



# Corporate Presentation

Corporate Access and Biotech Showcase during JP Morgan Healthcare Conference  
*January 8 – 12, 2023*

# Disclaimer & Looking Forward Statement

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# About GENFIT

## Mission & Expertise

- **Late-stage biopharmaceutical company** dedicated to improving the lives of patients with liver diseases characterized by high unmet medical needs
- **20+ years of expertise** from discovery phase to late-stage development
- **Strong track record** to develop long term collaboration: Ipsen, Genoscience Pharma, Labcorp, Terns Pharmaceuticals

## Pipeline

**Expanded pipeline of innovative assets**, comprising 6 independent programs with diversified mechanisms of action in 6 key therapeutic areas, and 2 diagnostics programs:

- **1 Phase III readout in 2023**
- **3 programs in Phase II in 2023**
- **2 preclinical programs**
- **2 diagnostic programs**

## Financials

- In 2021, **IPSEN** became one of GENFIT's largest shareholders, acquiring 8% of its share capital
- Cash position: **€163.6M** as of Sept 30, 2022



**150+ employees**



**700+ patents  
& patents application**

Fully committed in the continuous improvement  
of our **CSR & ESG Performance<sup>1</sup>**



**PAQTE**



**Euronext & NASDAQ  
listed: GNFT**



**Based in Lille, Paris,  
Zurich & Cambridge, MA**

# A new GENFIT, following execution of the 2021-2022 strategic plan

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## Leveraging GENFIT's strengths and experience in liver diseases...

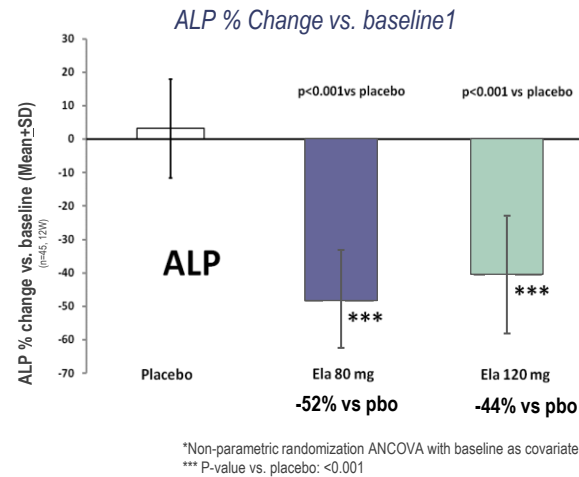
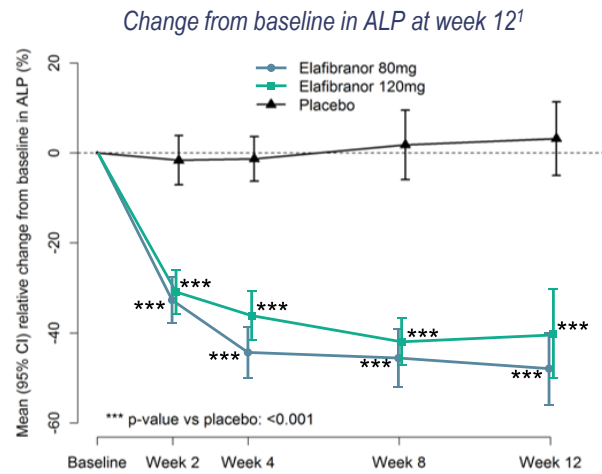
- *in Research*
- *in Clinical development*
- *in Regulatory affairs*
- *in Pre-commercialization*

**... to address the high unmet medical needs in several liver indications**



# Elafibranor as a Potential Treatment for PBC (1/3) – Positive Phase 2 data

Statistically significant treatment effects with both 80mg and 120mg doses on the primary end-point\* of serum alkaline phosphatase (ALP) change from baseline



Elafibranor is a competitive 2L candidate for PBC

Elafibranor*2 Phase 2a Week 12 Data NCT03124108 EudraCT2016-003817-80		
	80mg (N=15)	Placebo (N=14)
<b>Composite endpoint</b> % responders, ALP<1.67 x ULN; Bili<ULN and ALP reduction >15%	<b>67%</b> (p<0.001)	6.7%
<b>Alkaline phosphatase</b> (% change vs baseline)	<b>-48%</b> (p<0.001)	3%
Ocaliva™3, Phase 3 POISE Month 12 Data NCT01473524		
	10mg (N=73)	Placebo (N=72)
<b>Composite endpoint</b> % responders, ALP<1.67 x ULN; Bili<ULN and ALP reduction >15%	<b>47%</b> (p<0.001)	10%
<b>Alkaline phosphatase</b> (% change vs baseline)	<b>~-36%**</b> (p<0.001)	~-4%**

**Note:** Indirect Comparison of Selected Biochemical Endpoint<sup>1</sup>. Both studies were add-on investigational therapy to UDCA or monotherapy in patients unable to tolerate UDCA. 2L: Second-line. \***Elafibranor** – mITT: All subjects w/ available baseline value and at least one post baseline value under treatment for ALP. \*\*These are estimations-based figures as reported data is based on actual change from Baseline n ALP (U/L). Elafibranor is an investigational compound and has not been approved by any regulatory authority in any indication. Obeticholic acid is registered in US and EU under the trade name OCALIVA®, please refer to the approved PI and SmPC.

1. Data from referenced clinical trials; 2. Schattenberg et al. J. of Hepatol. 2021, Vol. 74, Issue 6:1344-1354; 3. Nevens, et al. NEJM 2016, 375(7):631-43.

Elafibranor awarded Breakthrough Therapy designation by the FDA and Orphan Drug Designation by the FDA & EMA for PBC<sup>2</sup>

**JOURNAL OF HEPATOLOGY**  
The Home of Liver Research

*A randomized placebo-controlled trial of elafibranor in patients with primary biliary cholangitis and incomplete response to UDCA<sup>1</sup>*

Jörn Schattenberg et al. | Journal of Hepatology. Feb. 2021

**Note:**\* confirmed in mITT\* set. mITT (All subjects w/ available baseline value and at least one post baseline value under treatment for ALP)=Placebo (N=15), Elafibranor 80mg (N=15), Elafibranor 120mg (N=14). Per Protocol Set = Placebo (N=14), Elafibranor 80mg (N=14), Elafibranor 120mg (N=13). ITT (intend to treat) = Placebo (N=15), Elafibranor 80mg (N=14), Elafibranor 120mg (N=15). 1. Schattenberg et al. J. of Hepatol. 2021, Vol. 74, Issue 6:1344-1354; 2. GENFIT Corporate Press Release June 29, 2019 "GENFIT Announces FDA Grant of Breakthrough Therapy Designation to Elafibranor for the Treatment of PBC.".

# Elafibranor as a Potential Treatment for PBC (2/3) – Commercial partnership with Ipsen



## Terms of the deal

- *€120M upfront payment*
- *Up to €360M in milestone payments*
- *Tiered double-digit royalties of up to 20%*
- *8% shareholder of GENFIT via an equity investment of €28M with premium*
- *Ipsen will assume responsibility for all additional clinical development, including completion of the long-term extension period of the ELATIVE™ trial, and global\* commercialization*

## Addressable market for second line post UDCA<sup>1</sup>

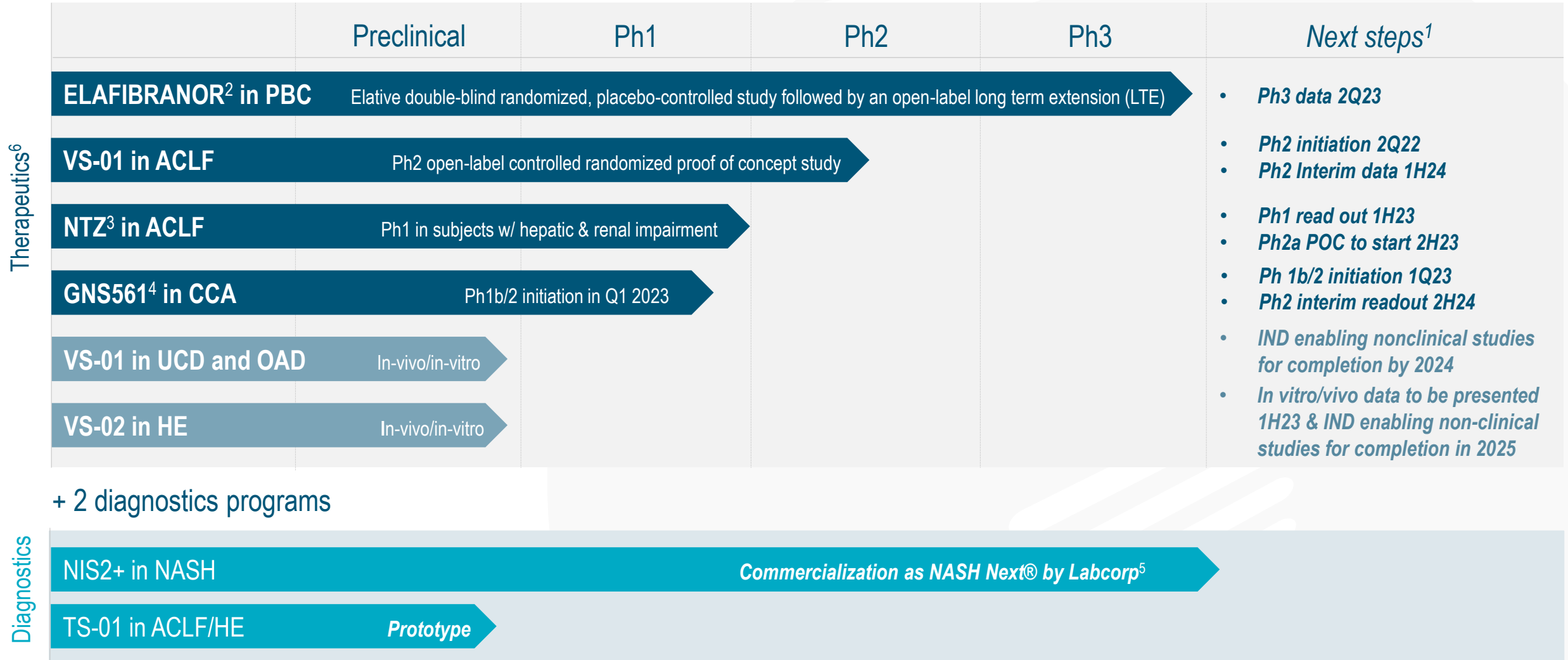
### Ballpark overall market size by 2030

- **\$2.3bn** US
- **\$0.8bn** EU
- **\$3.1bn** total

### Main assumptions

- Prevalence: **52k** (EU5) and **54k** (US) for 40% of patients moving into 2L
- Drug gross price ranges per year: **~\$30k** in EU5 in 2022 and **~\$84k** in US expected to slightly evolve as competition will arise in second line

# Today, 6 indications with high unmet medical need across 6 programs (4 clinical, 2 preclinical), with frequent milestones reporting expected in the coming three years



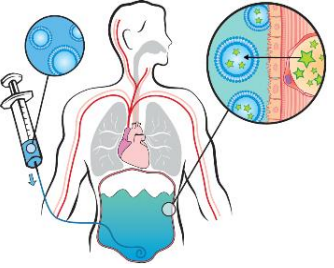

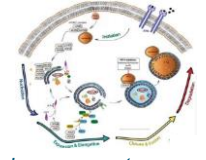
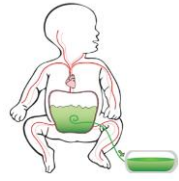
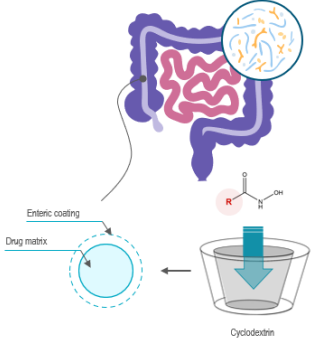
1. Reflects management's anticipated timelines, which are subject to change  
 2. Out-licensed to [Terns Pharmaceuticals](#) and [Ipsen Pharmaceuticals](#)  
 3. Repositioned molecule

4. In-licensed from [Genoscience Pharma](#)  
 5. [www.labcorp.com/tests/504960/nashnext](http://www.labcorp.com/tests/504960/nashnext)

6. All drugs under development are investigational compounds that have not been reviewed nor been approved by a regulatory authority in targeted indications



# Our therapeutic programs at a glance – MoAs and supporting evidence for further development

<p><b>ELAFIBRANOR</b> in PBC</p> <p><b>Dual agonist</b> of PPAR<math>\alpha/\delta</math></p>	<p><b>VS-01</b> in ACLF</p> <p><b>First-in-class</b> liposomal-based technology</p>	<p><b>NTZ</b> in ACLF</p> <p><b>Antibiotic and</b> anti-inflammatory activities</p>	<p><b>GNS561</b> in CCA</p> <p><b>Small molecule</b> PPT1 inhibitor</p>	<p><b>VS-01</b> in UCD &amp; OAD</p> <p><b>Potential first-</b> line peritoneal route treatment</p>	<p><b>VS-02</b> in HE</p> <p><b>Urease</b> inhibitor</p>
<p><b>PHASE 2 data</b></p> <p><b>JOURNAL OF HEPATOLOGY</b> <small>The Home of Liver Research</small></p> <ul style="list-style-type: none"> <li>A randomized placebo-controlled trial of elafibanor in patients with primary biliary cholangitis and incomplete response to UDCA (NCT03124108)</li> </ul> <p><b>IPSEN</b> <small>Innovation for patient care</small></p> <ul style="list-style-type: none"> <li>In December 2021, Ipsen and GENFIT entered into exclusive licensing agreement for elafibanor</li> </ul>	<p><b>PHASE 1b data</b></p>  <ul style="list-style-type: none"> <li>Impact on overall liver disease severity</li> <li>Dose-dependent ammonia removal from the body</li> <li>Improvement in psychometric tests</li> <li>Reduction of ACLF metabolites</li> <li>Reduction of infection-related metabolites</li> </ul>	<p><b>Preclinical data</b></p>  <ul style="list-style-type: none"> <li>Reduces LPS-induced inflammation in healthy rats</li> <li>Beneficial effects on liver function markers (bil, alb) in models of cirrhosis</li> <li>Reduces brain edema in models of ACLF (BDL)</li> <li>Reduces inflammation markers in models of ACLF (BDL)</li> <li>Improves survival in treatment models of Sepsis (CLP)</li> </ul>	<p><b>PHASE 1b data</b></p> <ul style="list-style-type: none"> <li>Antitumor activity in human cell lines (HCC, iCCA*)</li> <li>Decreases tumor number and size in transgenic HCC mouse model</li> <li>First-in-human effects of PPT1 inhibition using GNS561+ Ezurpimtrostat in patients with primary/secondary liver cancers<sup>1</sup></li> </ul>  <ul style="list-style-type: none"> <li>Autophagy promotes cancer cell survival, tumor growth and treatment resistance</li> <li>Combination of GNS561 w/ MEK inhibitor may block this survival by inhibiting late-stage autophagy</li> </ul>	<p><b>Proof of concept</b></p>  <ul style="list-style-type: none"> <li>Potential first-line treatment for acute hyperammonemic crises</li> <li>Fast implementation – shorter lead time vs. SOC</li> <li>Gentle as less hemodynamic disturbances and no vascular access damage</li> <li>Administered outside the dialysis and intensive care units</li> <li>Ease of administration to children, allowing broader access to peripheral hospitals</li> </ul>	<p><b>Proof of concept</b></p>  <ul style="list-style-type: none"> <li>Urease inhibitory activity in vitro over +15 screened hydroxamic acid derivatives</li> <li>Synthesis of lead candidate optimized and straightforward</li> </ul>



# Genfit JPM presentation

*IQVIA perspective on the commercial opportunity of GENFIT's pipeline*

January 2023

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# While keeping its footprint in hepatology, GENFIT is now moving to a diversified portfolio covering multiple rare liver related diseases with high unmet needs

## Urea Cycle Disorders (UCD)

UCDs are a set of **rare inherited metabolic conditions** in which there is a **full or partial deficiency** in the enzymes of the **urea cycle**, causing a defect in the metabolism of excess nitrogen, and leading to **hyperammonemia**.

## Organic Acidemia Disorders (OAD)

OADS are a spectrum of **rare inherited disorders** characterized by **enzymatic defects** in metabolism of amino-acids or some fatty acids leading to **toxic, and potentially life-threatening accumulation** of by-products

## Hepatic Encephalopathy (HE)

HE is **deterioration in brain** function when liver is unable to adequately remove **toxins** from the blood. It is often associated with **cirrhosis** and potentially **fatal**

Rare liver induced diseases

Cholestatic diseases

from NASH to 6 diseases

Acute events on chronic liver diseases and liver cirrhosis

## Cholangiocarcinoma (CCA)

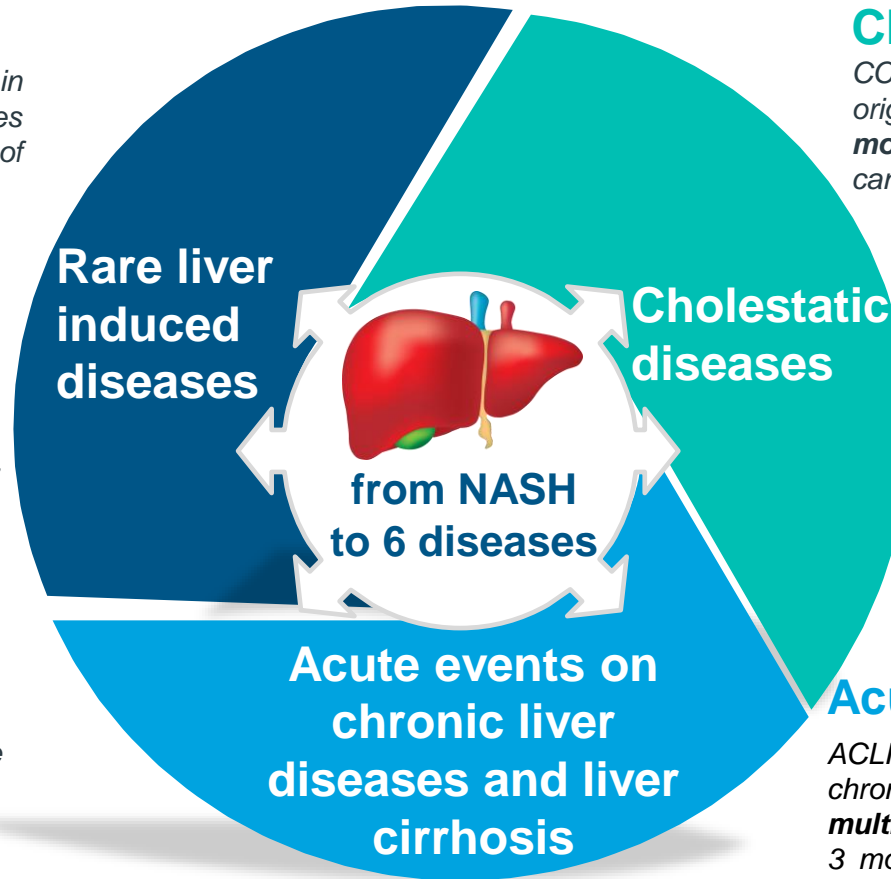
CCA are malignancies of the biliary duct system that may originate in the liver or extrahepatic bile ducts . It is the **second most common liver cancer**, accounting for 10-20% of all liver cancers

## Primary Biliary Cholangitis (PBC)

Primary biliary cholangitis (PBC) is **chronic and progressive cholestatic disease** of the liver. It is a rare autoimmune disease that can lead to **cirrhosis** if untreated

## Acute on Chronic Liver Failure (ACLF)

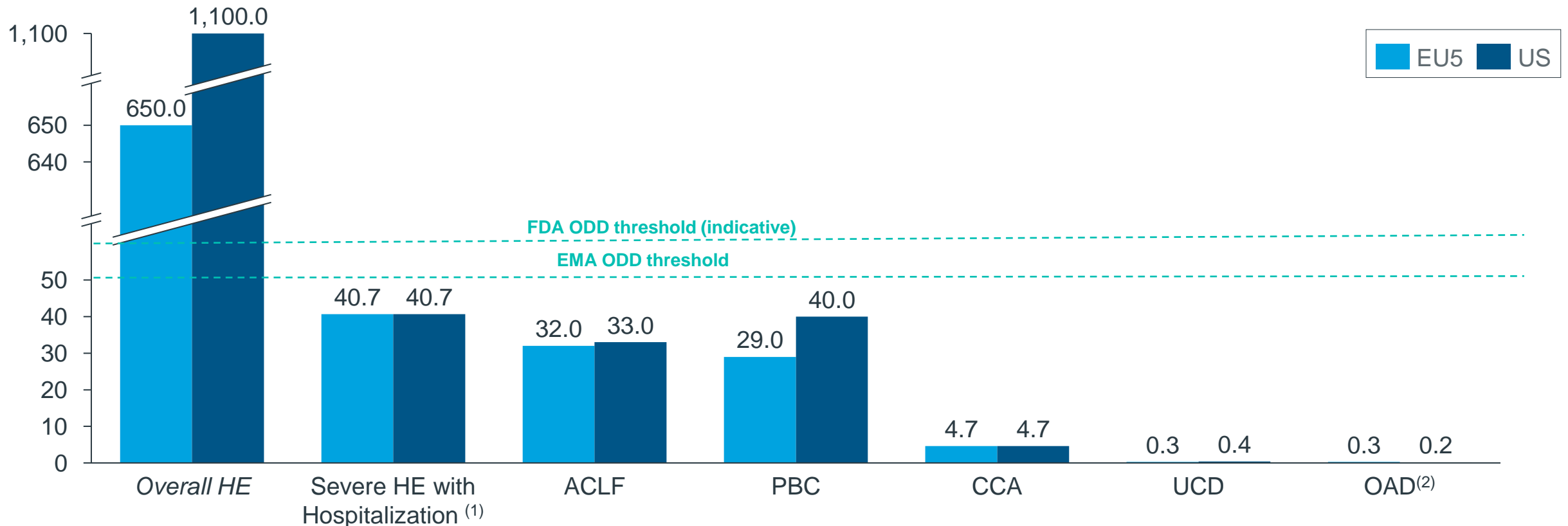
ACLF is **acute and life-threatening** condition in patients with chronic liver disease with or without cirrhosis that may progress into **multiple organ failure** with associated **high risk of mortality** within 3 months if not treated. However, it is potentially reversible with treatment



The recent Versantis investment is transformative, creating a sustainable platform for future therapies in liver and related disorders. GENFIT's know-how and expertise in physiopathology of liver failure and dysfunction will be the driving force in this success

# The six pursued diseases have low epidemiology and could potentially be eligible for orphan designation

Estimated current prevalence (1:100,000)



- **PBC:** Elafibranor has been granted orphan designation and breakthrough therapy designation
- **CCA:** Pemigatinib, Infigratinib and Futibatinib (FGFR2 mutation) have had accelerated approval from FDA. GNS561 granted ODD
- **UCD:** DTX301 and Pegzilarginase have been granted ODD
- **OAD:** HST-5040 granted FDA Orphan Drug, Fast Track and Rare Pediatric Disease designations for the treatment of MMA<sup>(2)</sup> and PA<sup>(3)</sup>

Note <sup>(1)</sup> Defined as the number of cirrhosis patients with HE events leading to hospitalization per year <sup>(2)</sup> 1:100,000 newborn (<18 years old) Source: Robert S. Rahimi, MD Et Al. AJM, <sup>(3)</sup> methylmalonic acidemia; <sup>(3)</sup> PA - propionic acidemia  
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# All 6 diseases have a high impact on patients' lives and high unmet needs

	Burden of disease	Unmet needs
HE	<ul style="list-style-type: none"> <li>Potentially <b>life-threatening condition</b></li> <li>Significant impairments in <b>multiple health-related quality of life domains</b> (sleep disturbances, functional impairments)</li> </ul>	<ul style="list-style-type: none"> <li>Current approved HE treatments are associated with <b>significant side effects and low compliance</b></li> </ul>
ACLF	<ul style="list-style-type: none"> <li><b>Mortality rate at 90 days of 50% in UE5<sup>1</sup> and 490% in US<sup>2</sup></b></li> <li>High cost per hospitalization of 50k US\$</li> </ul>	<ul style="list-style-type: none"> <li><b>No approved treatments for ACLF</b></li> </ul>
PBC	<ul style="list-style-type: none"> <li>After development of symptoms (cholestasis), and without treatment, <b>survival duration ranges from 5 to 12 years</b></li> <li>Associated with symptoms that impair quality of life such as fatigue, emotional dysfunction, or pruritus (itching)</li> </ul>	<ul style="list-style-type: none"> <li>UDCA in first line (40% suboptimal response). <b>Only OCA in second line with contraindications</b></li> </ul>
CCA	<ul style="list-style-type: none"> <li>The prognosis is poor, with <b>median survival of ~6 months</b> in unresectable advanced CCA patients</li> </ul>	<ul style="list-style-type: none"> <li>Despite increasing targeted therapies (e.g.: FGFR2, IDH1), <b>many patients with advanced CCA do not initiate therapy after chemo due to lack of efficacy</b></li> </ul>
OAD	<ul style="list-style-type: none"> <li>Children are at constant risk of having episodes of <b>decompensation and encephalopathy throughout lives and life-threatening symptoms</b></li> <li><b>Newborns who do not receive treatment are at risk of death</b></li> </ul>	<ul style="list-style-type: none"> <li><b>No current approved therapy</b></li> </ul>
UCD	<ul style="list-style-type: none"> <li>Symptoms like lethargy, abnormal motor function, which precedes first hyperammonemia are associated with <b>reduction of patient's QoL</b></li> <li><b>5y Mortality rate in neonatal onset UCD cases was 24%<sup>3</sup></b></li> </ul>	<ul style="list-style-type: none"> <li>Current approved UCDs treatments are <b>not effective or not approved for acute hyperammonemia</b></li> </ul>

1- Acute-on-Chronic Liver Failure Is a Distinct Syndrome That Develops in Patients With Acute Decompensation of Cirrhosis | Gastroenterology 2013;144:1426 –1437; 2- Prevalence and short-term mortality of acute-on-chronic liver failure: A national cohort study from the USA - PubMed (nih.gov); 3- Gillian Yeowell et al. The burden of pharmacological treatment on health-related quality of life in people with a urea cycle disorder: a qualitative study | Springer Open, Journal of Patient-Reported Outcomes 4- A longitudinal study of urea cycle disorders | ScienceDirect - GENFIT - Corporate Access and Biotech Showcase during JP Morgan Healthcare Conference - January 2023



# GENFIT has a well-balanced portfolio across disease areas with limited treatment options and lower development costs(1)

## Portfolio



- **Diversified portfolio** with multiple assets and modes of action across various indications

## Diseases areas



- **Six liver-related diseases** most<sup>(2)</sup> of which are **advanced, life threatening**, with high unmet needs
- **Easy diagnosis** with standard tests

## Clinical development



- **Smaller trials** (in comparison to NASH)
- **Short clinical development timelines**, leading to shorter time to inflection points

## Regulatory & reimbursement



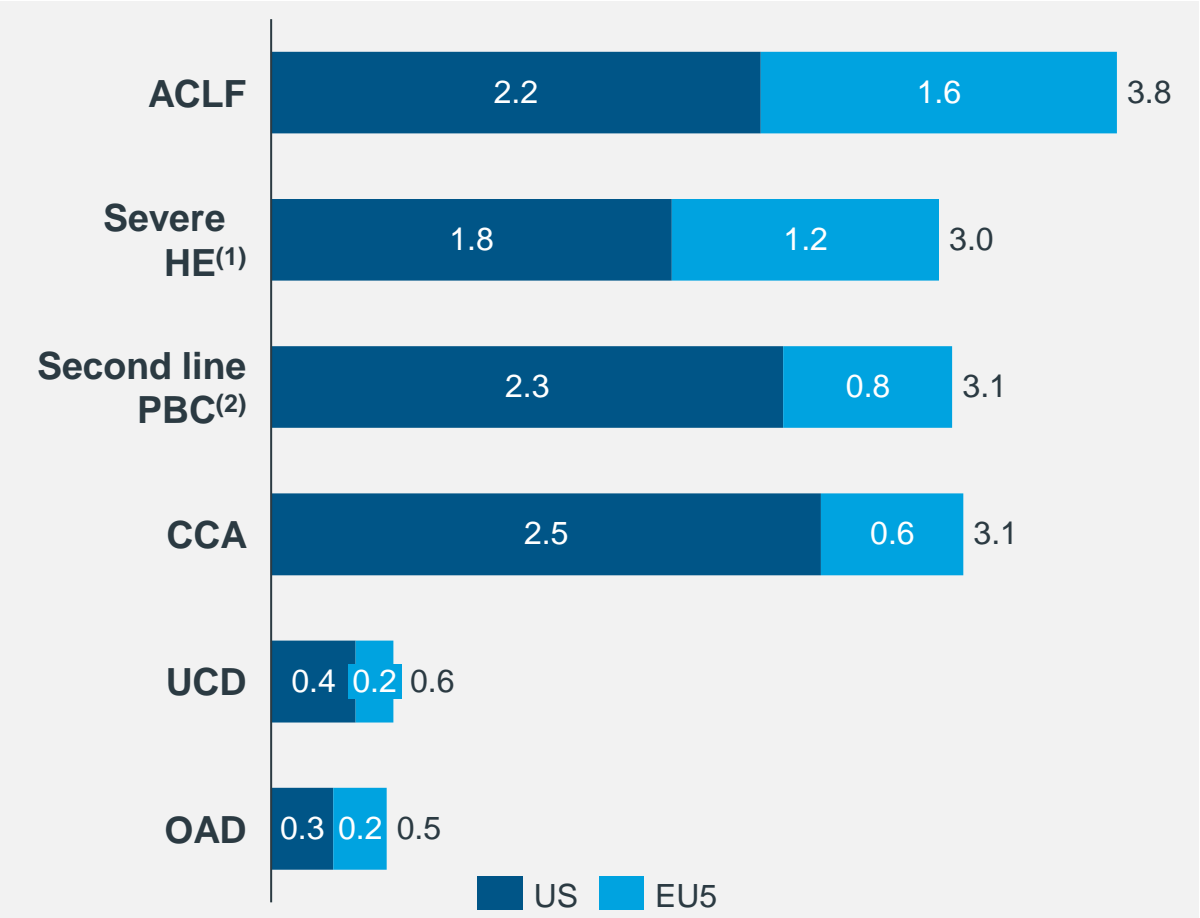
- **Potential orphan designation and accelerated regulatory pathway**
- Some are **pediatric indications** with high unmet need

**Moving from one asset in NASH into pipeline of assets across several diseases**

**Given high unmet needs and lower prevalence, the indications could benefit from accelerated regulatory pathways and lower development costs**

# These 6 diseases represent an overall ~14 bn USD market opportunity

## Ballpark overall market size by 2030, bnUSD



## Assumptions<sup>(3)</sup>

- Prevalence: 155k (EU5) / 80k (US) for grade 1 / 2 ACLF patients
- Drug price could amount to \$30-40k per patient in US and \$10-20k in EU based given the economic burden of hospitalizations
- Hospitalizations per year: 195k (EU5) / 200k (US)
- Drug price ranges: analogues in acute ICU costs would potentially range from \$15-20k in US and \$7-15k in EU5 based on economic burden of hospitalizations
- Prevalence: 52k (EU5) / 54k (US) for 40% of patients moving to 2L
- Drug gross price ranges per year: ~\$30k in EU5 in 2022 and ~\$84k in US expected to slightly evolve as competition will arise in second line
- Prevalence: 15k (EU5) / 15k (US)
- Drug price ranges per month: [\$500 – \$9k] in EU5 and [\$k – \$30k] in US
- Prevalence: 1k (EU5) / 1.3k (US)
- Drug price ranges per year: [\$500k - \$700k] in US and [\$300k - \$500k] in EU5
- Incidence in newborns: 129 (US), 198 (EU5)
- Drug price ranges per year: [\$96 – \$81k] in EU5 and [\$200 – \$300k] in US

(1) Only acute HE considered in estimations (2) Addressable market for second Line post UDCA (3) Estimation calculations include duration of treatment, potential eligibility to drug treatment, compliance rates based on analogues in rare diseases, gross-to-net price estimate depending on therapeutic area & disease  
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# Conclusion



## Novel mechanisms of action

- **Elafibranor**: only asset targeting both PPAR $\alpha/\delta$  receptors for PBC
- **VS-01**: First-in-class liposomal-based technology
- **VS-02**: novel urease inhibitor bringing a unique oral and colon active formulation for HE
- **GNS561**: novel MoA with autophagy inhibition for CCA



## Potential accelerated access

- **Orphan drug designation** granted for elafibranor (FDA/EMA), VS-01 (FDA) and GNS561 (FDA)
- **Breakthrough therapy designation** (Elafibranor)
- **Rare pediatric disease designation** (VS-01)
- Potential **priority review voucher** (VS-01)



## Sizable commercial opportunity

- **~14 bn USD** cumulative market across all disease areas
- Limited competitive intensity in OAD, UCD and ACLF



# Questions?