for Bylvay in Alagille syndrome (ASSERT); the Phase 1 trial for A3907; the IND-enabling studies for A2342; 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 long-term open-label extension study for Bylvay in PFIC, the BOLD and ASSERT trials, Phase 1 trial for A3907 and the IND-enabling studies for A2342; expectations that biliary atresia is one of the most common rare pediatric liver diseases and is the leading cause of liver transplants in children; potential regulatory approval by and discussions with the FDA or EMA regarding our programs; expectations that the Company’s distribution and supply agreements will drive availability of Bylvay in key markets globally, and potential revenue that may be generated by such agreements; potential regulatory approval and plans for potential commercialization of Bylvay in countries outside of the U.S. and Europe, including Japan; the potential benefits or competitive position of Bylvay or any other Albireo product candidate or program or the commercial opportunity in any target indication; the potential effects of Bylvay of the treatment of PFIC patients and its potential to improve the current standard of care; the potential benefits of an orphan drug designation; the length of time for which Albireo’s cash resources are expected to be sufficient, and the milestones and activities to be funded with those cash resources; or Albireo’s plans, expectations or future operations, financial position, revenues, costs or expenses. Albireo often uses words such as “anticipates,” “believes,” “plans,” “expects,” “projects,” “future,” “intends,” “may,” “will,” “should,” “could,” “estimates,” “predicts,” “potential,” “planned,” “continue,” “guidance,” or the negative of these terms or other similar expressions to identify forward-looking statements. Actual results, performance or experience may differ materially from those expressed or implied by any forward-looking statement as a result of various risks, uncertainties and other factors, including, but not limited to: there are no guarantees that Bylvay will be commercially successful; we may encounter issues, delays or other challenges in launching or commercializing Bylvay; whether Bylvay receives adequate reimbursement from third-party payors; the degree to which Bylvay receives acceptance from patients and physicians for its approved indication; challenges associated with execution of our sales activities, which in each case could limit the potential of our product; challenges associated with supply and distribution activities, which in each case could limit our sales and the availability of our product; results achieved in Bylvay in the treatment of patients with PFIC may be different than observed in clinical trials, and may vary among patients; other potential negative impacts of the COVID-19 pandemic, including on manufacturing, supply, conduct or initiation of clinical trials, or other aspects of our business; whether favorable findings from clinical trials of Bylvay to date, including findings in indications other than PFIC, will be predictive of results from other clinical trials of Bylvay; there is no guarantee that Bylvay will be approved in jurisdictions or for indications beyond the jurisdictions in which or indications for which Bylvay is currently approved; there is no guarantee that our other products candidates will be approved; estimates of the addressable patient population for target indications may prove to be incorrect; the outcome and interpretation by regulatory authorities of the ongoing third-party study pooling and analyzing of long-term PFIC patient data; the timing for initiation or completion of, or for availability of data from, clinical trials of Bylvay, including BOLD and ASSERT, and the Phase 1 clinical trial of A3907, and the outcomes of such trials; Albireo’s ability to obtain coverage, pricing or reimbursement for approved products in the United States or Europe; delays or other challenges in the recruitment of patients for, or the conduct of, the Company’s clinical trials; and the Company’s critical accounting policies. These and other risks and uncertainties that Albireo faces are described in greater detail under the heading “Risk Factors” in Albireo’s most recent Annual Report on Form 10-K or in subsequent filings that it makes with the Securities and Exchange Commission. As a result of risks and uncertainties that Albireo faces, the results or events indicated by any forward-looking statement may not occur. Albireo cautions you not to place undue reliance on any forward-looking statement. In addition, any forward-looking statement in this presentation represents Albireo’s views only as of the date of this presentation and should not be relied upon as representing its views as of any subsequent date. Albireo disclaims any obligation to update any forward-looking statement except as required by applicable law.

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# Agenda

A2342 Next Gen Oral HBV/HDV Treatment

PEDFIC 1 & 2 Bylvay™ (odevixibat) Data

Update on A3907

Q&A

## Albireo Leadership Here Today

**Ron Cooper**
*President and CEO*

**Former Bristol-Myers Squibb (President of Europe)**

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

**Former AstraZeneca**

**Pat Horn, MD, PhD**
*Chief Medical Officer*

**Former Orphan Technologies, Dyax, Tetraphase, Abbott**

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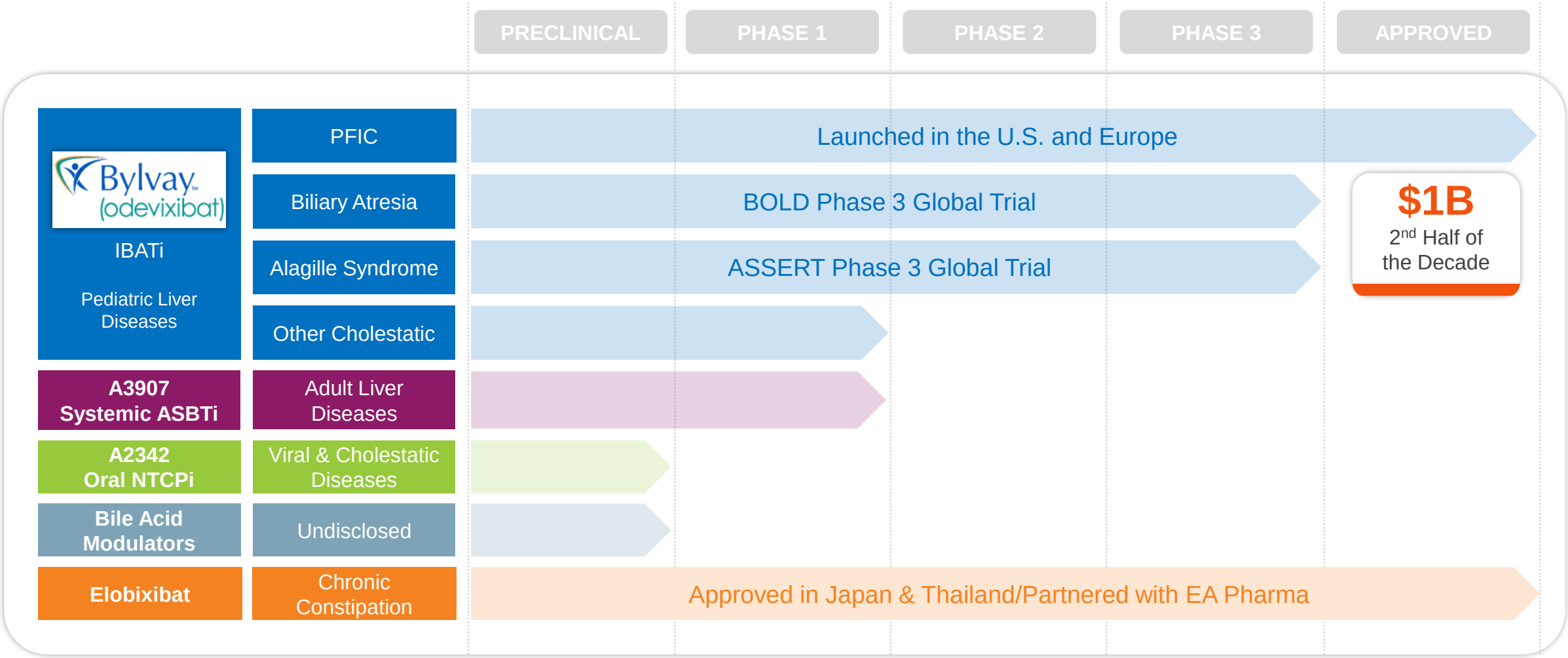
# Robust Platform with Multiple Growth Opportunities




IBATi = ileal bile acid transport inhibitor, ASBT = apical sodium-dependent bile acid transporter, NTCPi = sodium-taurocholate co-transporting peptide inhibitor

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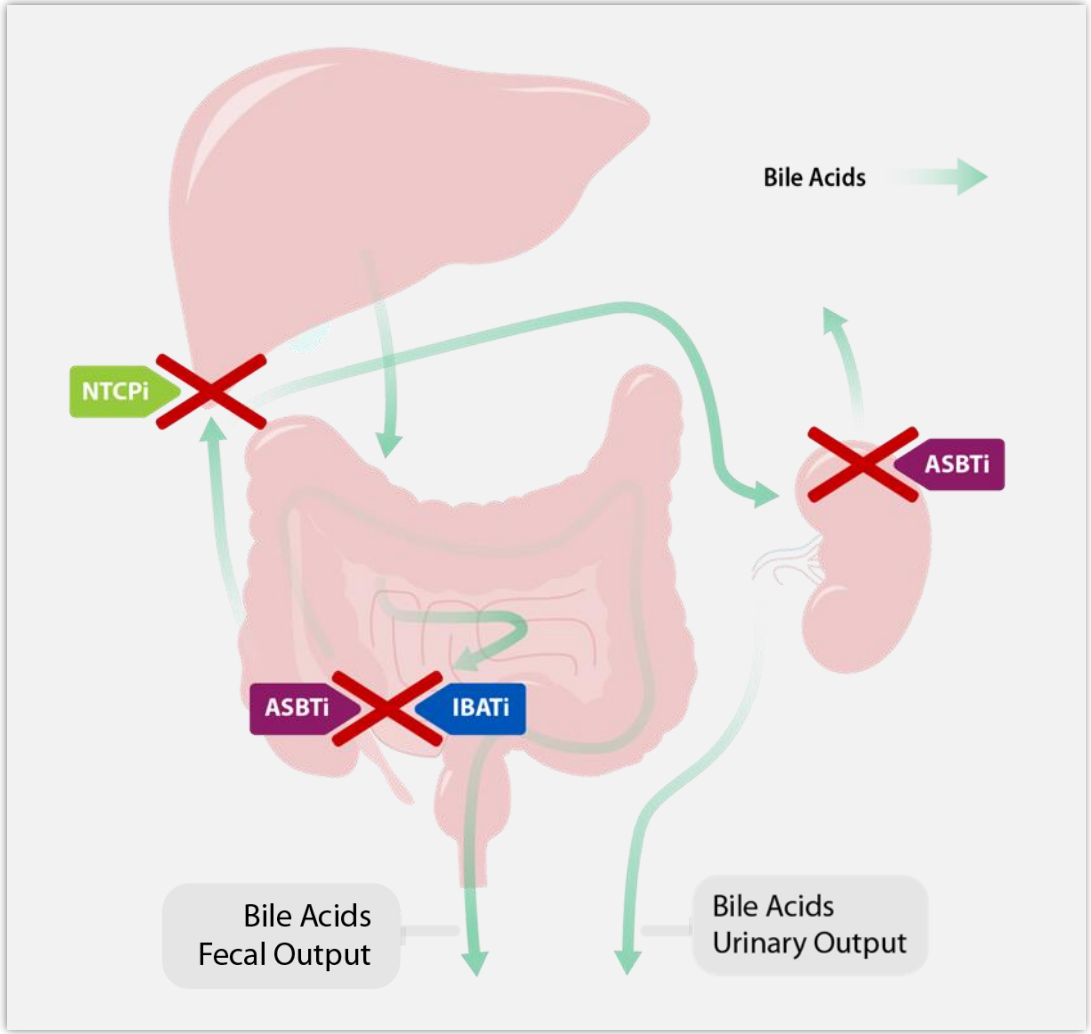
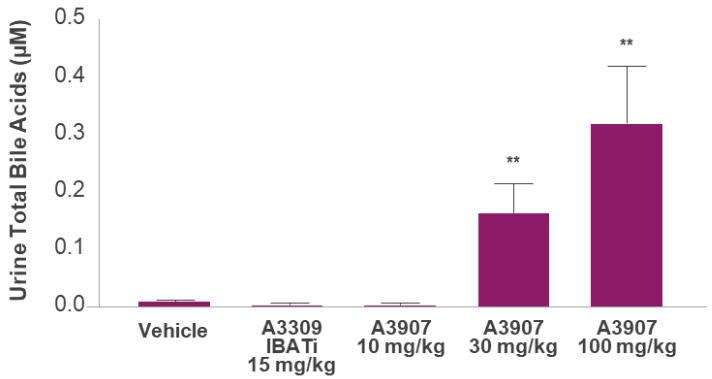
# Novel Compounds with Distinct MOA Designed to Regulate Bile Acid Movement in Specific Diseases




| BYLVAY/ELOBIXIBAT | BYLVAY/ELOBIXIBAT |
|---|---|
| IBATi Ileum<br>Liver<br>Kidney | **Pediatric Cholestasis**<br>**Constipation** |
| **A3907** | **A3907** |
| ASBTi Ileum<br>Liver<br>ASBTi Kidney | **Adult Cholestasis** |
| **A2342** | **A2342** |
| Ileum<br>NTCPi Liver<br>Kidney | **Hepatitis B & D**<br>**Cholestasis** |

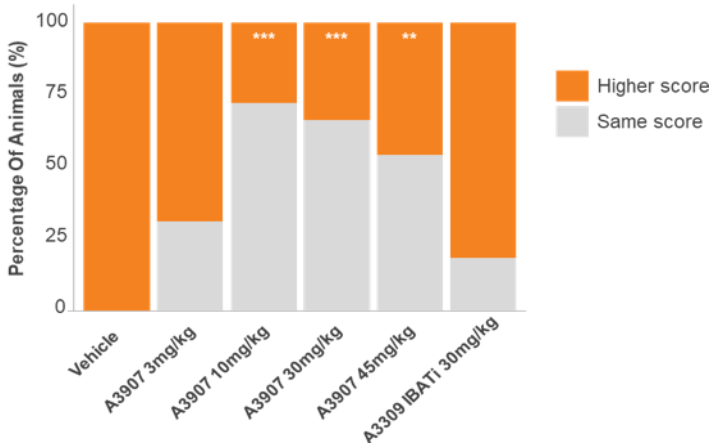
# Next Gen Bile Acid Modulator A3907: Different vs IBATi[1]

## Increases Urinary Bile Acids[2]

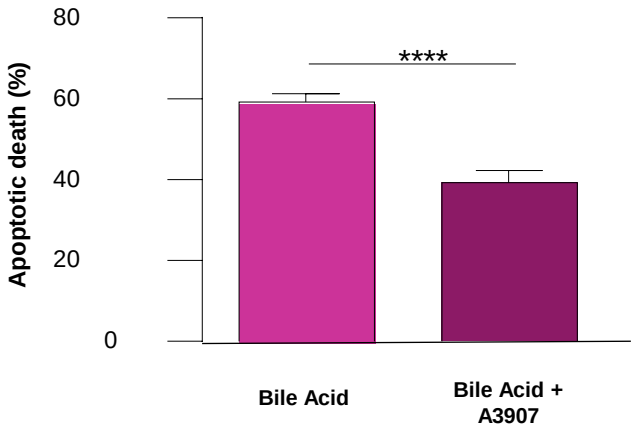



## Differential Gene Expression vs IBATi[4]


## Improved Fibrosis Stage vs IBATi[3]



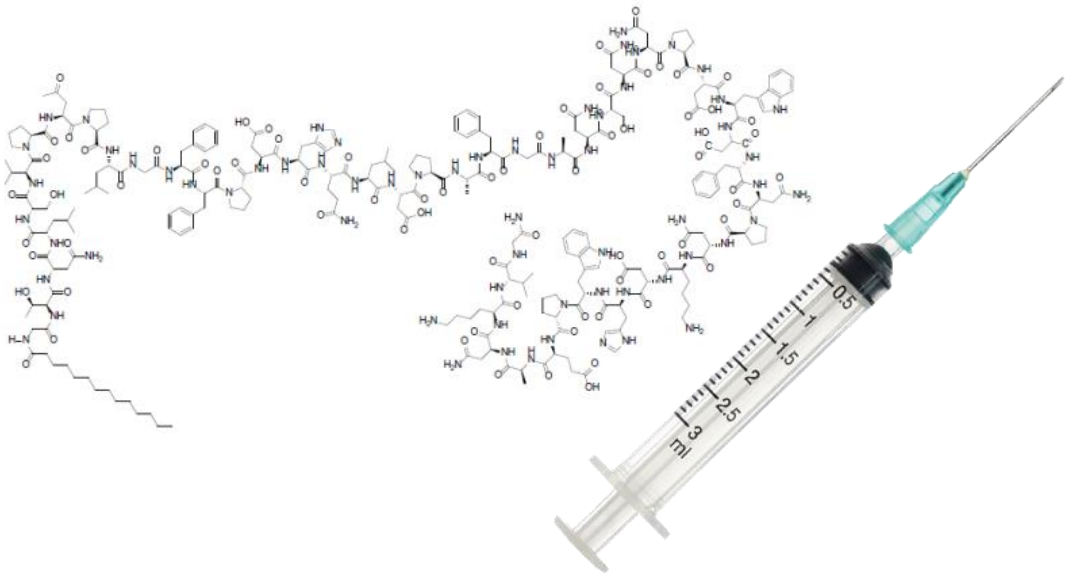

## Reduction of Bile Acid Induced Cholangiocyte Apoptosis[5]




1. Data from preclinical models
2. Gillberg et al. 2020 Hepatology 72 (Suppl 1)
3. Åkerblad et al. 2020 Hepatolgy 72 (Suppl 1)
4. 4-weeks treatment in diet induced mouse NASH model
5. Vehicle or Glycodeoxycholic acid induced apoptosis of rat cholangiocytes for 48h in vitro

# A2342: Clinically Proven Rationale for HBV/HDV Treatment

**Now: Peptide, SubQ NTCP Inhibitor Hepcludex®**



**Future: Small Molecule, Oral NTCP Inhibitor A2342**

**A2342: First Oral NTCP & Viral Entry Inhibitor**

Oral NTCP inhibitor with potential for optimal efficacy, flexible dosing, increased convenience and greater combinational options with other HBV and HDV therapies.

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# Strong Initial Bylvay™ (odevixibat) Data

**PEDFIC 1 Topline Data**
September 2020

- 24-week topline data set
- Improvements in pruritus
- Reductions in serum bile acids, normalized in many patients
- Pruritus improvements demonstrated in PFIC 1, 2 & 3
- Overall AE profile similar to placebo with low diarrhea

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# Rapid, Durable & Sustained Clinical Benefits of Bylvay™ (odevixibat)

**PEDFIC 1 Topline Data**
September 2020

- 24-week topline data set
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- Overall AE profile similar to placebo with low diarrhea

**PEDFIC 1 & 2 Study Data**
November 2021

- 72-week data, with some patients treated up to 128 weeks
- Sustained improvements in pruritus severity
- Rapid reductions in serum bile acids
- Improved liver health and function across PFIC types
- Observed safety and tolerability profile of Bylvay was consistent across studies, treatment groups and doses, regardless of PFIC classification or BSEP subtype

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# A2342
# Hepatitis B & D

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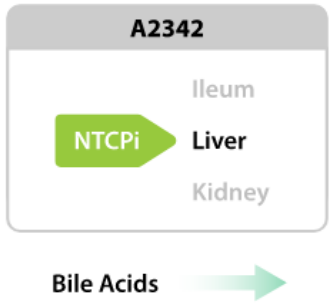
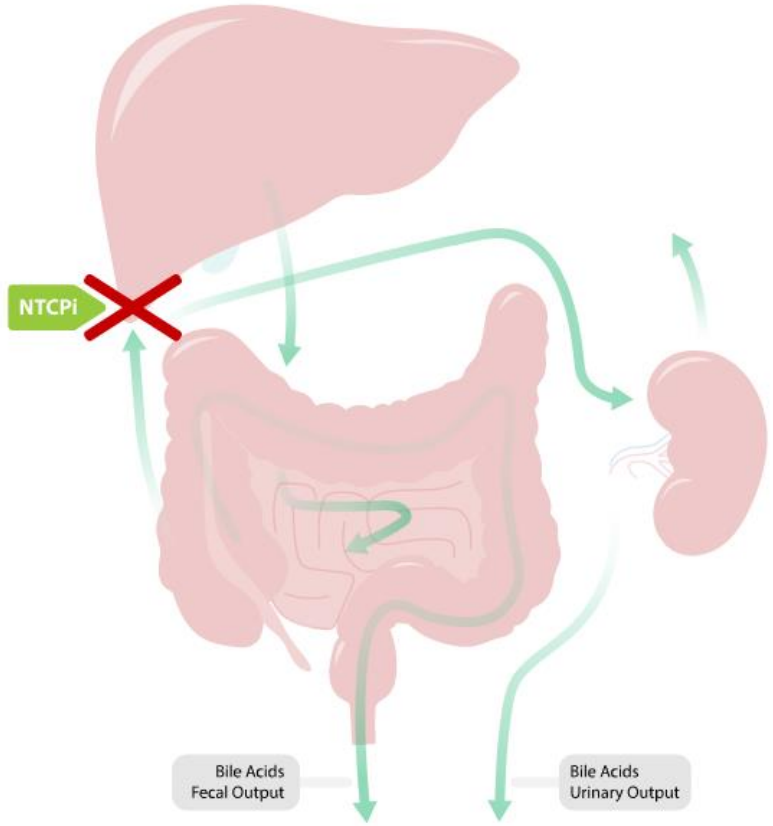
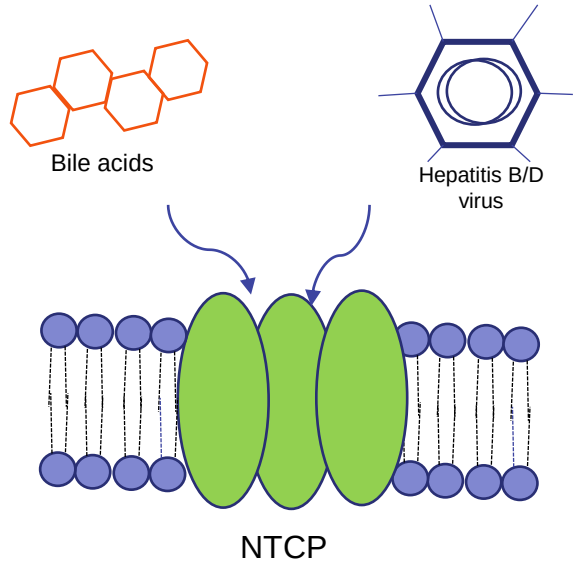
# A2342: Novel Oral NTCP Inhibitor to Improve Efficacy of Next-Generation Combination Therapies in HBV and HDV

Goal to develop an oral NTCP inhibitor with optimal efficacy, increased convenience and greater combinational options with other therapies

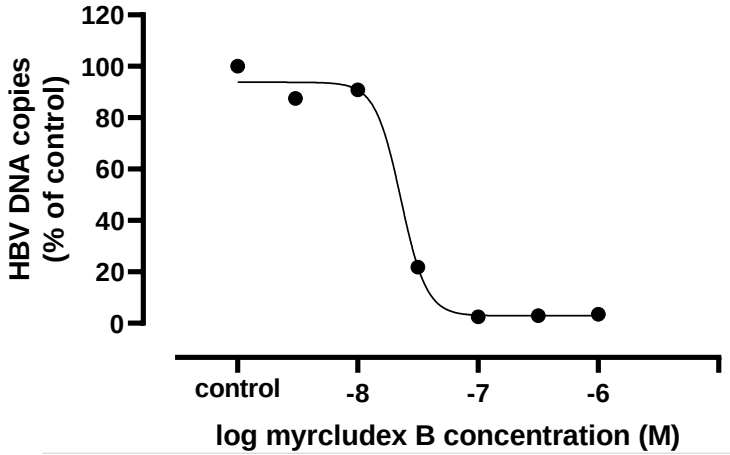


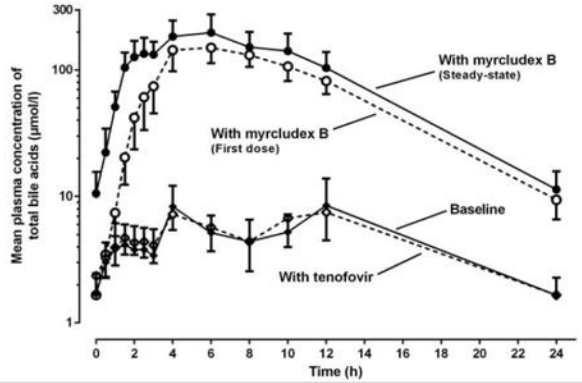
# Hepcludex® (Bulvertide, Myrcludex) Profile

## Viral Load Reduction[1]




## NTCP Target Engagement In Vivo[2]

Transient sBA increase




## SubQ Delivery

- Approved dose in EU: 2 mg
- Refrigerated product for once-daily SubQ delivery

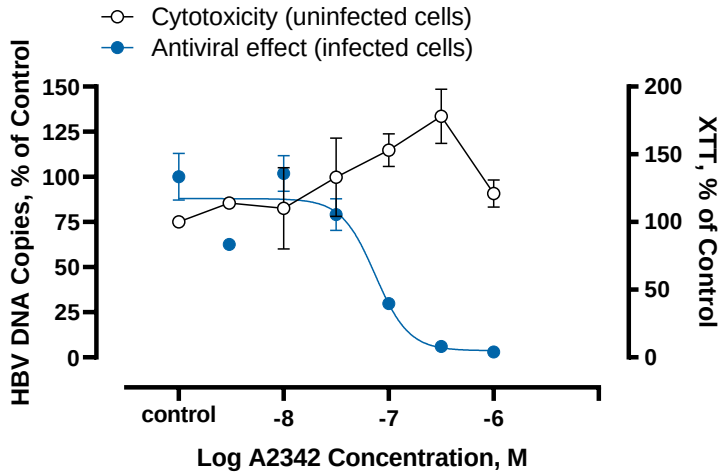
## Safety and Tolerability

- NTCP inhibition - well tolerated and safe in clinical trials[3]
- Monotherapy in HDV patients[3]

[1] HBV infection of isolated human hepatocytes for 10-days in vitro
[2] Blank et al. Clin. Pharmacol. Ther. 2018
[3] Kang C and Syed YY. 2020, Drugs 80; EMA Hepcludex assessment report ((www.ema.europa.eu/en/medicines/human/EPAR/hepcludex))

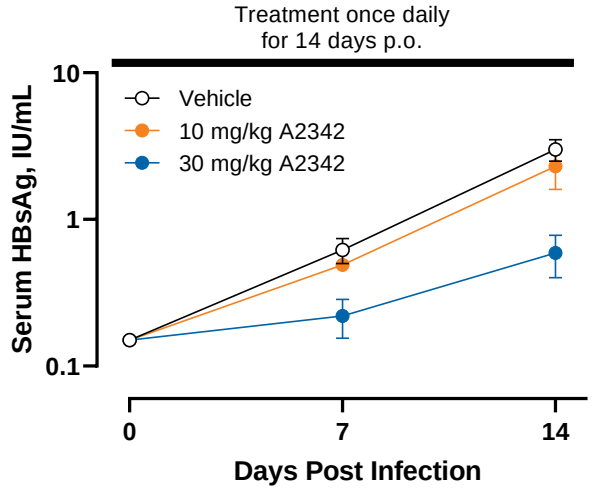
# A2342 Blocks HBV Infection In Vitro and In Vivo

## Prevents HBV Entry In Vitro




- Dose-dependent efficacy in HBV-infected human hepatocytes
- Potency similar to Myrcludex B
- No cytotoxicity detected

## Prevents HBV Infection In Vivo (Oral Administration)




- A2342 attenuates HBV replication in humanized uPA/SCID mice in a dose-dependent manner

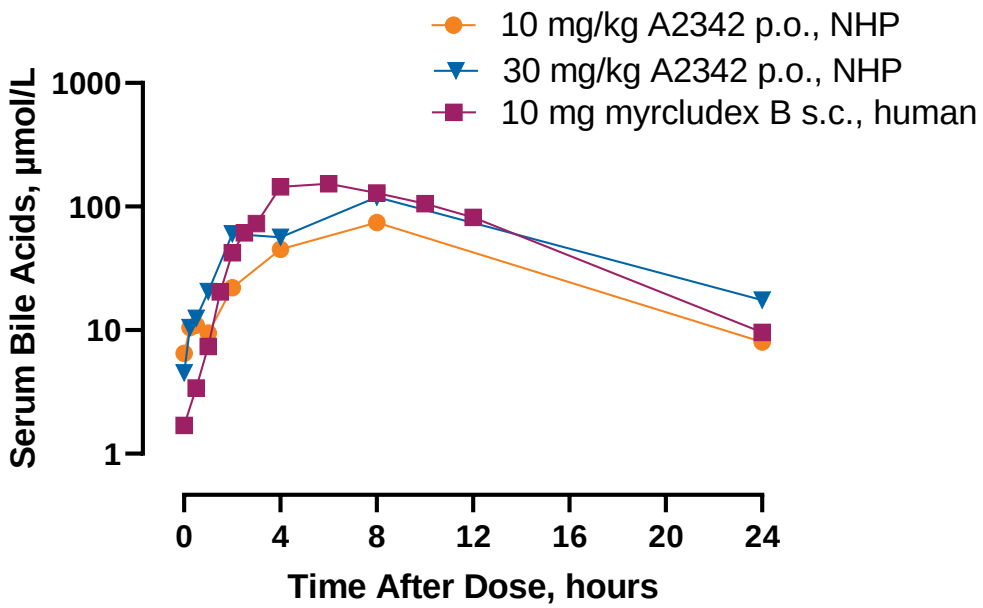
**Preliminary data show similar efficacy on HDV infection**

Mean (SEM) values (n=3) are depicted. HBV, hepatitis B virus.
Mean (SEM) values for 2–4 mice per group are depicted. HBeAg, hepatitis B e-antigen; HBsAg, hepatitis B surface antigen; HBV, hepatitis B virus; p.o., orally.
Lindström E, et al. Poster Presentation at AASLD: The Liver Meeting 2021, American Association for the Study of Liver Diseases; November 12-15, 2021.

# A2342 Shows NTCP Target Engagement in NHP

## Similar transient increase in serum bile acids as with Hepcludex® in humans

**Comparison Between the Effect of A2342 on Serum Bile Acids in NHPs and the Effect of Myrcludex B in Human Volunteers**




- 10 mg Myrcludex B (s.c.) increases serum bile acids 10-15-fold in human volunteers
- 10 and 30 mg/kg A2342 (p.o.) increases serum bile acids to a similar degree in non-human primates
- This indicates that the NTCP target engagement achieved by Myrcludex B is readily achievable with A2342

Mean values from n=2 animals per A2342 dose group or n=12 human volunteers are depicted. NHP data with A2342 were generated in the current study. Myrcludex B data reflect digitized published data from Blank A, et al. Clin Pharmacol Ther. 2018;103:341-8. NHP, nonhuman primate. p.o., orally; s.c., subcutaneously.

Lindström E, et al. Poster Presentation at AASLD: The Liver Meeting 2021, American Association for the Study of Liver Diseases; November 12-15, 2021.

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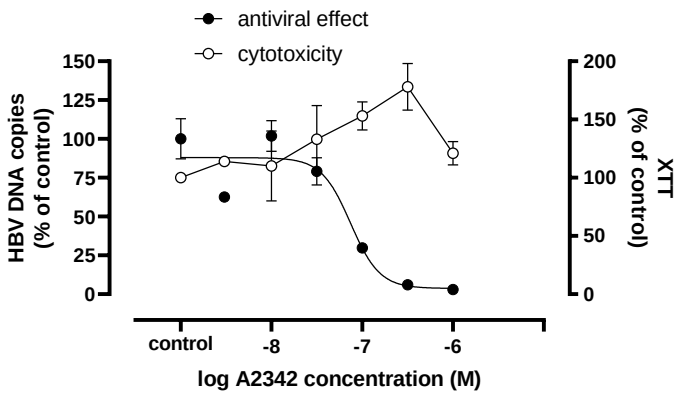
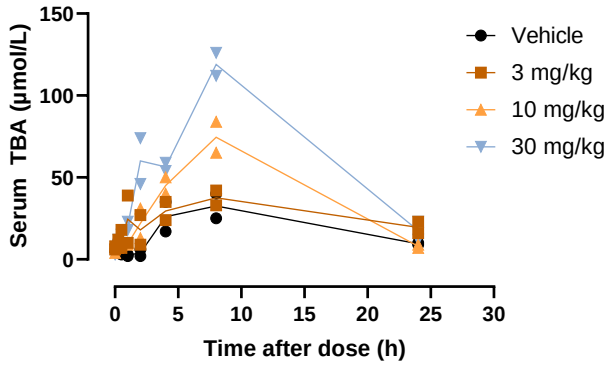
# A2342 Follows Hepcludex® Proof of Concept+

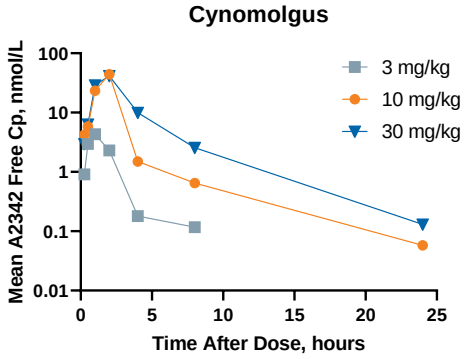
## Viral Load Reduction




## NTCP Target Engagement In Vivo




## Oral Administration & PK Profile




## Safety and Tolerability


Mean (SEM) values (n=3) are depicted. HBV, hepatitis B virus.
Mean (SEM, if available) values for 2–4 animals per group are depicted. OATP KO, organic anion transporting polypeptide 1a/1b knockout; SEM, standard error of the mean; TBA, total bile acid.
Mean values for 2–5 animals per group are depicted. Cp, plasma concentration; OATP KO, organic anion transporting polypeptide 1a/1b knockout.
Lindström E, et al. Poster Presentations at AASLD: The Liver Meeting 2021, American Association for the Study of Liver Diseases; November 12-15, 2021.

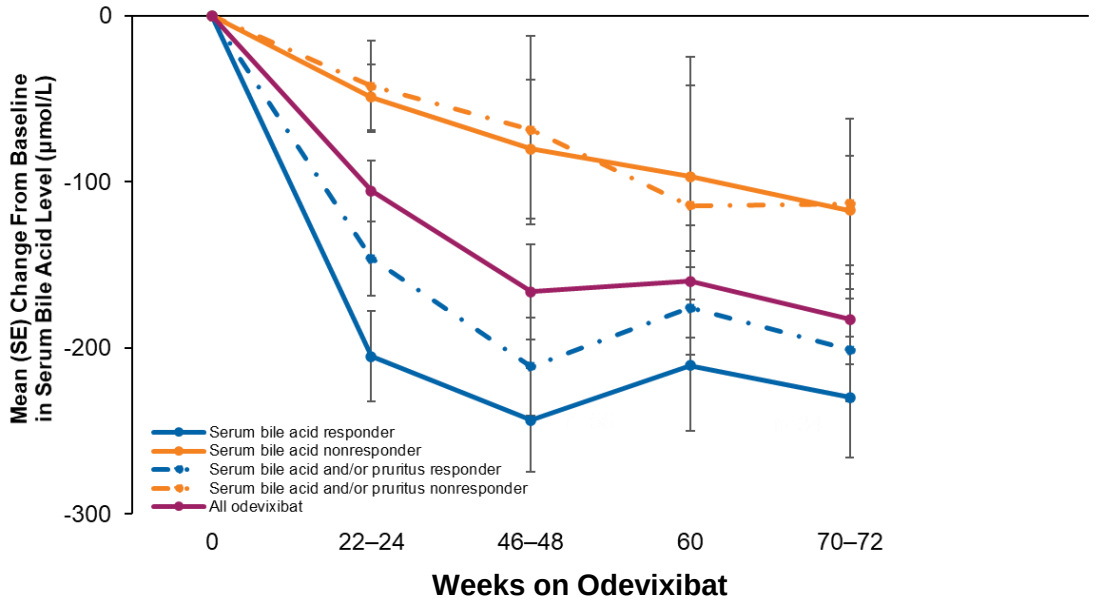
# Bylvay™ (odevixibat) PEDFIC 1 & 2 Studies

For Media & Investors Only

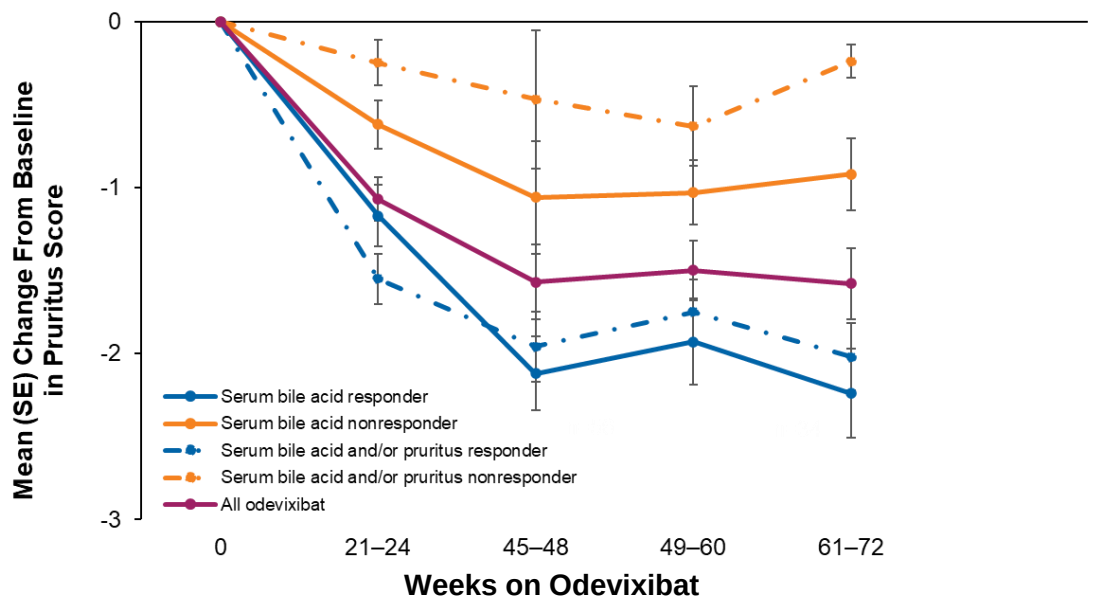
# Bylvay™ (odevixibat) Long-Term Clinical Treatment Benefits

## Bylvay therapy up to 128 weeks showed improved liver health and function

### Serum Bile Acids




### Pruritus




- Bylvay responders had sustained improvements in mean serum bile acids and pruritus scores over time
- sBA responders had larger improvements in pruritus than sBA non-responders/partial responders
- Bylvay was generally well tolerated in both responders and non-responders
- Data at 72 weeks, with trends continuing out to 128 weeks

This pooled analysis covers the period from the first-ever dose of odevixibat in PEDFIC 1 or PEDFIC 2 through December 4, 2020. Values at the right side of the graph represent mean changes during the last assessment interval. Serum bile acid response: ≥70% reduction in serum bile acids or serum bile acids ≤70 µmol/L (baseline level had to be >70 µmol/L for this analysis) at last available assessment up to week 72. Serum bile acid and/or pruritus response: Serum bile acid response at last available assessment and/or pruritus score reduction of ≥1 point from baseline based on last available monthly or 12-week interval score up to week 72. SE, standard error. Loomes KM, et al. Poster Presentation at AASLD: The Liver Meeting 2021, American Association for the Study of Liver Diseases; November 12-15, 2021.

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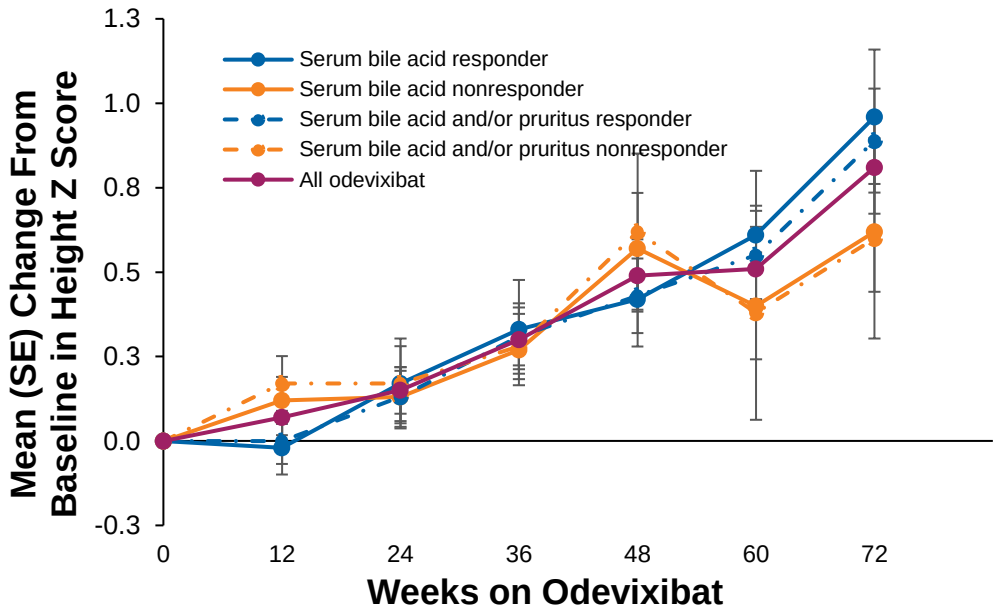
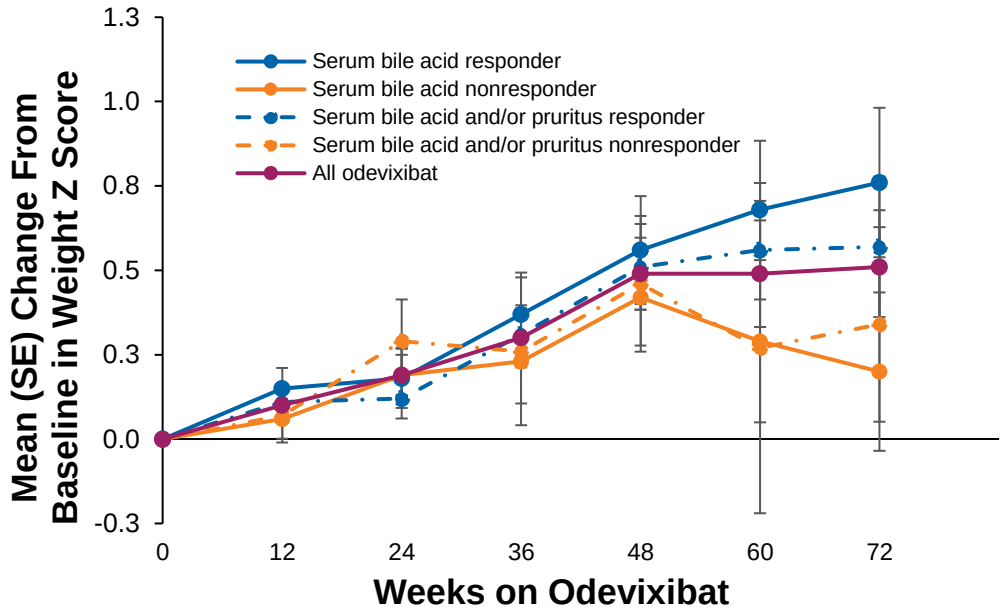
# Evidence for Disease Modification

## Long-term data showed Bylvay™ (odevixibat) therapy up to 128 weeks sustained improvements in hepatic health, quality of sleep and growth, reducing disease burden

**Height Z Score Over Time**




**Weight Z Score Over Time**




With Bylvay treatment, mean height and weight Z scores increased in the overall population of Bylvay-treated patients in both responders and non-responders.

This pooled analysis covers the period from the first-ever dose of odevixibat in PEDFIC 1 or PEDFIC 2 through December 4, 2020. SE, standard error.

McKiernan P, et al. Poster Presentation at AASLD: The Liver Meeting 2021, American Association for the Study of Liver Diseases; November 12-15, 2021.

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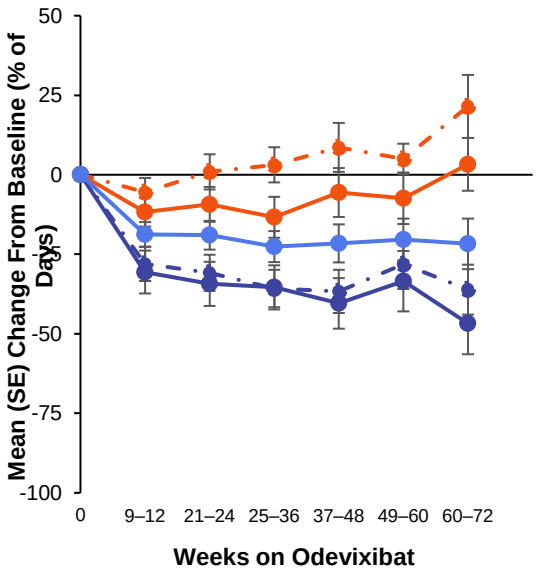
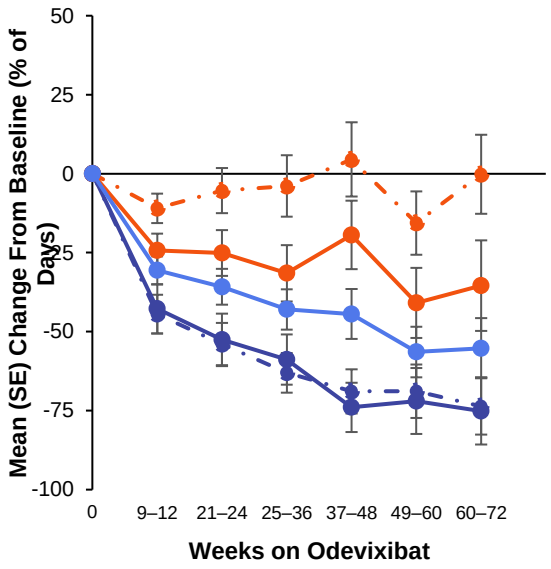
# Evidence of Improvement in Quality of Life Measures

After 72 weeks of treatment, the overall population of Bylvay-treated patients as well as Bylvay responders had large decreases (i.e., improvements) in several caregiver-reported sleep parameters

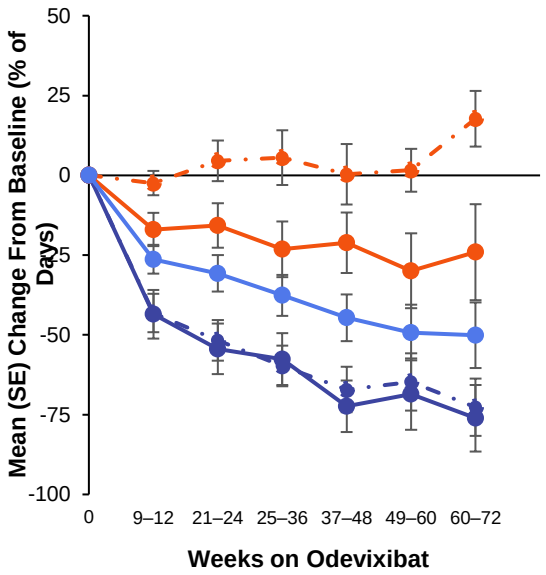
**Seeing Blood Due to Scratching**



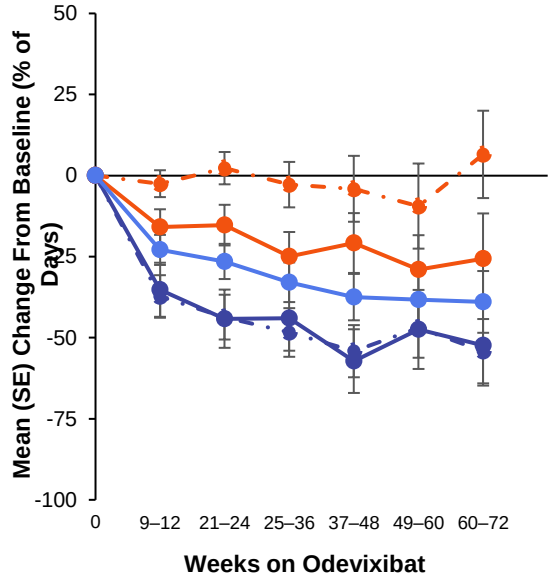

**Needing Help Falling Asleep**

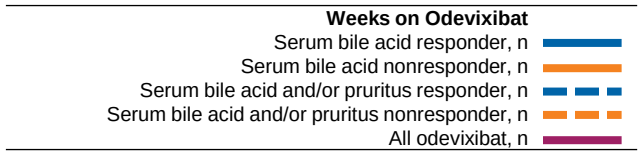



**Needing Soothing**




**Sleeping With Caregiver**



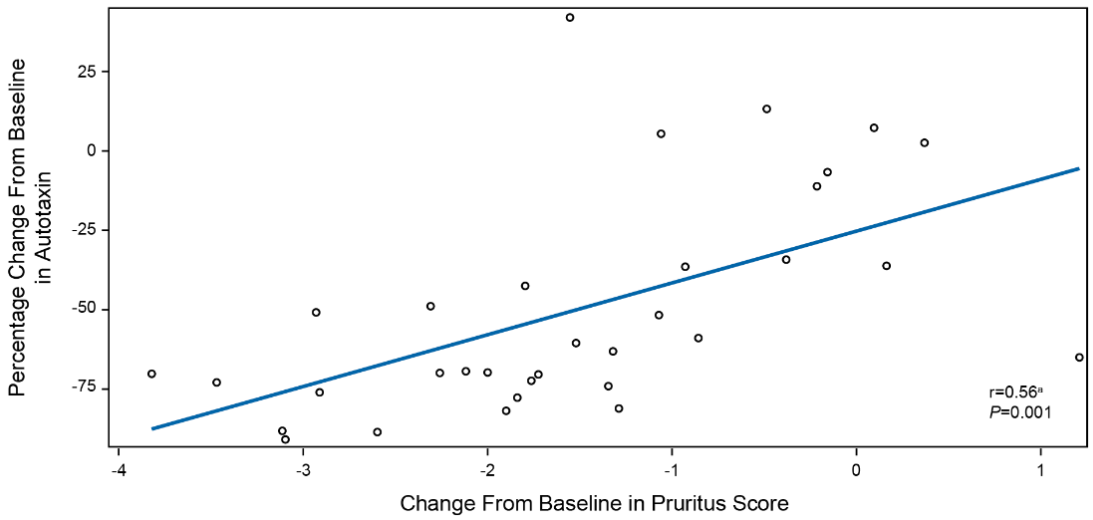




This pooled analysis covers the period from the first-ever dose of odevixibat in PEDFIC 1 or PEDFIC 2 through December 4, 2020. SE, standard error.

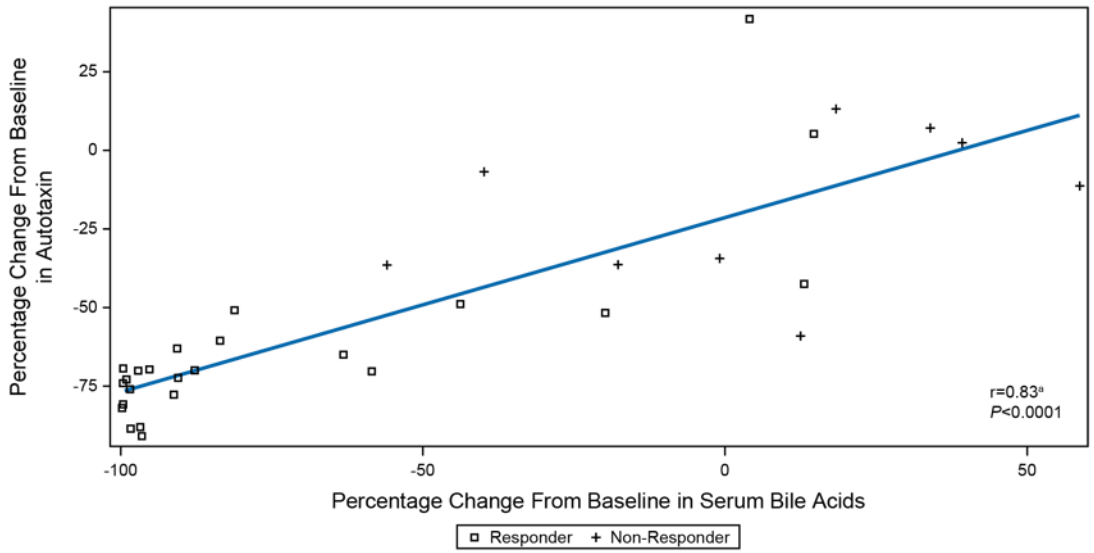
McKiernan P, et al. Poster Presentation at AASLD: The Liver Meeting 2021, American Association for the Study of Liver Diseases; November 12-15, 2021.

# Significant Correlations Observed Between Reductions in Pruritus and Biomarkers of Autotaxin and Serum Bile Acids with Bylvay™ (odevixibat)

**Percentage Change in Autotaxin vs Change in Pruritus**




**Percentage Change in Autotaxin vs Percentage Change in Serum Bile Acids**




- Results of this pooled analysis show that Bylvay reduced autotaxin (-50%), pruritus (-1.4) and sBAs (-49)
- Autotaxin can be a marker of liver injury in cholestatic patients and elevated levels have been also associated with increased pruritus

This pooled analysis covers the period from the first-ever dose of odevixibat in PEDFIC 1 or PEDFIC 2 through the PEDFIC 2 interim data cut (July 15, 2020). [a]n=33. Responder defined as ≥70% reduction from baseline in serum bile acids or serum bile acids ≤70 µmol/L at last assessment up to week 48 and/or pruritus score reduction of ≥1 point from baseline based on last available monthly or 12-week interval score up to week 48. r, Pearson coefficient.

Gonzalès E, et al. Oral Presentation at AASLD: The Liver Meeting 2021, American Association for the Study of Liver Diseases; November 12-15, 2021.

# Reductions in sBAs and Improvements in Pruritus, Growth and Sleep Parameters Across PFIC Types

High percentage of patients with PFIC3 and PFIC6 experienced clinical benefits of Bylvay™ (odevixibat).
>1 point decrease deemed clinically relevant.

| | 24 weeks | | 54 weeks | |
|---|---|---|---|---|
| | PFIC1 N = 12 | PFIC2 N = 30 | PFIC3 N = 5 | PFIC6 N = 1 |
| **Patients with improved pruritus score** | 95% | 80% | 80% | 100% |
| **Pruritus mean reduction vs. baseline (points)[a]** | -1.13 | -1.13 | -1.6 | -1.8 |
| **sBA mean reduction vs. baseline** | -31.7 μmol/L | -120.8 μmol/L | -91 μmol/L | -78 μmol/L |

- Bylvay was generally well tolerated
- Most TEAEs were mild to moderate in severity, there were no serious TEAEs, discontinuation or death

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Reduction from baseline pruritus score (0 to 4 point scale)
This analysis includes PEDFIC 2 data up to the December 4, 2020 cutoff. aMean change to weeks 34–36. ALT, alanine aminotransferase; MDR3, multidrug resistance protein 3; MYO5B, myosin 5B; PFIC, progressive familial intrahepatic cholestasis. Özen H, et al. Poster Presentation at AASLD: The Liver Meeting 2021, American Association for the Study of Liver Diseases; November 12-15, 2021.

# Observed Safety Profile

**The observed safety and tolerability** profile of Bylvay™ (odevixibat) was consistent across studies, treatment groups and doses, regardless of PFIC classification or BSEP subtype.

**No drug-related** serious adverse events were reported in either PEDFIC 1 or PEDFIC 2.

**The diarrhea rate** in the pooled data set over 72 weeks was 20% in the Bylvay-treated patients compared to 10% in the placebo-treated patients in PEDFIC 1 over 24 weeks.*

| Safety in Pooled Data Analysis<br>Patients, n (%) | All Odevixibat N=84 |
|---|---|
| Any TEAEs* | 71 (85) |
| Mild | 30 (36) |
| Moderate | 33 (39) |
| Severe | 8 (10) |
| TEAEs leading to discontinuation | 5 (6) |
| Drug-related TEAEs | 35 (42) |
| Serious TEAEs | 9 (11) |

*TEAEs of interest in greater than 10%: diarrhea, increased blood bilirubin, increased ALT, increased INR
For additional Important Safety Information on Bylvay, see full Prescribing Information at Bylvay.com

BYLVAY™ (odevixibat) [package insert]. Boston, MA: Albireo; July 2021.

# PEDFIC Results Provide Confidence in Ongoing Phase 3 Alagille Syndrome and Biliary Atresia Studies

## Significant read-through from PEDFIC to other Phase 3 studies

| Parameter | PEDFIC Studies (PFIC) | ASSERT Study (Alagille Syndrome) | BOLD Study (Biliary Atresia) |
|---|---|---|---|
| Bile acid lowering demonstrated | As high as 98% | √√√ | √√√ |
| Effective dose | 40 and 120 mcg/kg effective | √√√ | √√√ |
| Low treatment-related diarrhea rate | 9.5% vs 5.0% placebo | √√√ | √√√ |
| Validation of pruritus measurement tool | Determined that 1 point drop significant | √√√ | N/A |

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# A3907

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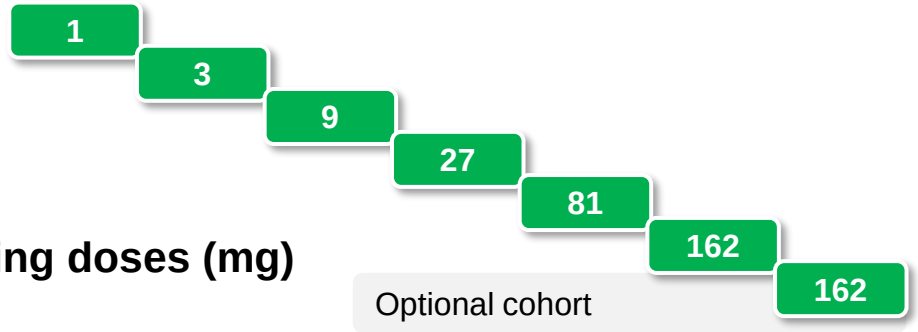
# A3907 Phase 1 Study Design

## Phase 1 SAD/MAD




**Single ascending doses (mg)**
**Active + Placebo 6+2**


**7d multiple ascending doses (mg)**
**Active + Placebo 6+2**

**Endpoints**

**Primary:** Tolerability, Safety PK

**Exploratory:** FGF19, LDLc, C4, bile acids in serum and urine

# Market Opportunity & Key Milestones

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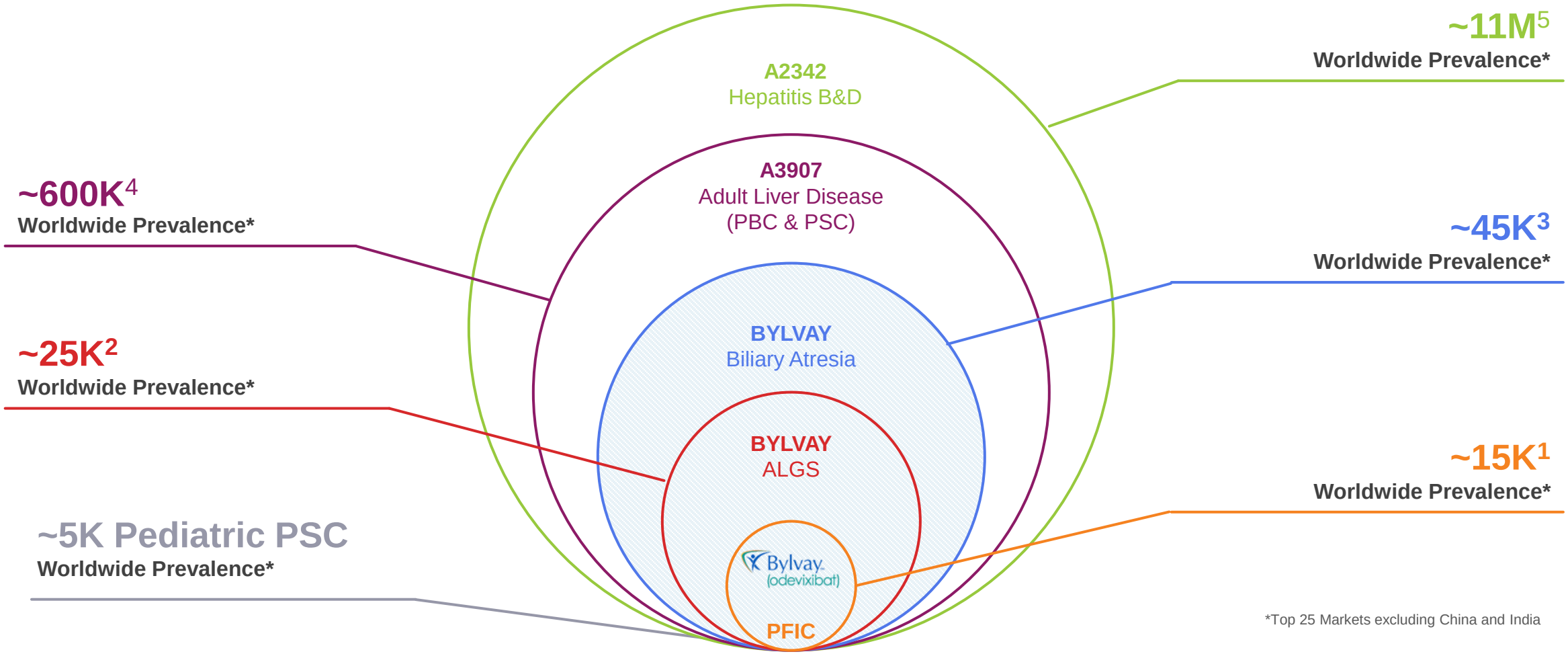
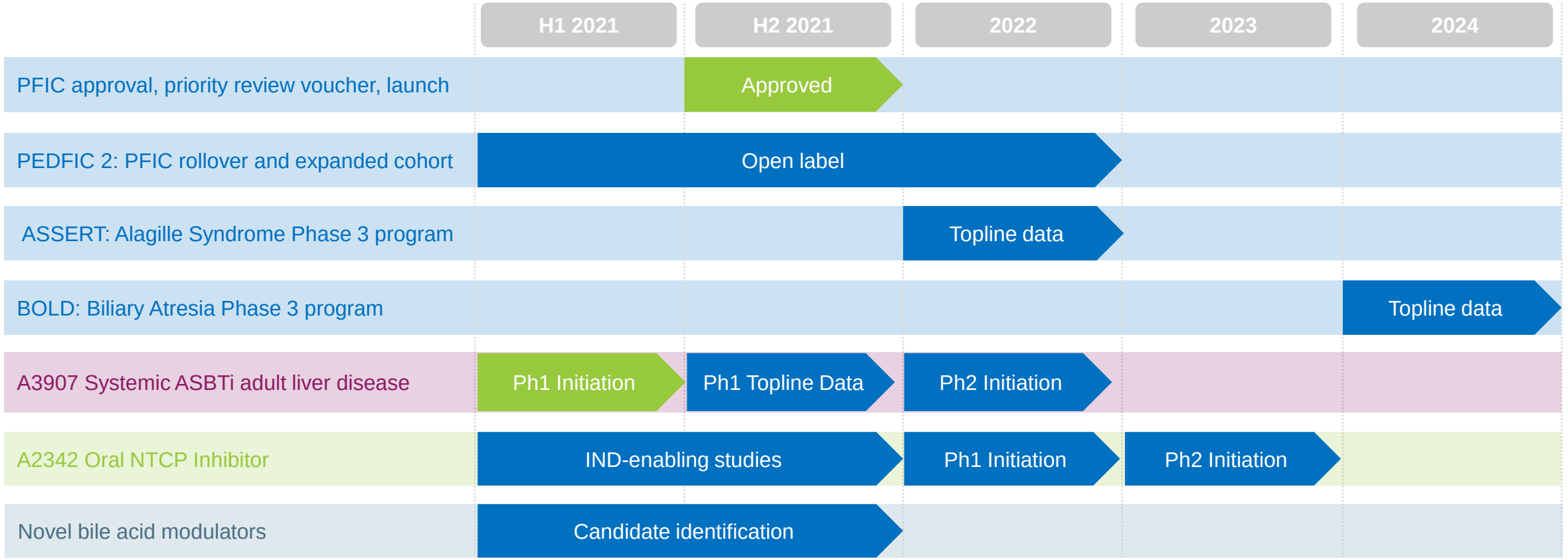
# Albireo Significant Growth Opportunity




*Top 25 Markets excluding China and India

1. Jacquemin E; Progressive Familial Intrahepatic Cholestasis; Clin Res Hepatol Gastroenterol. 2012 Sep;36 Suppl 1:S26-35; Pawlikowska L, et al. Differences in presentation and progression between severe FIC1 and BSEP deficiencies. Journal of Hepatology. 2010;53(1):170-178; Jain A, et al. Long-Term Survival After Liver Transplantation in 4,000 Consecutive Patients at a Single Center. Annals of Surgery. 2000; Vol. 232, No. 4, 490-500; 2. Leonard, et al. Clinical utility gene card for: Alagille Syndrome. European Journal of Human Genetics. 2014; 22; Vandriel S, et al. Clinical features and natural history of 1154 Alagille Syndrome patients: results from the international multicenter GALA study group. Journal of Hepatology. 2020;vol. 73; S401-S652; Kamath, et al. Outcomes of Childhood Cholestasis in Alagille Syndrome. Hepatology Communications; 2020; vol. 4; no 3; 3.Hopkins PC et al. Incidence of Biliary Atresia and Timing of Hepatoportoenterostomy in the United States. J Pediatr. 2017 Aug;187:253-257; Verkade H et al. Biliary atresia and other cholestatic childhood diseases: Advances and future challenges. J. of Hepatology 2016;65:631-642 4. PSC: Gochunaur; Clinical Liver Disease; 2020; Tabibian; Gastroenterology and Hepatology; 2018; Tanaka; Hepatology Research; 2019, PBC: Lu; Clinical Gastroenterology and Hepatology; 2018; Marschall; Scientific Reports; 2019; Kumagi; Orphanet; 2008. 5. HBV and HDV: Data on file; Delveinsight Chronic Hepatitis B Virus and Hepatitis D Virus Reports 2020.

# Sustained Growth Through Multiple Catalysts




IBATi = ileal bile acid transport inhibitor, ASBT = apical sodium-dependent bile acid transporter, NTCPi = sodium-taurocholate co-transporting peptide inhibitor

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# Hope for Families with Liver Diseases

November 16, 2021

(Nasdaq: ALBO)

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