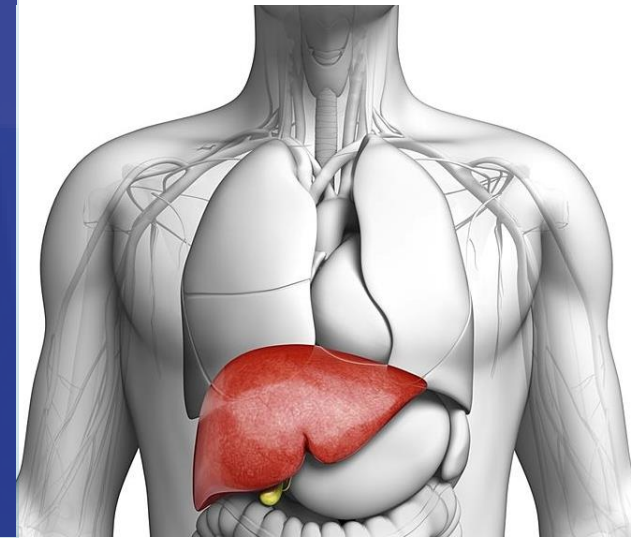


ADVANCING
CARDIOVASCULAR
AND
NASH
OPPORTUNITIES



PEDIATRIC NAFLD/NASH PRESENTATION

January 2018

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Agenda

Pediatric NAFLD/NASH Epidemiology

Key Features for Pediatric NAFLD/NASH Success

Rationale for Gemcabene in Pediatric NAFLD/NASH

Pediatric NAFLD Phase 2a Study Design

U.S. Market Assumptions for NAFLD/NASH

Q&A

Pediatric NAFLD/NASH – A Growing Epidemic

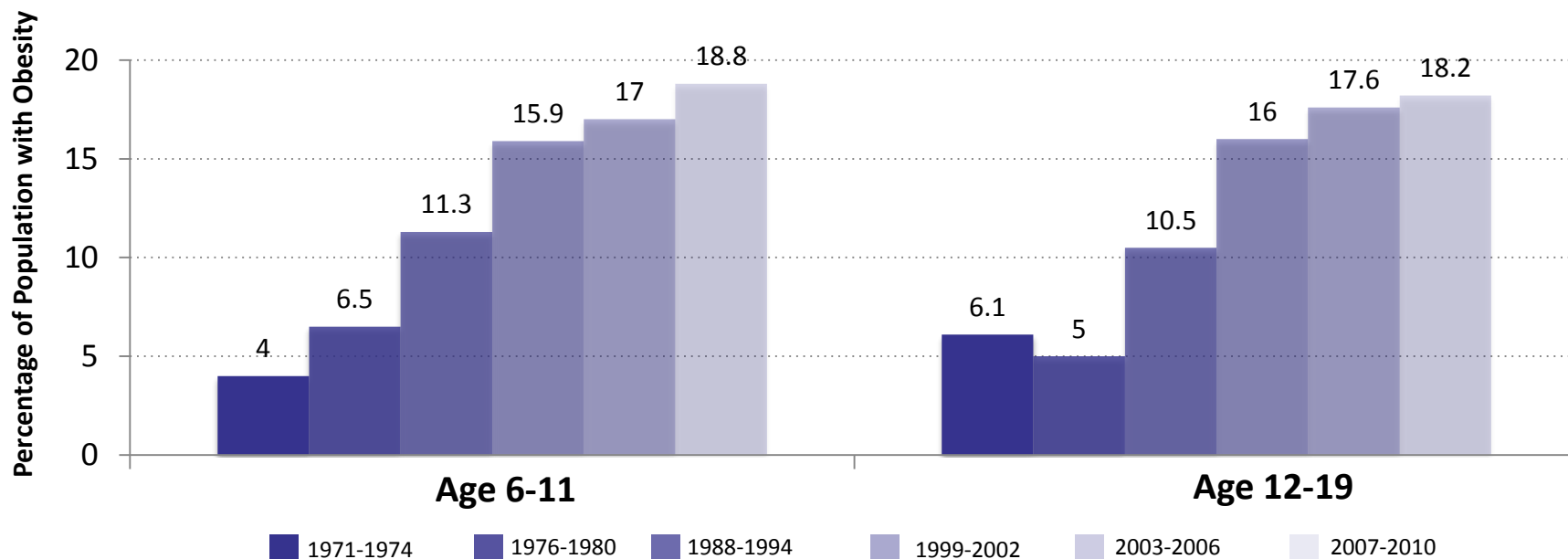
Gemcabene's MOA Targets Many Underlying Pathologies of NAFLD/NASH



- Non-alcoholic fatty liver disease (NAFLD) describes the **continuum of liver disease** from isolated steatosis through disease progression to non-alcoholic steatohepatitis (NASH)
- NASH is characterized by **excessive fat accumulation, inflammation, ballooning** and **fibrosis** in the liver
- NAFLD is estimated to affect **7M children** overall in the US; the estimated population prevalence for **Pediatric NASH is 2M children**
- 2017 estimates: 20% of children between ages 12-19 are obese, and 38% of obese children have NAFLD
- Gemcabene's impact on **LDL-C, TGs, hsCRP** and **improved glucose disposal rate** target known abnormalities of NAFLD/NASH patients
- **No approved therapies** to treat Pediatric NASH!

The Growing Obesity Epidemic in Children

Obesity is the Single Greatest Risk Factor for Pediatric NAFLD



24M

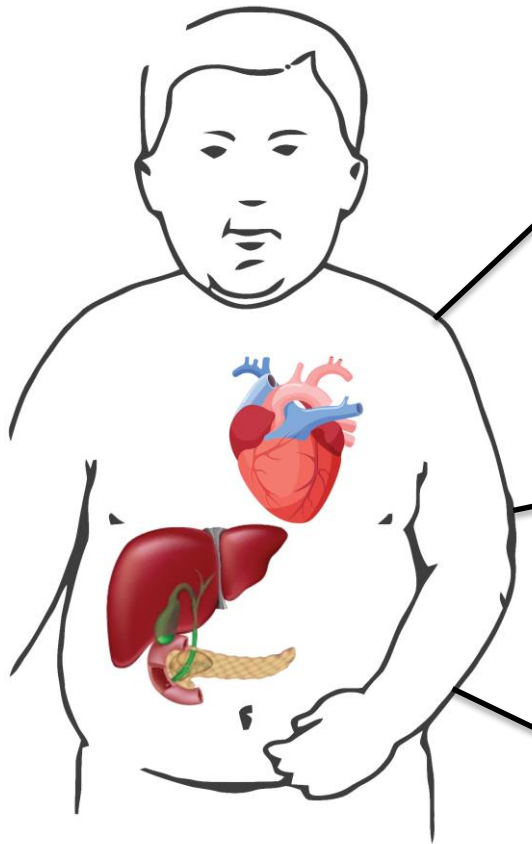
OVERWEIGHT OR
OBESE CHILDREN
(AGES 2-19)

13M

OBESE
CHILDREN
(AGES 2-19)

NHANES: 1971-1974, 1976-1980, 1988-1994, 1999-2002, 2003-2006 and 2007-2010; Data derived from Health, United States, 2011 (NCHS); AHA, 2013

Complications in Children with NAFLD/NASH



Dyslipidemia and Cardiovascular Disease

- Hypercholesterolemia, hypertriglyceridemia, low HDL
- Highly atherogenic lipid profile with more severe liver disease
- Left ventricular systolic and diastolic dysfunction
- Hypertension reported in about 20% - 30%

Cirrhosis / Hepatocellular Carcinoma

- 10% - 25% progress to advanced fibrosis/cirrhosis by 3rd-4th decade of life
- Pediatric NAFLD/NASH patients are more likely than adults to progress to decompensation (permanent liver damage)

Diabetes

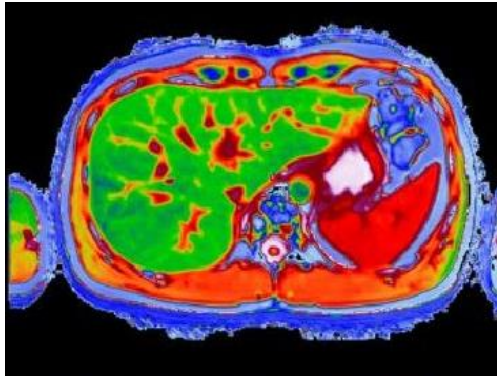
- An individual's risk of developing diabetes is increased approximately 5-fold if they have NAFLD
- Prevalence of prediabetes and diabetes are 23% and 6.5%, respectively

Pediatric NAFLD is associated with a 1,360% increase in mortality in the 20 years following diagnosis (13.6 SMR)

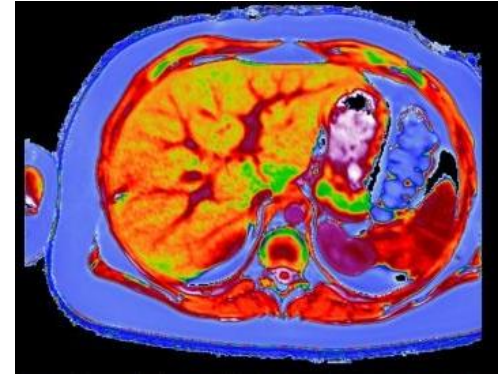
Selvakumar PKC, *Pediatr Clin North Am* 2017 Jun;64(3):659-675; *J Clin Gastroenterol* 2017; Hazlehurst J, *Metabolism*. 2016 Aug;65(8): 1096-1108.

Gemcabene is Focused on Treating the Earlier Stages of NAFLD/NASH

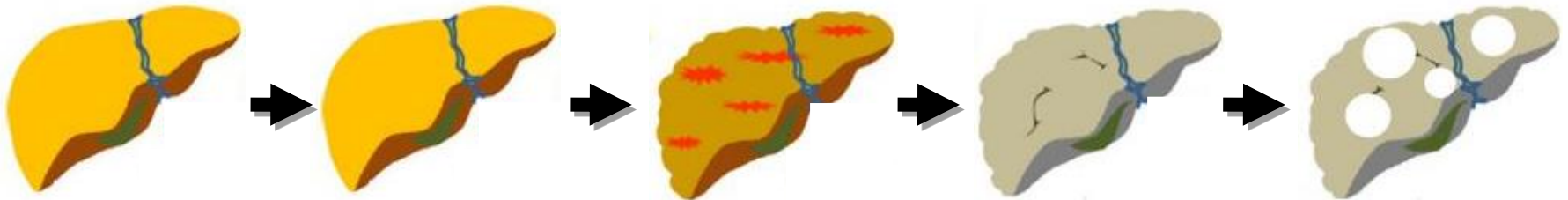
Healthy Liver



12 Year Old, Obese w/ NASH



Source: Berkot, B: New Market for liver disease spawns race for better testing, Reuters (2015)



NAFLD

NASH

Cirrhosis

Steatosis

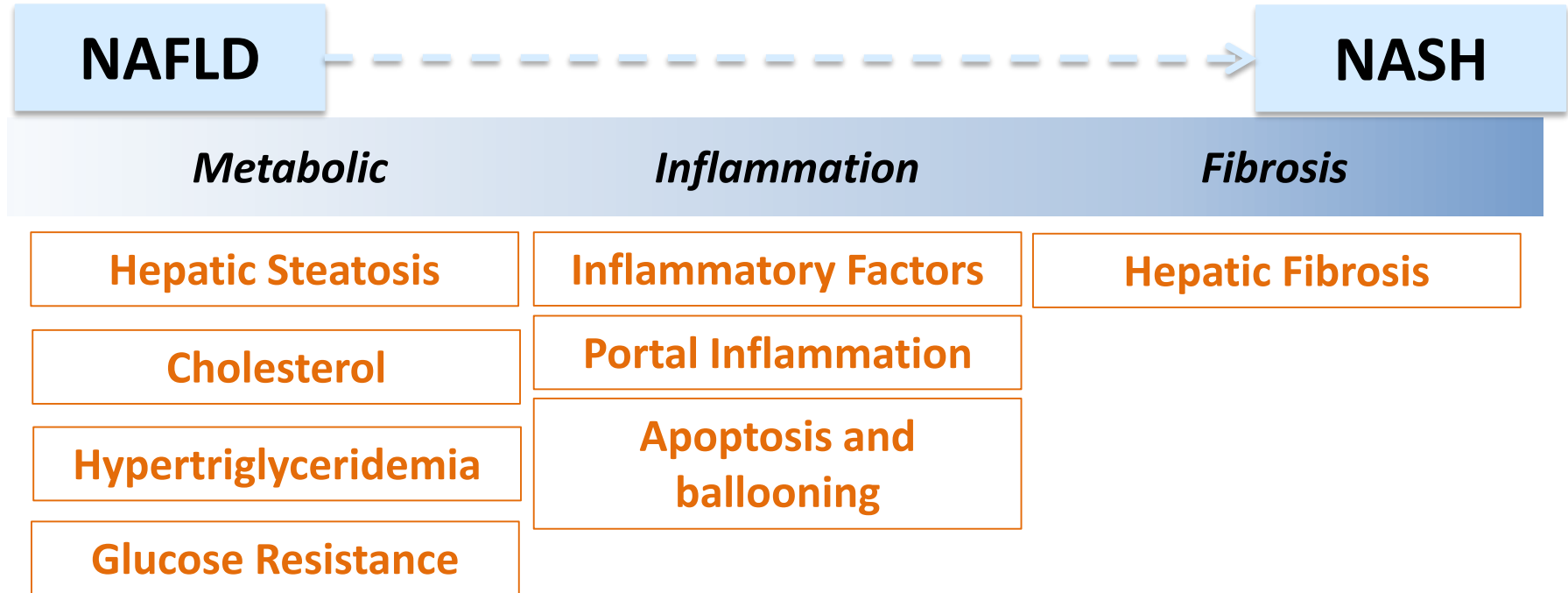
Steatosis +
Inflammation

Steatosis + Hepatocyte
Injury (Ballooning)

Steatosis +
Fibrosis

Gemcabene's Target Patients

The NAFLD/NASH Patient



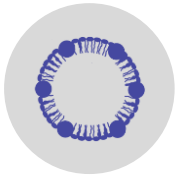
Gemcabene impacts all of the key underlying pathologies of NASH and has the potential to be a foundational therapy

Gemcabene: Key Features for Pediatric NAFLD/NASH Success



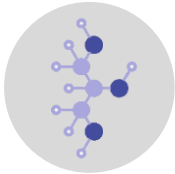
SAFETY

- Nearly 1,100 patients treated with gemcabene
- No muscle or liver toxicities in patients treated
- No drug interactions with statins or metformin



ATHEROGENIC PROFILE

- Significant LDL-C reduction as monotherapy and on top of statins
- Significant atherogenic burden with reductions in non-HDL-C, apoB and apoE



TRIGLYCERIDES

- Significant triglyceride reductions in hypertriglyceridemic patients
- For patients with TG \geq 200, GEM lowered TG 39%; TG \geq 500, GEM lowered TG 60%



INFLAMMATION

- Gemcabene has demonstrated over 40% reductions in hsCRP

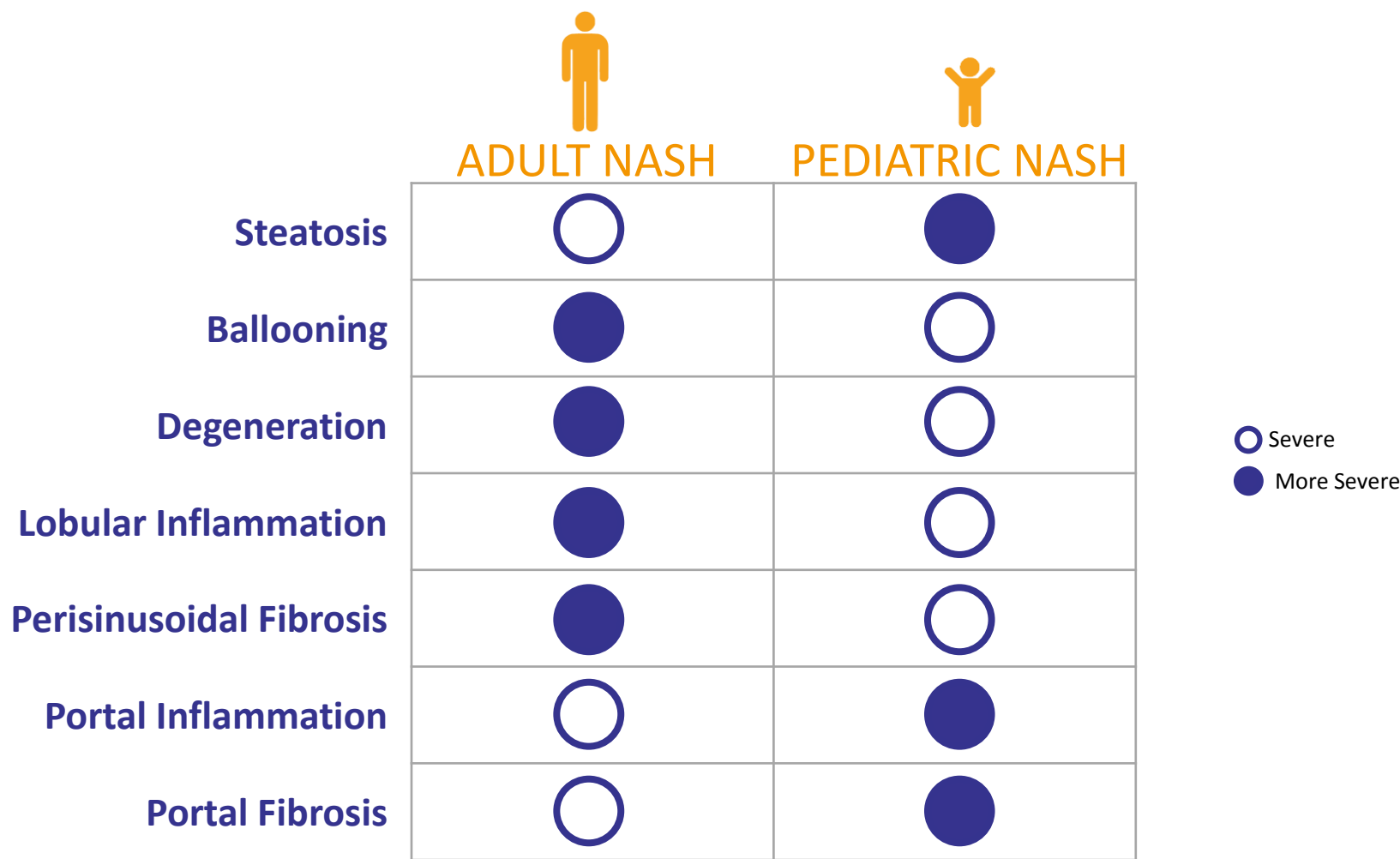


INSULIN SENSITIVITY

- Gemcabene demonstrated a doubling of the glucose disposal rate suggesting potential effects on insulin sensitivity

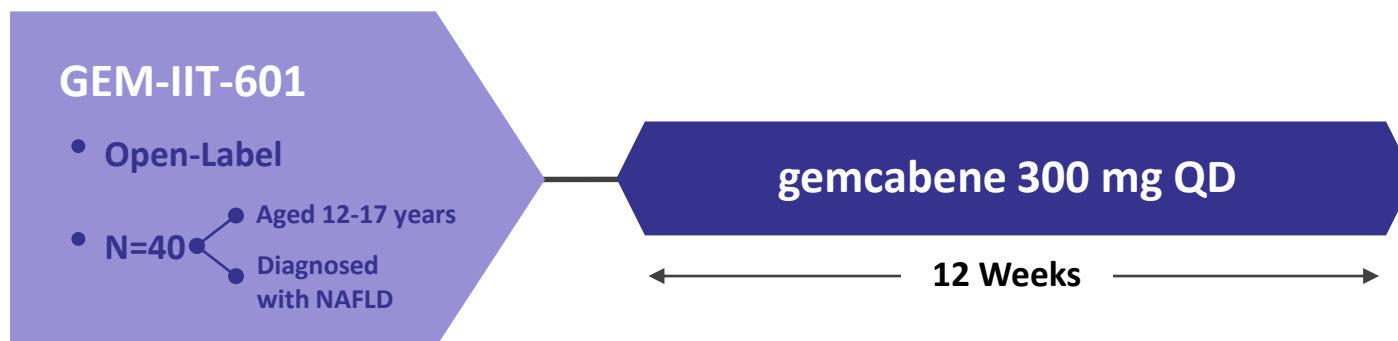
Adults and Children Manifest NASH Differently

Steatosis and Portal Inflammation are More Severe in Pediatric NAFLD



Pediatric NAFLD Phase 2a Study Design

Principal Investigator: Miriam Vos, MD, MSPH
Emory University School of Medicine



PRIMARY ENDPOINT:

- % change in ALT from baseline to 12 weeks

SECONDARY ENDPOINTS:

- Change in hepatic steatosis as measured by MRI-PDFF
- Change in liver inflammation and fibrosis (LIF) score by MRI Liver Multiscan
- Change in AST, insulin sensitivity, serum lipids (including TG), apolipoproteins, and inflammatory markers (including hsCRP)
- Safety and tolerability



REUTERS

“

*Fatty liver disease
fastest growing
reason for
transplant in
young U.S. adults*

”

– Carolyn Crist

OCTOBER 17, 2017

“

I see kids at ages 7 and 8 with this problem, and one of my youngest patients developed cirrhosis at 13.

In Texas in particular, nonalcoholic steatohepatitis is the No. 1 indicator for transplant in adults... It now affects 1 in 3 adults and 1 in 10 children.

Following the childhood obesity explosion in the '80s and '90s, we're seeing young adults with old bodies... Although they're 30, their organs are sick. ”

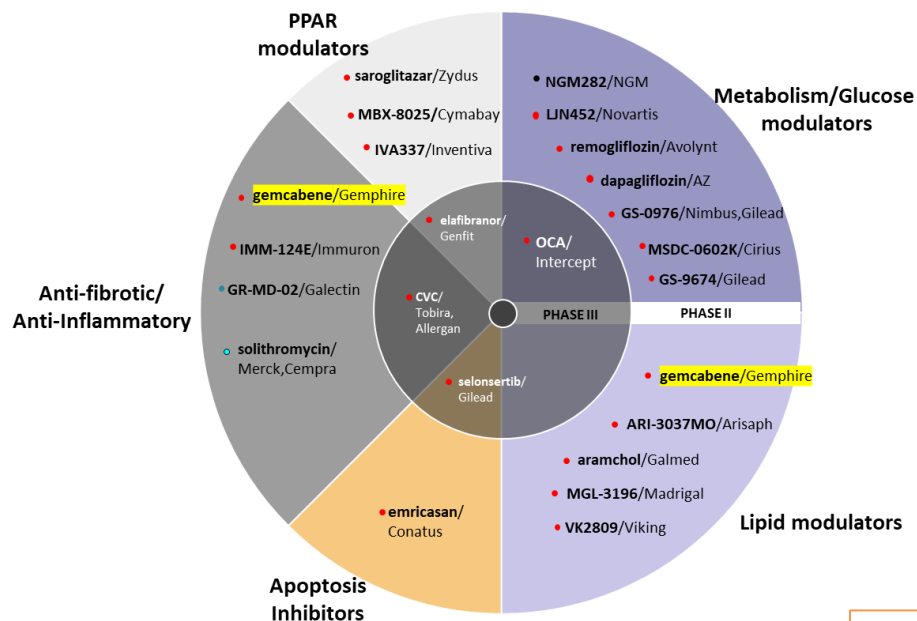
– DR. NAIM ALKHOURI
Senior study author,
Director of the metabolic study program,
Texas Liver Institute, San Antonio, Texas

The number of liver transplants performed for NASH increased from 0.53% in 2002 to 4.46% in 2012, **a nine-fold jump**, the study team reports in the Journal of Clinical Gastroenterology (2017)

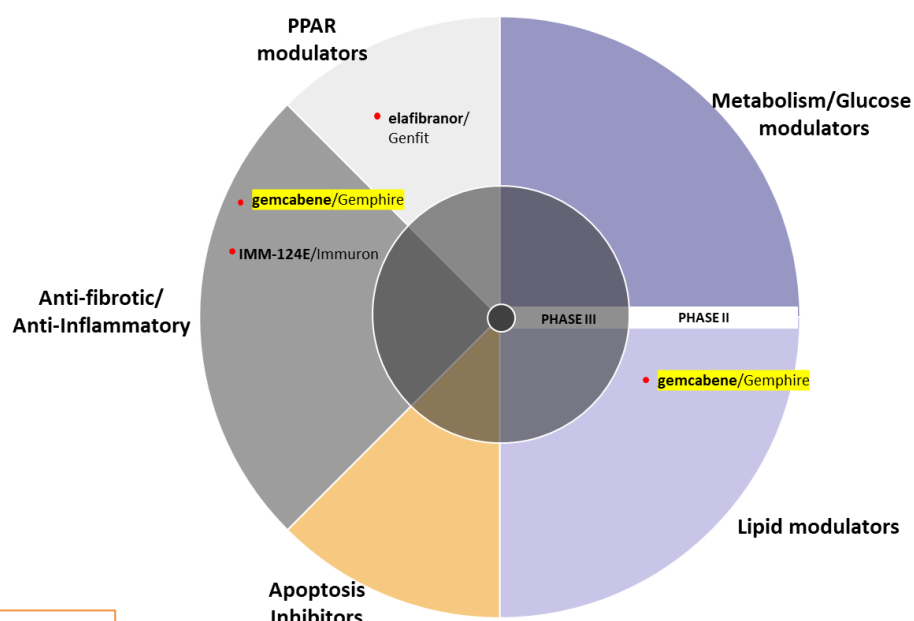
Gempfire
Therapeutics

Phase 2/3 NASH Trials Currently Underway

Adult



Pediatric



- Oral
- Subcutaneous
- IV
- Parenteral

Gemphire's Rationale for Pediatric NAFLD/NASH Development

No FDA approved therapies for pediatric NAFLD/NASH

Safe and tolerable, oral, once-daily cost-effective therapy

Few competing compounds in clinical trials

Potential for **faster recruitment** as compared to Adult NASH trials –
bolus of pediatric NASH patients available

Working with KOLs and agency to **create pathway forward**

Size of market is growing due to obesity trends and
diagnostic/imaging advancements

Efficacious pediatric NAFLD/NASH therapy has **the potential to
impact long term costs to payers**

U.S. Market Assumptions: Adult and Pediatric

Adult NASH

~6M Patients



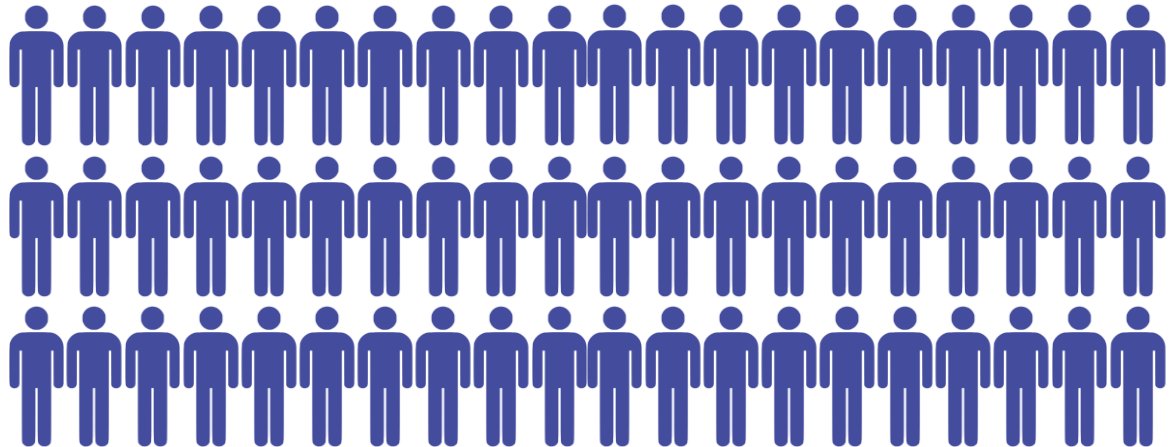
Pediatric NASH

~2M+ Patients



Adult NAFLD

~60M+ Patients



Pediatric NAFLD

~7M+ Patients



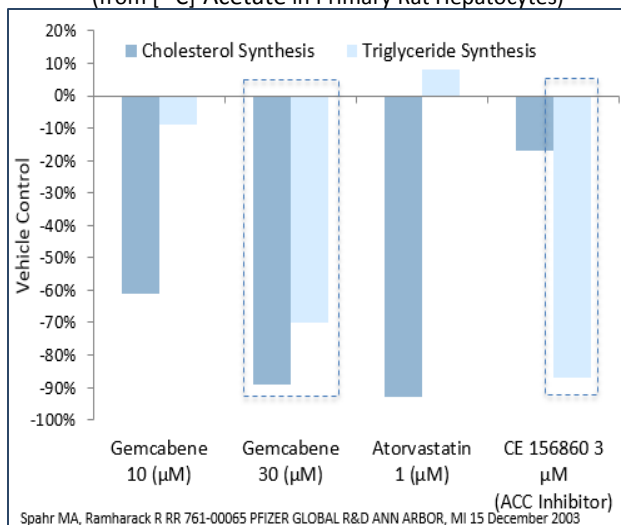
Appendix

Rationale for Gemcabene in NAFLD/NASH

Reduced Liver TGs by 74% in Preclinical Study

Gemcabene Inhibits *de novo* Synthesis of Both Cholesterol and Triglycerides

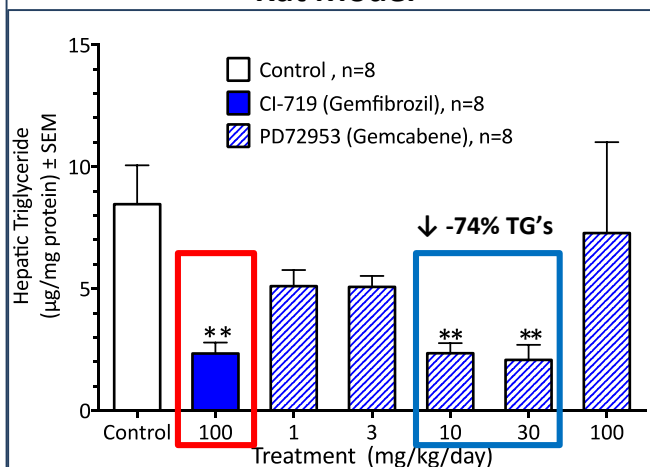
(from [¹⁴C]-Acetate in Primary Rat Hepatocytes)



In Vitro Rat Liver POC TG

Gemcabene reduces hepatic *de novo* cholesterol and TG synthesis from acetate

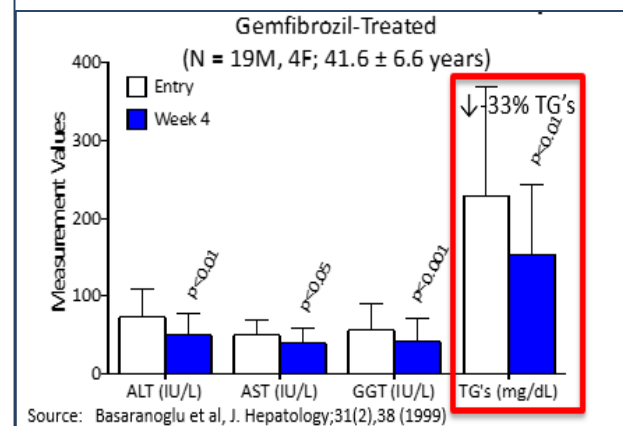
Gemcabene Reduces Hepatic Triglycerides in Sprague Dawley Rat Model



In Vivo Rat Liver POC TG

Gemcabene significantly reduces hepatic triglycerides by -74% in a rat model similar to reductions seen by gemfibrozil

A Controlled Trial of Gemfibrozil in the Treatment of Patients with Nonalcoholic Steatohepatitis



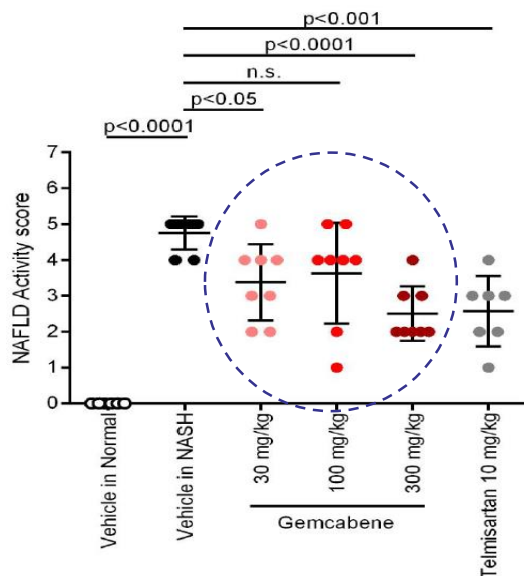
In Vivo Human POC TG

Gemfibrozil reduces TG and other NASH biomarkers in human trials; potentially supportive for gemcabene

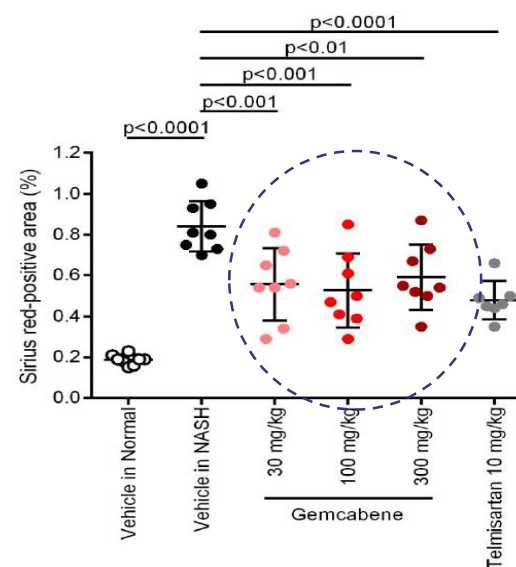
Gemcabene Improves NASH in Rodent Model

Gemcabene Lowers NAFLD Activity Score (NAS) and Fibrosis in STAM™ Model

↓ NAS Score¹ 25% to 48%



↓ Fibrosis ~33%



Gemcabene Comparable to Other Late Stage Compounds in STAM™ Model^{2,3,4}

TOBIRA'S CVC (CCR2/CCR5 INHIBITOR):
~23% to 30% improvement in NAS score
~60% reduction in fibrosis

INTERCEPT'S OCA (FXR AGONIST):
~23% improvement in NAS score

ENANTA'S EDP-305 (FXR AGONIST):
~30% improvement in NAS score

1. NAFLD Activity Score (NAS) composited comprised of steatosis, inflammation, & ballooning; 2. This comparison is for illustrative purposes as these were separate studies; 3. E. Lefebvre et al., The Liver Meeting AASLD, Abstract 30 presentation, 2013; 4. Enanta Pharmaceuticals Company Presentation, 2016

Gemcabene Has Been Shown to Improve Many Parameters in NASH

Lowered Activity of Inflammation & Lipid Metabolism Genes in STAM™ Model

Select Hepatic Gene Expression and Plasma Markers

Category	Gene Expression/ Plasma Markers	Vehicle in NASH	Gemcabene (300 mg/kg)
		(vs Vehicle in Normal)	(vs Vehicle in NASH)
Inflammation	<i>IL-6</i>	▲	▼
	<i>CRP</i>	--	▼
	<i>CCR2</i>	▲	▼
	<i>CCR5</i>	▲	▼
	<i>TNF-α</i>	▲	▼
	<i>MCP-1</i>	▲	▼
	<i>MIP-1β</i>	▲	▼
	<i>NF-kB</i>	▲	▼
Fibrosis	<i>TIMP-1</i>	▲	▼
	<i>MMP-2</i>	▲	▼
Lipid Metabolism	<i>ApoC-III</i>	▼	▼
	<i>SULF-2</i>	▲	▼
	<i>ADH4</i>	--	▼
	<i>ACC1</i>	--	▼

Key:

-- No significant difference ▲ significant increase ▼ significant decrease