# Nonalcoholic Fatty Liver Disease in Children in the U.S.: **Clinical Characteristics of Participants Enrolled in TARGET-NASH**

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## INTRODUCTION

- With rising levels of obesity the prevalence of NAFLD in children is likely to rise but there is little data available outside specialist centers.
- Clinical trials in progress may not always reflect the spectrum of NAFLD disease in participants in real-world clinical practice.
- The overall aim of TARGET-NASH is to determine the natural history of NAFLD and to evaluate treatment regimens used in real world clinical practice.
- This current analysis describes the baseline characteristics of the pediatric TARGET-NASH cohort.

### METHODS

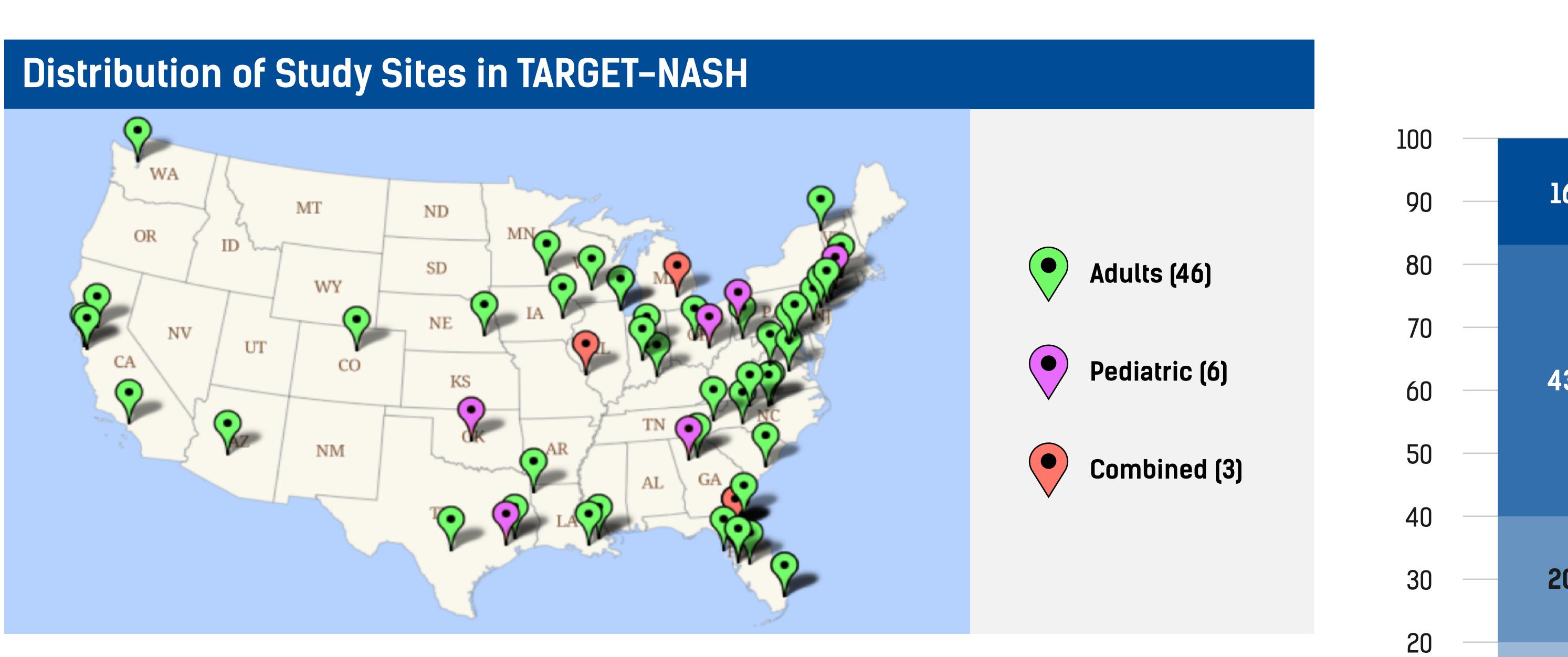
- TARGET-NASH is a longitudinal observational study of participants followed at 55 sites (41 academic/14 community) in the U.S., and includes patients across the entire spectrum of NAFLD as defined by pragmatic case definitions.
- Data from medical records (including narratives, labs, imaging, pathology, procedures, and outcomes) are centrally abstracted and monitored for completeness and accuracy.
- Detailed demographics, patient comorbidities, medications and disease progression are assessed as adverse outcomes.
- To date, 3,970 participants have enrolled in TARGET–NASH, 302 of whom are pediatric participants and are the focus of this analysis.

### **DISEASE CATEGORY** DEFINITIONS

	Confirmed by biopsy: • Steatohepatitis by Brunt criteria OR • NAS total score $\geq 4$ Clinical diagnosis: • Adults: ALT > 19 U/L for female, > 30 U/L for men and; • Hepatic steatosis on biopsy or CT/US/MRI and; • $\geq 1$ of the following: BMI $\geq 30$ , type II diabetes, dyslipidemia
<section-header></section-header>	History of NAFLD with: 1) Liver biopsy with fibrosis stage = 4 OR 2) Liver biopsy with fibrosis stage = 3 and $1 \ge$ clinical signs of 3) 2 or more clinical signs of cirrhosis OR 4) FibroScan <sup>®</sup> stiffness result $\ge$ 11 kPa
NAFL	Any participant not meeting criteria for clinical NASH or cirrho

### cirrhosis OR

osis



## RESULTS

### **Clinical Characteristics for the Pediatr**

	All Participants (N=302)	NAFL Cirrhosis (N=11)	NASH (N=219)	NAFL (N=72)
Age at Study Entry (years) <sup>1</sup> Median (Range)	14 7 - 20	14 9 - 16	14 7 - 19	14 7 - 20
Gender (%) Female Male	32.1 67.9	45.5 54.5	33.3 66.7	26.4 73.6
BMI (kg/m²) at Enrollment – Median	32.0	33.0	32.0	31.5
Comorbidities (%) <sup>2</sup> Type II Diabetes Hypertension Cardiac Disorder Anxiety or Depression Sleep Apnea Syndrome	11.9 13.9 2.3 15.9 8.6	18.2 18.2 0.0 9.1 27.3	11.9 15.1 1.8 15.1 9.1	11.1 9.7 4.2 19.4 4.2
Medications of Interest (%) <sup>2</sup> Vitamin E Metformin Pioglitazone	12.3 11.3 0.0	18.2 18.2 0.0	14.6 11.9 0.0	4.2 8.3 0.0

<sup>1</sup>Age calculated based on year of consent minus birth year <sup>2</sup> From Medical history, AEs or medical history indicative of condition

# All Par

- ascertainment bias.

## **STATEMENT & DISCLOSURES**

TARGET-NASH is a collaboration among academic & community investigators, the pharmaceutical industry, and NASH patient community advocates. TARGET–NASH is sponsored by TARGET PharmaSolutions, Inc. TARGET thanks the study staff, nurses, health care providers and patients at each study center for their contributions to this work. Listings of Principal Investigators and Industry Partners are available upon request by emailing info@targetpharmasolutions.com.

Sirish Palle, MD disclosures: nothing to disclose Miriam B. Vos, MD disclosures: research for Resonance Health, Immuron, Shire, Gemphire, TARGET PharmaSolutions and consulting for AMRA, Axcella Health, Boehringer Ingelheim, Bristol Myers Squibb, Immuron, Intercept, Shire, TARGET PharmaSolutions, Mallinckrodt Pharmaceuticals



### **Biopsy Characteristics for the Pediatric TARGET-NASH Participants – NAS Total Scores %**

.6.7	25	<b>16.1</b>	
3.3			
0.0	75	<b>21.4</b>	
3.3		14.3	
6.7		7.1	
rtipants	IAFLD Cirrhosis	NASH	

## **SUMMARY & CONCLUSIONS**

 Pediatric participants enrolled in TARGET-NASH were more likely to be male and have NASH. • This population had a substantially higher rate of diabetes than in the general pediatric population and the median BMI was 32.0 kg/m<sup>2</sup>

• Pediatric participants in TARGET-NASH had a high rate of medical comorbidities such as diabetes, hypertension, anxiety or depression and sleep apnea. This may represent

• More than 20% of participants were treated with other medications.