**INTRODUCTION**

- Fatty Liver Disease (NAFLD) and NASH are highly prevalent in both children and adults.
- NAFLD can lead to cirrhosis, hepatocellular carcinoma and death from liver disease.
- NAFLD is also associated with increased risk of type II diabetes and cardiovascular events.
- Current treatment, limited to weight loss and exercise, is difficult for patients to achieve and sustain.
- Thus, pharmacologic therapies are greatly needed, and many are in various stages of development.
- To date, clinical trials have relied on surrogate markers (primarily liver histology) to show benefit due to the prolonged natural course of NAFLD.
- Large, observational cohorts are needed to better understand the spectrum of NAFLD by obtaining real-world data that avoids ascertainment bias from studies in tertiary care centers alone and allows for further validation of histology and noninvasive biomarkers.

**AIMS**

**TARGET-NASH** is a long-term, observational study of pediatric and adult patients with NAFLD designed to address questions that go beyond those explored in registration trials. The aims of TARGET-NASH are:

- To establish an understanding of the current natural history of NASH at community and academic medical centers
- To evaluate diagnostic modalities and treatment regimens for NASH currently being used in usual clinical practice
- To provide post-marketing data on clinical effectiveness and safety on pharmacologic agents for the treatment of NAFLD are available
- To collect and maintain a bio-specimen repository linked to carefully collected clinical data for translational studies and biomarker validation

**METHODS**

- Research plan is developed by the Steering Committee, which is comprised of academic thought leaders, regulatory agencies, patient advocates and pharma partners.
- Academic and community sites representing gastroenterology, hepatology, endocrinology and primary care are included.
- Multidata monitoring is performed to increase completeness / accuracy.
- TARGET databases are 21 CFR Part 11 compliant, meet CDISC standards and incorporate WHODRUG and MedDRA coding.
- Patients are consented for submission of medical records, biospecimens and patient reported outcomes surveys (PRQs).
- Biospecimens are collected annually.
- Patient comorbidities, concomitant medications, interventions for NAFLD and disease progression are assessed.
- Adverse outcomes, including cardiovascular and neoplastic complications and those related to medications are recorded.

**DISCLOSURES**

- The authors would like to thank all the investigators, participants and study staff associated with TARGET-NASH.
- The authors and their institutions have received payments, honoraria, or other remuneration from sponsor of the study for their work during the development of the study design and the conduct of the study, and will continue to do so during the publication of the results.

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**CONCLUSIONS**

- TARGET-NASH is a large, diverse, real-world cohort of patients with NAFLD who represent the full spectrum of the disease.
- Patients are being studied without ascertainment bias at both academic and community practices.
- Longitudinal collection of patient level data and disease outcomes will be leveraged to develop and validate non-invasive biomarkers for the diagnosis and progression of NAFLD and to identify clinically meaningful endpoints for treatment trials of NASH.

**RESULTS**

As of 23MAY2017:

- 35 sites in the United States
- 1,441 enrolled patients
- Data and bio-specimens are being collected
- Interim analysis is being initiated using data from the first 1,000 enrolled patients

Green: actively enrolling sites
Yellow: sites in start-up