UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 OR 15(d)
of The Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): **May 6, 2020**

AXCELLA HEALTH INC.

(Exact name of registrant as specified in its charter)

Delaware	**001-38901**	**26-3321056**
(State or other jurisdiction of incorporation)	(Commission File Number)	(IRS Employer Identification No.)

840 Memorial Drive **Cambridge, Massachusetts**	**02139**
(Address of principal executive offices)	(Zip Code)

Registrant's telephone number, including area code: **(857) 320-2200**

Not Applicable
(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.001 Par Value	**AXLA**	**Nasdaq Global Market**

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 8.01. Other Events

On May 6, 2020, Axcella Health Inc. issued a press release announcing positive top-line data from its clinical study showing multifactorial activity in adult subjects with NAFLD and hosted a conference call and webcast to discuss such top-line data. A copy of the press release is attached hereto as Exhibit 99.1 and a copy of the accompanying presentation used during the conference call and webcast is attached hereto as Exhibit 99.2.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits:

Exhibit No.	Description
99.1	Press release issued by Axcella Health Inc., dated May 6, 2020
99.2	Presentation of Axcella Health Inc., dated May 6, 2020

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

AXCELLA HEALTH INC.

Date: May 6, 2020

By: /s/ William R. Hinshaw, Jr.
William R. Hinshaw, Jr.
Chief Executive Officer, President and Director

Exhibit 99.1

Axcella Announces Positive Top-Line Data from AXA1125-003 Clinical Study Showing Multifactorial Activity in Adult Subjects with NAFLD

- *Clinically relevant reductions in liver fat content, insulin resistance and fibroinflammation markers observed with AXA1125 along with favorable tolerability, supporting its potential to be a first-line NASH therapy*
- *Greater activity in key markers seen among subjects with type 2 diabetes receiving AXA1125*
- *Company plans to engage with FDA regarding IND submission for AXA1125, proposed Phase 2b clinical trial in adult NASH and pediatric development program*
- *Conference call to be held today at 8:30 a.m. ET*

Cambridge, Mass., May 6, 2020 – Axcella (Nasdaq: AXLA), a clinical-stage biotechnology company focused on leveraging endogenous metabolic modulators (EMMs) to pioneer a new approach for treating complex diseases and improving health, today announced positive top-line data from AXA1125-003.

AXA1125-003 is a placebo-controlled, randomized, multi-arm clinical study assessing the impact of AXA1125 and AXA1957 on safety, tolerability and effects on structures and functions of the liver, as measured by a comprehensive panel of imaging and soluble biomarkers related to metabolism, inflammation and fibrosis. Both of these distinct product candidates are proprietary compositions of amino acids and derivatives that have been designed to support liver health. In this non-IND study, 102 adult non-alcoholic fatty liver disease (NAFLD) subjects with presumed nonalcoholic steatohepatitis (NASH), based on inclusion criteria, were enrolled and dosed in a 2:2:2:1 ratio to receive AXA1125, one of two AXA1957 doses, or placebo administered twice daily for 16 weeks. Study subjects were stratified based on the presence or absence of type 2 diabetes.

Results from the study showed that AXA1125 and AXA1957 were generally well-tolerated, with sustained reductions noted for both product candidates versus placebo in key biomarkers of metabolism, inflammation and fibrosis over 16 weeks. Overall, as compared to placebo, AXA1125 demonstrated larger and more consistent reductions in clinically relevant biomarkers than AXA1957. Among subjects receiving AXA1125, 39% achieved a ≥30% relative reduction in liver fat content (MRI-PDFF), 39% achieved a ≥17 U/L reduction in alanine aminotransaminase (ALT), and 35% achieved a ≥80 mSec reduction in corrected T1 (cT1). Among the 11 subjects with type 2 diabetes receiving AXA1125, a greater proportion achieved each of these thresholds. Emerging evidence suggests that these thresholds of activity increase the likelihood of histopathological improvement in NASH subjects. Notably, the above results were seen without impacting mean body weight or serum lipids.

Manu Chakravarthy, M.D., Ph.D., Chief Medical Officer of Axcella, said, “In AXA1125-003, we were seeking to evaluate safety and tolerability while also determining what differential responses may be seen from AXA1125 and AXA1957 across markers of metabolism, inflammation and fibrosis. It is gratifying that multifactorial activity and a favorable tolerability profile were noted for AXA1125 in this multi-arm, placebo-controlled randomized study, which replicates findings from our previous clinical study in subjects with NAFLD and type 2 diabetes. We believe that these data, coupled with AXA1125’s oral route of administration and favorable tolerability profile to date, reinforce its potential to meaningfully improve the lives of patients with NASH.”

“These data indicate that AXA1125 holds the potential to be a first-line therapy in NASH, with impressive, concordant metabolism, inflammation and fibrosis activity as well as a favorable tolerability profile with no meaningful changes to serum lipids and body weight,” said Stephen A. Harrison, M.D., Medical Director of Pinnacle Clinical Research in San Antonio, TX, visiting professor of Hepatology at the University of Oxford, UK and the principal investigator of AXA1125-003. “Additionally, in the majority of subjects with type 2 diabetes receiving AXA1125, clinically relevant thresholds of activity were observed in non-invasive tests that suggest a higher probability of positive outcomes histopathologically. This is a potential differentiator and is particularly encouraging given that nearly 40% of the NASH population is diabetic and the disease is known to be more severe in these patients.”

Select Measures of Relevance at 16 Weeks

Measure	Placebo	AXA1125	AXA1957 Low	AXA1957 High
Subjects dosed	15	29	26	32
Mean relative reduction in liver fat content (MRI-PDFF)	-6%	-23%	-20%	-8%
Subjects with ≥30% relative reduction in liver fat content	8%	39%	23%	19%
Mean relative reduction in ALT	-7%	-22%	-19%	-21%
Subjects with ≥17 U/L reduction in ALT	25%	39%	32%	37%
Subjects with ≥80 mSec reduction in cT1	17%	35%	23%	23%
Mean absolute change in proC3 (ng/mL)	-0.7	-3.4	-3.1	-4.1

** Mean values and percentages above only include subjects for whom data was available at the week 16 timepoint.*

AXA1125 and AXA1957 were both generally well tolerated in the study. The adverse events (AEs) experienced in ≥10% of subjects were gastrointestinal (diarrhea, nausea, reduced appetite) and upper respiratory infection. Gastrointestinal AEs were generally mild and transient, self-resolving in two to three weeks on average. Two serious adverse events were reported, both of which were determined to be unrelated to study product administration.

“The findings from this clinical study further validate the strength of our EMM platform and its ability to identify product candidates with the potential to address complex diseases in a multi-targeted manner,” said Bill Hinshaw, President and Chief Executive Officer of Axcella. “Given the strength and consistency of data on AXA1125, we have selected it as our product candidate for NASH and have decided that we will not reinitiate our AXA1957-002 pediatric study, which had recently been suspended due to COVID-19. In the months ahead, we plan to engage with the U.S. Food and Drug Administration (FDA) to discuss our investigational new drug (IND) application for AXA1125, proposed Phase 2b clinical trial in adults and pediatric development program. We give our thanks to the many subjects and investigators who participated in AXA1125-003 and will continue working diligently toward our goal of providing them with an effective and safe treatment option.”

Conference Call Information

Axcella will host a conference call today at 8:30 a.m. ET to discuss the top-line data from AXA1125-003. The conference call webcast and accompanying slides will be made available shortly before the start of the call on the company’s website at www.axcellahealth.com in the Investors & News. To access the call via telephone, please dial (866) 652-5200 (U.S. toll free) or (412) 317-6060 (international) five minutes prior to the start time. For those unable to listen in live, a webcast archive will be available on the company’s website for 30 days following the call.

About Endogenous Metabolic Modulators (EMMs)

EMMs are a broad family of molecules, including amino acids, that regulate human metabolism. Axcella is developing a range of novel product candidates that are comprised of multiple EMMs engineered in distinct combinations and ratios to simultaneously impact multiple metabolic pathways to modify the root causes of various complex diseases and improve health.

About Axcella’s Clinical Studies

Each of the company’s clinical studies to date, including AXA1125-003, are or have been conducted as non-investigational new drug application (IND) clinical studies under U.S. Food and Drug Administration regulations and guidance supporting research with food. These studies evaluate product candidates for safety, tolerability and effects on the normal structures and functions in humans, including in individuals with disease. They are not designed or intended to evaluate a product candidate’s ability to diagnose, cure, mitigate, treat or prevent a disease. If Axcella decides to further develop a product candidate as a potential therapeutic, as is the case with AXA1665 and AXA1125, any subsequent clinical studies will be conducted under an IND.

Internet Posting of Information

Axcella uses its website, www.axcellahealth.com, as a means of disclosing material nonpublic information and for complying with its disclosure obligations under Regulation FD. Such disclosures will be included on the company's website in the "Investors and News" section. Accordingly, investors should monitor such portions of the company's website, in addition to following its press releases, SEC filings and public conference calls and webcasts.

About Axcella

Axcella is a clinical-stage biotechnology company focused on leveraging endogenous metabolic modulators (EMMs) to pioneer a new approach for treating complex diseases and improving health. The company's product candidates are comprised of EMMs and their derivatives that are engineered in distinct combinations and ratios to simultaneously impact multiple biological pathways. Axcella's pipeline includes lead therapeutic candidates for non-alcoholic steatohepatitis (NASH) and the reduction in risk of overt hepatic encephalopathy (OHE) recurrence. Additional muscle- and blood-related programs are in earlier-stage development. For more information, please visit www.axcellahealth.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements regarding the characteristics, competitive position and development potential of the company's EMM product candidates, including AXA1125, the design, status and timing of the company's ongoing clinical studies and planned IND-enabled clinical trials, including with respect to the company's planned adult and pediatric clinical trials for AXA1125, the subject and timing of the company's interactions with the FDA, including with respect to an IND application, Phase 2b clinical trial in adults and pediatric development plans for AXA1125, and the potential of the company's product candidates to impact health and/or disease, including AXA1125's potential in NASH. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, those related to the potential impact of COVID-19 on the company's ability to conduct and complete its planned clinical trials in a timely manner or at all due to patient or principal investigator recruitment or availability challenges, clinical trial site shutdowns or other interruptions and potential limitations on the quality, completeness and interpretability of data we are able to collect, other potential impacts of COVID-19 on our business and financial results, including with respect to our ability to raise additional capital and operational disruptions or delays, , changes in law, regulations, or interpretations and enforcement of regulatory guidance, whether data readouts and/or FDA feedback support our planned timing for an IND filing, clinical trial design and target indication for AXA1125, the clinical development and safety profile of the company's product candidates and their health or therapeutic potential, whether and when, if at all, the company's product candidates will receive approval from the FDA or other comparable regulatory authorities, and for which, if any, indications, competition from other biotechnology companies, past results from clinical studies not being representative of future results, and other risks identified in the company's SEC filings, including Axcella's Annual Report on Form 10-K, Quarterly Report on Form 10-Q and subsequent filings with the SEC. The company cautions you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. Axcella disclaims any obligation to publicly update or revise any such statements to reflect any change in expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements. Any forward-looking statements contained in this press release represent the company's views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. The company explicitly disclaims any obligation to update any forward-looking statements.

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Exhibit 99.2

NASDAQ: AXLA

AXA1125-003
Top-Line Data

May 6, 2020

Forward-Looking Statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements regarding the characteristics, competitive position and development potential of the company's EMM product candidates, including for AXA1125 and AXA1665, the design, status and timing of the company's ongoing clinical studies and planned IND-enabled clinical trials, the company's anticipated program milestones, including the timing of AXA1665-002 and AXA4010 top line data readouts, the subject and timing of the company's planned interactions with the FDA on the AXA1665 and AXA1125 programs, including potential timing of IND application submissions, and the potential of the company's product candidates to impact health and/or disease, including AXA1125's potential in NASH and AXA1665's potential in OHE. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this presentation are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this presentation, including, without limitation, those related to the potential impact of COVID-19 on the company's ability to conduct and complete its ongoing or planned clinical studies and IND enabled clinical trials and planned interactions and submissions to FDA or other regulatory authorities in a timely manner or at all due to patient or principal investigator recruitment or availability challenges, clinical trial site shutdowns or other interruptions and potential limitations on the quality, completeness and interpretability of data we are able to collect in our ongoing AXA1665-002 and AXA4010-001 clinical studies and potential delays in disclosure of the same, other potential impacts of COVID-19 on our business and financial results, including with respect to our ability to raise additional capital and operational disruptions or delays, changes in law, regulations, or interpretations and enforcement of regulatory guidance, whether data readouts and/or FDA feedback support our IND submission and clinical trial initiation plans and timing, clinical trial design and target indication for AXA1125 and AXA1665, the clinical development and safety profile of the company's product candidates and their health or therapeutic potential, whether and when, if at all, the company's product candidates will receive approval from the FDA or other comparable regulatory authorities, and for which, if any, indications, competition from other biotechnology companies, past results from clinical studies not being representative of future results, and other risks identified in the company's SEC filings, including Axcella's Annual Report on Form 10-K, Quarterly Report on Form 10-Q and subsequent filings with the SEC. The company cautions you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. Axcella disclaims any obligation to publicly update or revise any such statements to reflect any change in expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements. Any forward-looking statements contained in this presentation represent the company's views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. The company explicitly disclaims any obligation to update any forward-looking statements.

About Axcella's Development Model and Clinical Approach

EMMs have a fundamental role in biology and function. Using the Axcella Knowledge Base, Axcella designs and develops novel EMM compositions to engage identified biologies and pathways. Axcella then selects whether to evaluate a product candidate in a non-investigational new drug application (non-IND) clinical study under U.S. Food and Drug Administration regulations and guidance supporting research with food, or under an IND clinical trial. Axcella's non-IND clinical studies evaluate product candidates for safety, tolerability and effects on the normal structures and functions in humans, including in individuals with disease. The company's non-IND clinical studies include a substantial number of biomarkers that may inform biologies relevant to health but are not designed or intended to evaluate a product candidate's ability to diagnose, cure, mitigate, treat or prevent a disease or other health condition. They are conducted at reputable medical centers following Good Clinical Practices (GCPs), including Institutional Review Board (IRB) approval, and utilize qualified investigators. Using a combination of data from these studies and/or other relevant information, the company decides whether to advance a product candidate's development as a therapeutic or supplement (independently or in partnership), or terminate its development.

To date, Axcella has evaluated its current product candidates as investigational food products in non-IND clinical studies. Axcella has determined its lead compounds – AXA1665 and AXA1125 – to be therapeutic product candidates, meaning that pending and subject to final data readouts from ongoing non-IND clinical studies and FDA feedback, any future development of these product candidates will be under IND clinical trials. These IND clinical trials would therefore be designed to evaluate each product candidate's ability to diagnose, cure, mitigate, treat or prevent targeted diseases. The company's current plans are to target OHE with AXA1665 and NASH with AXA1125 in IND clinical trials.

In the future, if Axcella decides to pursue therapeutic development for a new product candidate, it will initiate clinical development under an IND-enabled trial.

This presentation refers to Axcella's non-IND clinical studies as "Clinical Studies" and its planned IND-enabled clinical trials as "Clinical Trials."

AXA1125: A Potential First-Line NASH Therapy

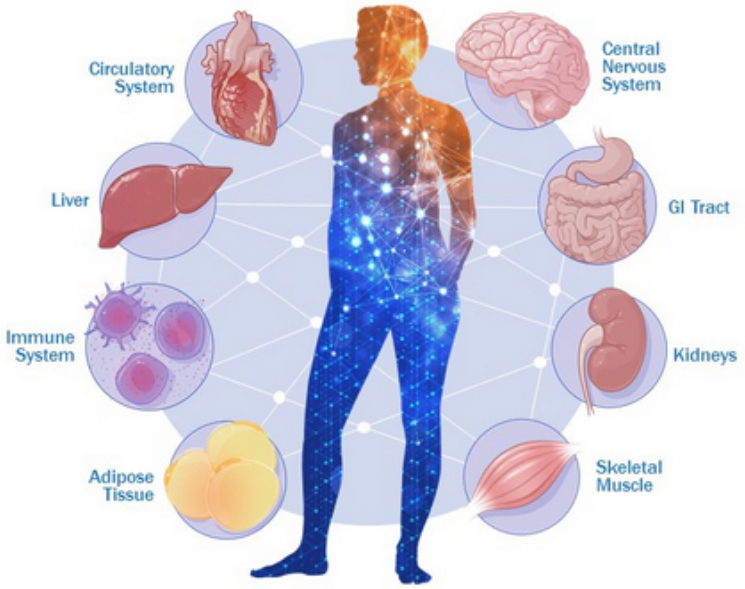
- AXA1125-003 findings further validate Axcella's platform and approach utilizing EMMs
- Multifactorial activity observed for both AXA1125 and AXA1957, while being well tolerated
- AXA1125 generated more clinically relevant reductions in liver fat content, insulin resistance and fibroinflammation markers when compared with AXA1957
  - Greater activity in key markers seen among subjects with type 2 diabetes receiving AXA1125
- Plan to engage with FDA to discuss an IND application for AXA1125, a proposed Phase 2b clinical trial in adults and Axcella's pediatric development program

EMM, Endogenous Metabolic Modulator; FDA, U.S. Food and Drug Administration; NASH, nonalcoholic steatohepatitis.

Axcella's Focus: Endogenous Metabolic Modulators (EMMs)

EMMs Act as Signaling Agents and Master Regulators in Human Biology

- EMMs, including amino acids, are well established agents used to support health
- Single EMMs and simple EMM combinations have been approved as treatments for various diseases
- Leveraging advances in machine learning and systems biology, Axcella is developing novel EMM compositions that:
  - Include up to 10 amino acids and derivatives
  - Are designed to affect multiple biological pathways simultaneously
  - Have disease-modifying potential



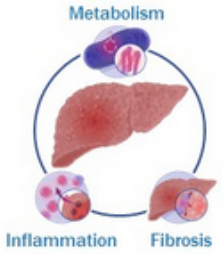
NASH: A Complex Disease with High Unmet Needs

State of the market:

- >15 million U.S. patients and growing[1]
- Similar prevalence in the European Union 5
- 30-40% of the population is diabetic[2]
- Expected to be the leading liver transplant cause this year
- U.S. market expected to reach at least $8 billion by 2027[1]

State of development:

- No approved therapies in the U.S.
- Single-target mechanisms for most agents in development
- Safety/tolerability challenges (lipids, pruritis, etc.)
- Combination therapy already an area of focus
- Very limited pediatric development activity



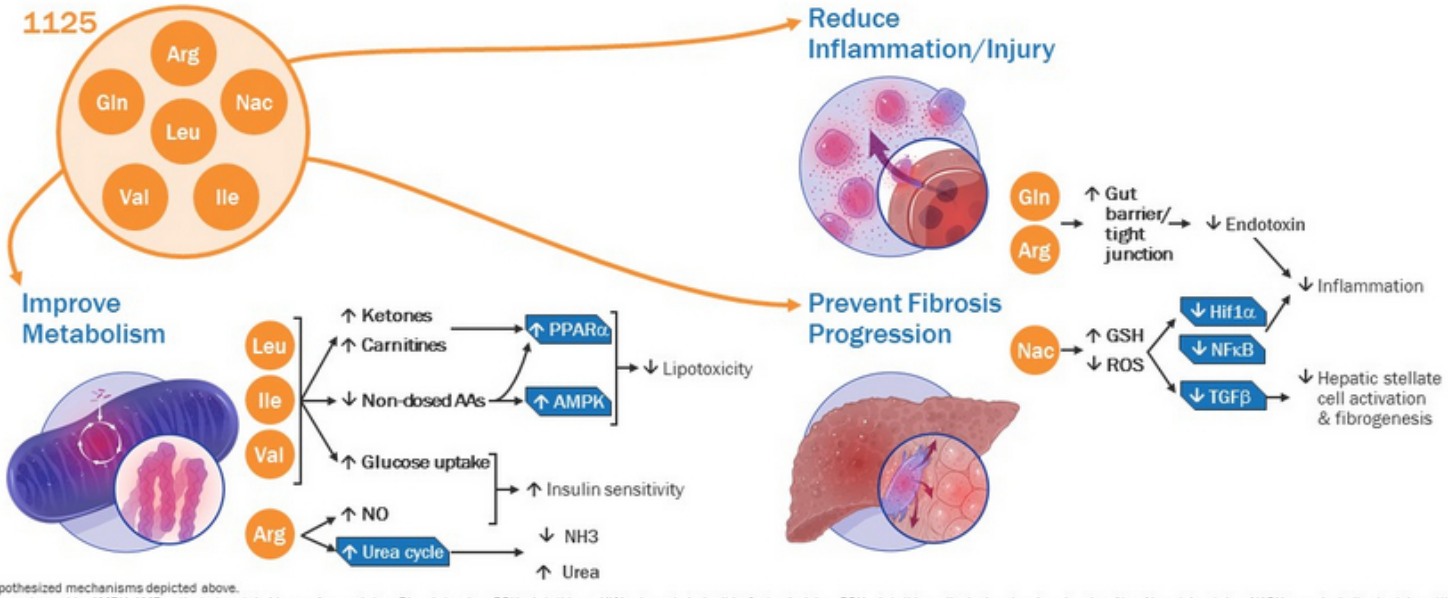
Potential Axcella Differentiators:

- Oral candidate for first-line therapy
- Multi-targeted approach
- Favorable safety/tolerability profile to date
- Amenable to combination approaches
- Planning for pediatric development

1. Company estimates based on Decision Resources Group (DRG). Non-alcoholic Steatohepatitis Landscape & Forecast. DRG
2. Cusi, K. Diabetologia. 2016

AXA1125 - A Novel Oral Product Candidate for NASH

Designed to target multiple metabolic pathways; potential for multifactorial activity relevant to NASH



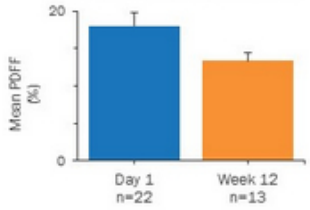
Hypothesized mechanisms depicted above.
AAs, amino acids; AMPK, AMP-activated protein kinase; Arg, arginine; Gln, glutamine; GSH, glutathione; Hif1α, hypoxia-inducible factor 1 alpha; GSH, glutathione; Ile, isoleucine; Leu, leucine; Nac, N-acetylcysteine; NASH, nonalcoholic steatohepatitis; NFκB, nuclear factor kappa-light-chain-enhancer of activated B cells; NH3, ammonia; NO, nitric oxide; PPARα, peroxisome proliferator-activated receptor alpha; ROS, reactive oxygen species; TGFβ, transforming growth factor beta; Val, valine.

Positive Results Seen in Previous Clinical Study of AXA1125

AXA1125-002: A single-arm Clinical Study of 32 subjects with type 2 diabetes and NAFLD

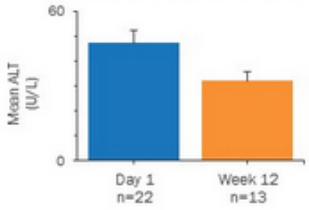
Metabolism

Reduced Liver Fat (MRI-PDFF)

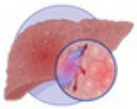


Inflammation

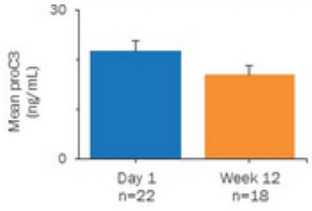
Reduced ALT (inflammation marker)



Fibrosis



Reduced ProC3 (fibrogenic marker)



AASLD THE LIVER MEETING® 2018 SAN FRANCISCO NOVEMBER 9-13

KEYSTONE SYMPOSIA on Molecular and Cellular Biology
Accelerating Life Science Discovery

AASLD THE LIVER MEETING® NOVEMBER 8-12 2019 BOSTON

1. Non-IND Clinical Study initiated prior to therapeutic development path decision. Please refer to slide 3 for further detail.
23 subjects received AXA1125 24 g TID and had baseline PDFF >10%; 5 received AXA1125 24 g TID with baseline PDFF <10%; 4 received AXA1125 6 g TID.
ALT, alanine aminotransferase; IND, investigational new drug; MRI-PDFF, magnetic resonance imaging proton density fat fraction; NAFLD, non-alcoholic fatty liver disease; ProC3, propeptide of type III collagen; TID, 3 times a day.

Design of AXA1125-003

STUDY BACKGROUND[1]

Primary endpoint: Safety & tolerability

Key assessments include:

- Liver structure by MRI-PDFF and cT1
- Liver function: Markers of glucose and lipid homeostasis, inflammation, apoptosis, and fibrosis

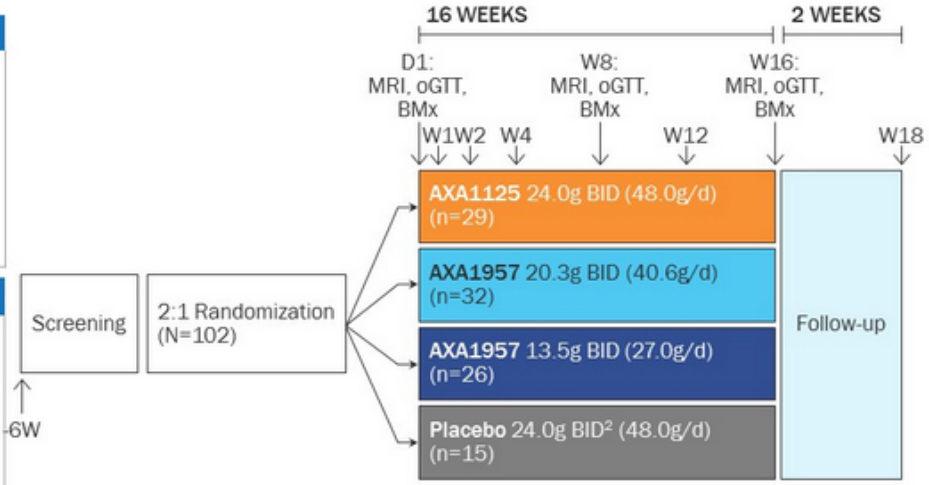
Not powered for statistical significance

STUDY DESIGN

Multicenter (Summit Network), 16-week, randomized, placebo-controlled Clinical Study of AXA1125 and AXA1957

Key inclusions: >10% fat by MRI-PDFF, cT1 >830 mSec

Stratified by T2D and non-diabetic subjects



1. Non-IND Clinical Study initiated prior to therapeutic development path decision. Please refer to slide 3 for further detail.
2. Calorie-matched placebo control.

BID, 2 times a day; BMx, biomarkers; D, Day; cT1, corrected T1; IND, investigational new drug; MRI-PDFF, magnetic resonance imaging proton density fat fraction; oGTT, oral glucose tolerance test; T2D, type 2 diabetes; W, Week.

Demographics Indicative of a Population with Presumed NASH

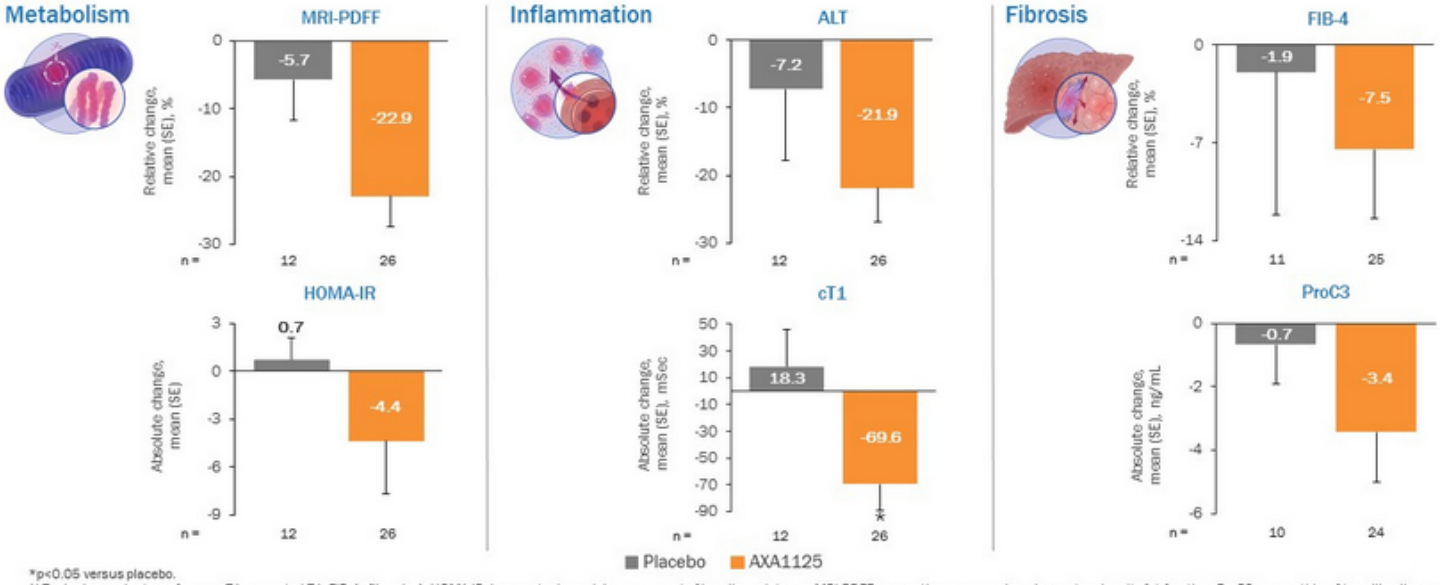
Baseline Demographic/Metric	Placebo (n=15)	AXA1125 (n=29)	AXA1957 high (n=32)	AXA1957 low (n=26)
Age, mean (SD), years	53.2 (9.62)	49.2 (12.79)	50.1 (12.79)	49.6 (10.74)
Sex				
Female, n (%)	10 (66.7)	17 (58.6)	19 (59.4)	16 (61.5)
Male, n (%)	5 (33.3)	12 (41.4)	13 (40.6)	10 (38.5)
Weight, mean (SD), kg	118.25 (31.75)	102.86 (23.82)	102.31 (26.14)	106.24 (23.50)
BMI, mean (SD), kg/m^2	42.0 (9.39)	36.8 (7.32)	38.5 (8.49)	37.4 (6.11)
Diagnosed type 2 diabetes, n (%)	6 (40.0)	12 (41.4)	12 (37.5)	10 (38.5)
Metabolism				
Liver fat content by MRI-PDFF, %	21.19 (1.51)	22.35 (0.93)	22.27 (0.93)	21.01 (1.16)
HOMA-IR	8.59 (1.23)	13.51 (3.39)	10.82 (1.78)	9.62 (1.35)
Inflammation				
ALT, U/L	50.5 (8.73)	55.2 (4.89)	50.5 (4.11)	60.6 (5.66)
AST, U/L	41.3 (34.3)	37.3 (22.7)	40.6 (21.0)	34.9 (16.6)
cT1, mSec	1022.3 (41.35)	960.6 (16.84)	1017.2 (24.30)	959.5 (21.67)
Fibrosis				
Fibroscan score, mean (SD), kPa	13.51 (5.69)	11.73 (6.67)	11.18 (3.82)	16.31 (15.76)
ProC3, ng/mL	15.85 (1.82)	17.07 (1.56)	16.76 (1.73)	17.02 (1.90)

All values are mean (SE) unless otherwise noted.
ALT, alanine aminotransferase; AST, alanine transaminase; BMI, body mass index; cT1, corrected T1; HOMA-IR, homeostasis model assessment of insulin resistance; MRI-PDFF, magnetic resonance imaging proton density fat fraction; ProC3, propeptide of type III collagen; SD, standard deviation; SE, standard error.

Activity Seen in Subjects Receiving AXA1125 vs. Placebo

AXA1125: Reductions Noted in Key Biomarkers

Changes from baseline at week 16

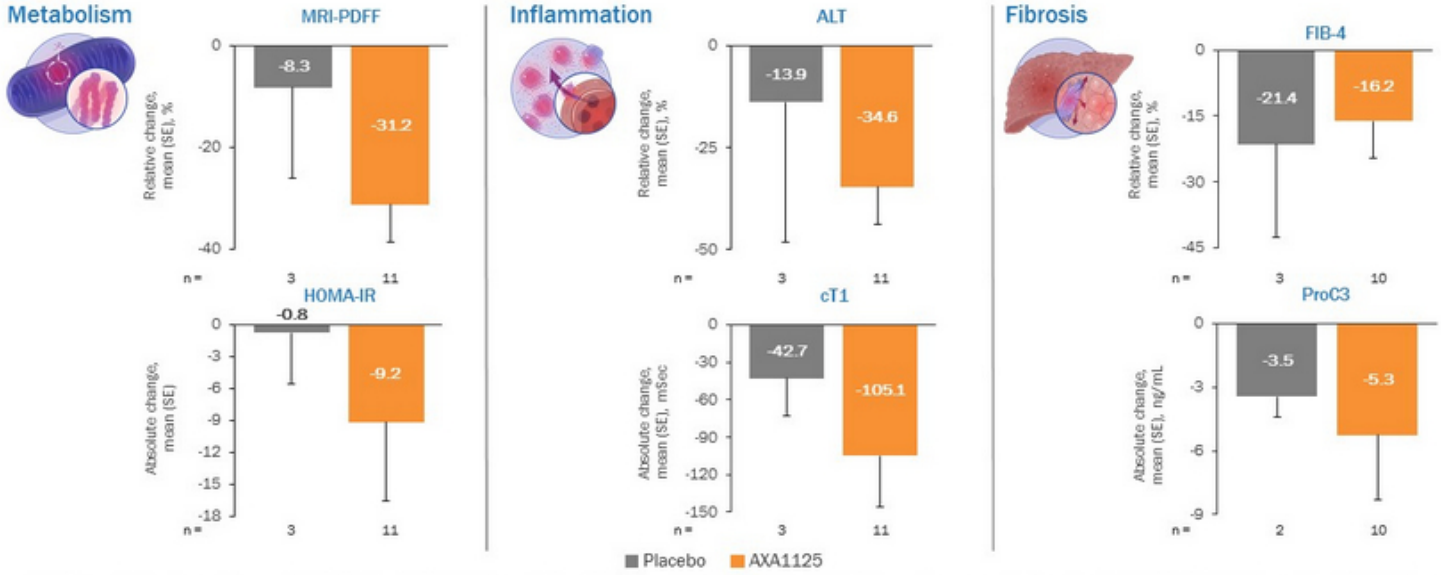


*p<0.05 versus placebo.

ALT, alanine aminotransferase; cT1, corrected T1; FIB-4, fibrosis 4; HOMA-IR, homeostasis model assessment of insulin resistance; MRI-PDFF, magnetic resonance imaging proton density fat fraction; ProC3, propeptide of type III collagen; SE, standard error.

AXA1125: Even Greater Activity Seen in Subjects with Diabetes

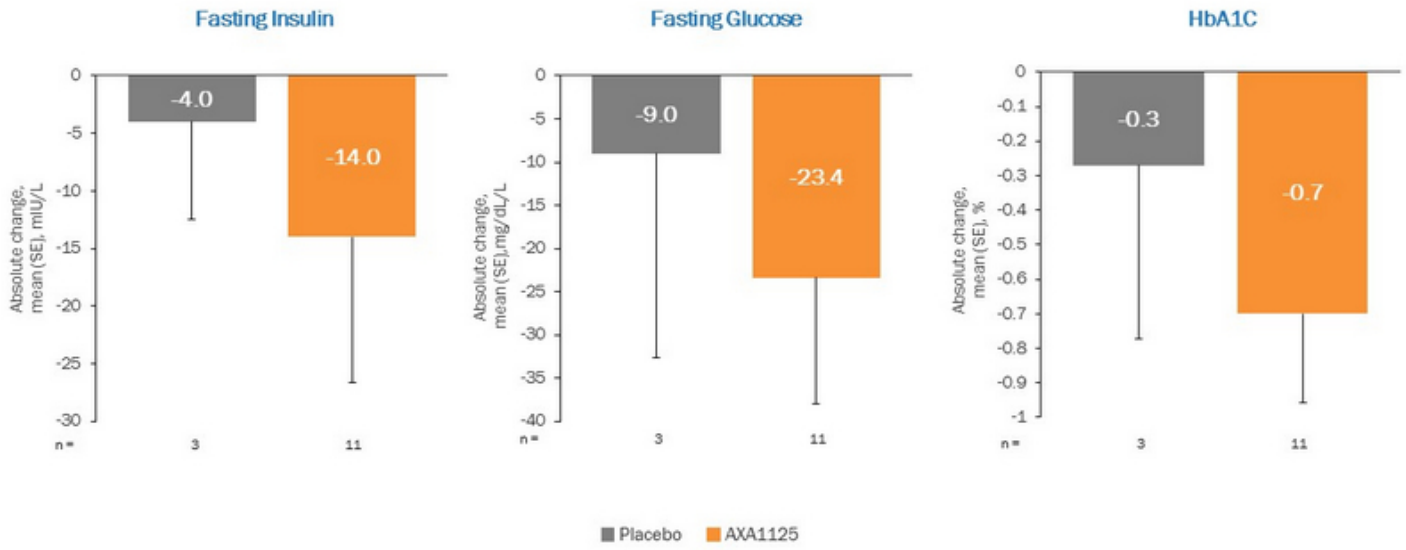
Changes from baseline at week 16



ALT, alanine aminotransferase; cT1, corrected T1; FIB-4, fibrosis 4; HOMA-IR, homeostasis model assessment of insulin resistance; MRI-PDFF, magnetic resonance imaging proton density fat fraction; ProC3, propeptide of type III collagen; SE, standard error.

AXA1125: Insulin and Glucose Changes in Type 2 Subjects with Diabetes

Changes from baseline at week 16



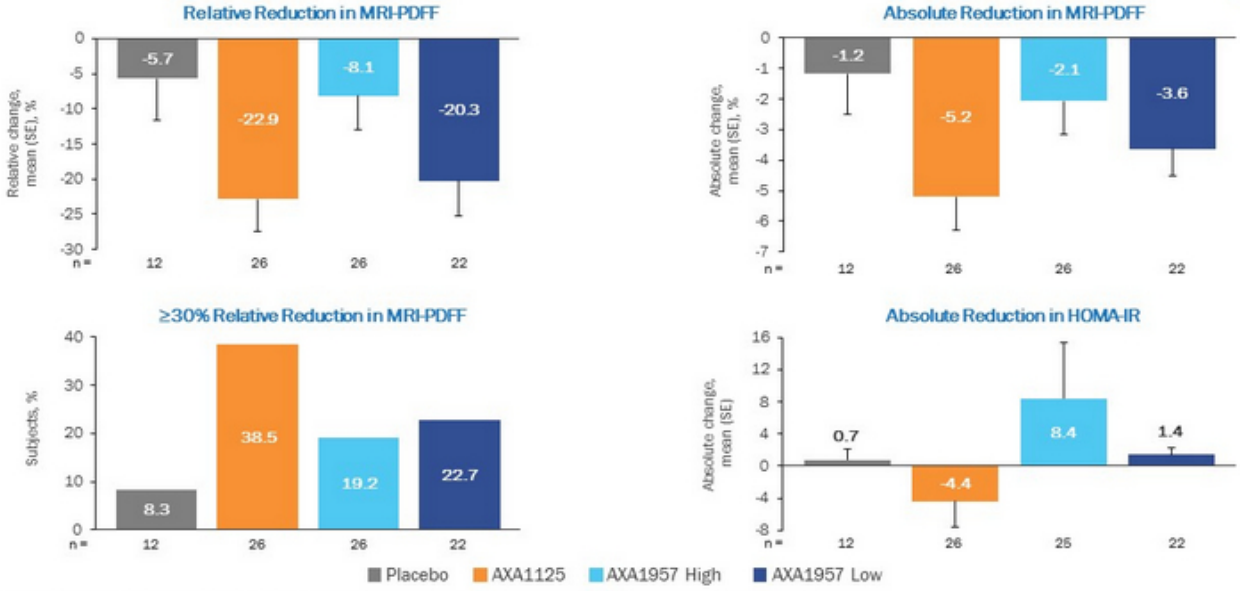
HbA1c, glycated hemoglobin; SE, standard error.

Activity Seen in Subjects Receiving AXA1125 and AXA1957 vs. Placebo

Metabolism

Greater Metabolism Activity with AXA1125

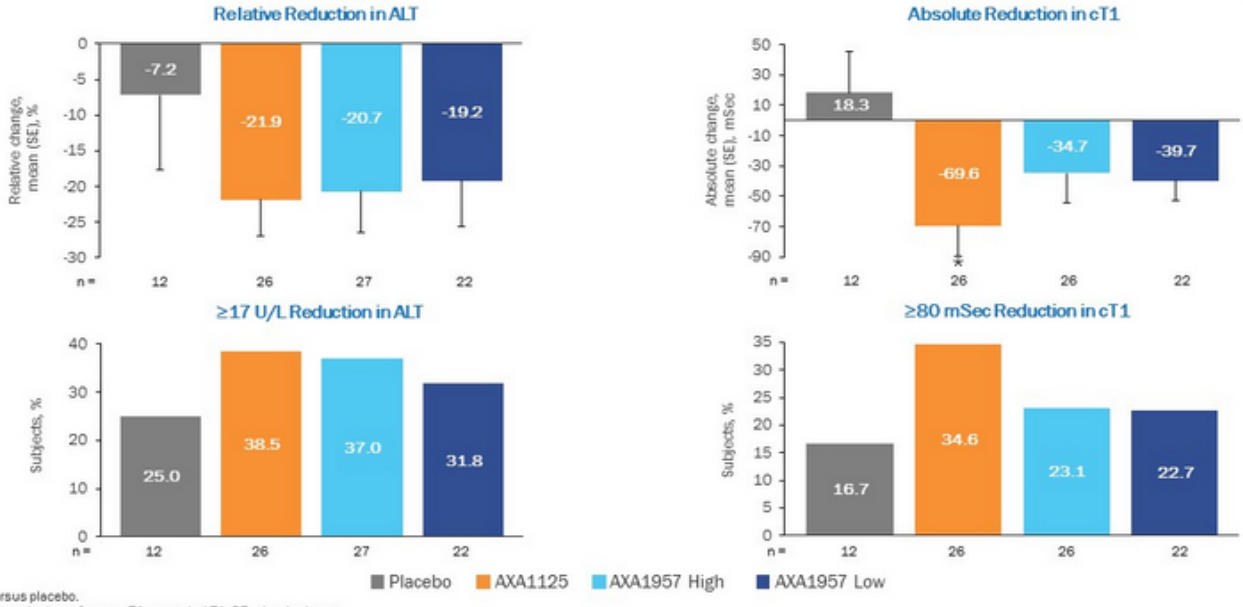
Changes from baseline at week 16



HOMA-IR, homeostasis model assessment of insulin resistance; MRI-PDFF, magnetic resonance imaging proton density fat fraction; SE, standard error.

Greater Impact on Inflammation with AXA1125

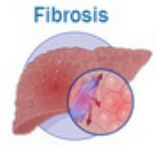
Changes from baseline at week 16

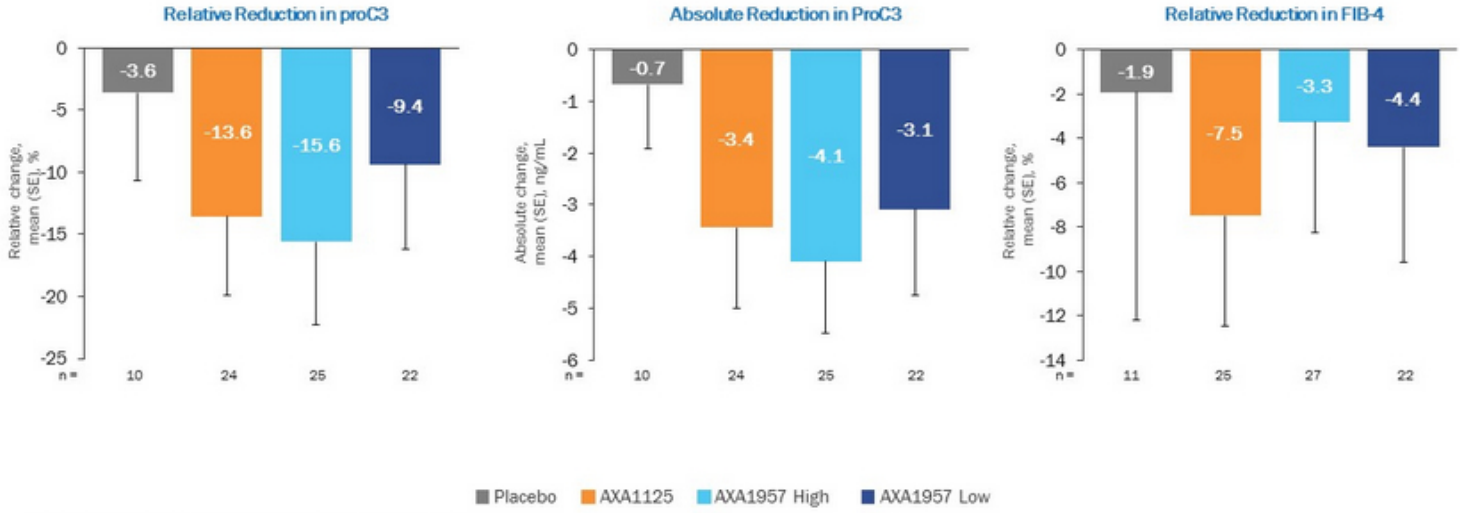


*p<0.05 versus placebo.
ALT, alanine aminotransferase; cT1, corrected T1; SE, standard error.

Equivalent Activity on Fibrosis Markers with AXA1125

Changes from baseline at week 16

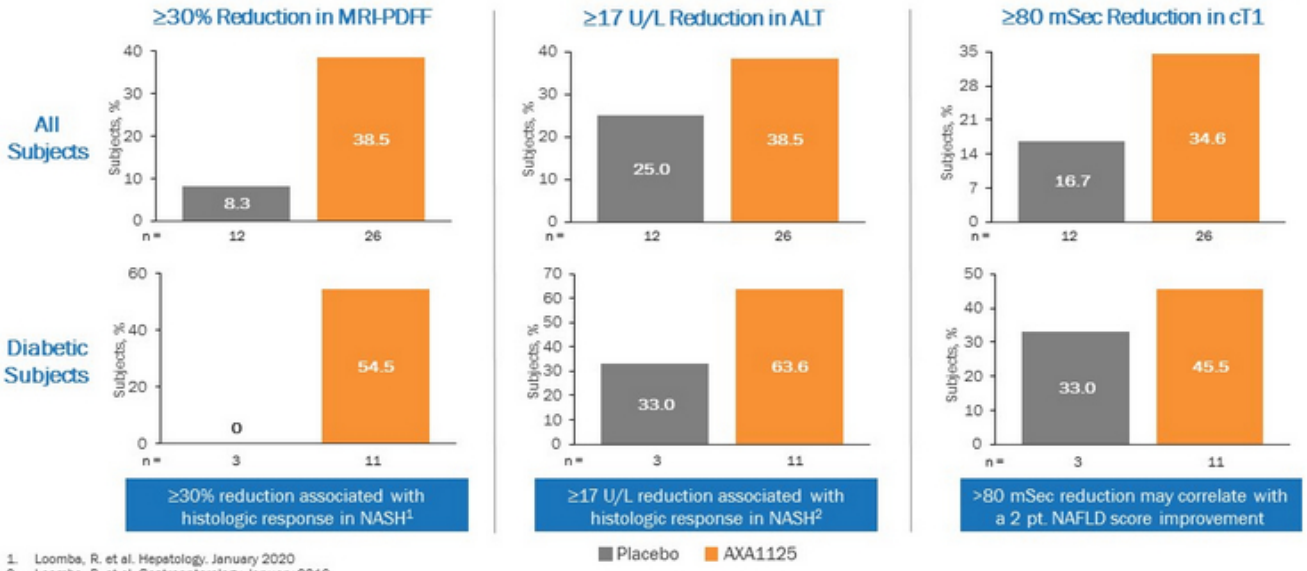




FIB-4, fibrosis 4; ProC3, propeptide of type III collagen; SE, standard error.

AXA1125: Meaningful Thresholds of Activity Achieved

Increasing evidence linking PDFF, ALT and cT1 with improved histological outcomes



1. Loomba, R. et al. Hepatology. January 2020
2. Loomba, R. et al. Gastroenterology. January 2019

ALT, alanine aminotransferase; cT1, corrected T1; MRI-PDFF, magnetic resonance imaging proton density fat fraction; NASH, nonalcoholic steatohepatitis; NAFLD, non-alcoholic fatty liver disease; SE, standard error.

AXA1125 and AXA1957 were Generally Well Tolerated

- AEs were mild to moderate
- Most frequent AEs for AXA1125 involved GI issues:
  - These AEs were generally mild and transient, self-resolving in two to three weeks on average
- Only one discontinuation due to AEs for placebo and AXA1125
- Two SAEs reported; both assessed as unrelated to study product administration
- No meaningful changes seen in lipids or weight in active arms

Subjects with product-emergent AEs, n (%)[1]	Placebo (n=15)	AXA1125 (n=29)	AXA1957 High (n=32)	AXA1957 Low (n=26)
All PEAEs	10 (66.7)	24 (82.8)	19 (59.4)	19 (73.1)
All PEAEs reported in >10% for any arm:				
Diarrhea	1 (6.7)	10 (34.5)	6 (18.8)	3 (11.5)
Nausea	1 (6.7)	4 (13.8)	3 (9.4)	3 (11.5)
Upper respiratory infection	1 (6.7)	4 (13.8)	2 (6.3)	0
Decreased appetite	0	3 (10.3)	1 (3.1)	2 (7.7)
Headache	1 (6.7)	1 (3.4)	2 (6.3)	4 (15.4)

Severity of product-emergent AEs, n (%)[1]		Placebo (n=15)	AXA1125 (n=29)	AXA1957 High (n=32)	AXA1957 Low (n=26)
Diarrhea	Mild	1 (6.7)	6 (20.7)	5 (15.6)	3 (11.5)
	Moderate	0	4 (13.8)	1 (3.1)	0
	Severe	0	0	0	0
Nausea	Mild	1 (6.7)	3 (10.3)	2 (6.3)	1 (3.8)
	Moderate	0	1 (3.4)	1 (3.1)	2 (7.7)
	Severe	0	0	0	0

1. Safety based on what subject received on day 1 of dosing. Subjects counted only once if they had more than one event reported during the product administration period.
AE, adverse event; GI, gastrointestinal; SAE, serious adverse event; PEAEs, product-emergent adverse events

AXA1125: A Potential First-Line NASH Therapy

- AXA1125-003 findings further validate Axcella's platform and approach utilizing EMMs
- Multifactorial activity observed for both AXA1125 and AXA1957, while being well tolerated
- AXA1125 generated more clinically relevant reductions in liver fat content, insulin resistance and fibroinflammation markers when compared with AXA1957
  - Greater activity in key markers seen among subjects with type 2 diabetes receiving AXA1125
- Plan to engage with FDA to discuss an IND application for AXA1125, a proposed Phase 2b clinical trial in adults and Axcella's pediatric development program

EMM, Endogenous Metabolic Modulator; FDA, U.S. Food and Drug Administration; NASH, nonalcoholic steatohepatitis.

Multiple Anticipated Milestones in 2020

THERAPEUTIC PRODUCT CANDIDATES

AREA OF FOCUS	PRODUCT CANDIDATE	POTENTIAL INDICATION	Q1 2020	Q2 2020	Q3 2020[1]	Q4 2020[1]
LIVER	AXA1125	NASH	✓ Reported interim findings from AXA1125-003	✓ Reported top-line data from AXA1125-003	Engage with FDA regarding potential Phase 2b Clinical Trial and pediatric development program	
	AXA1665	Reduction in Risk of Overt Hepatic Encephalopathy Recurrence	✓ Completed enrollment in AXA1665-002		Report top-line data from AXA1665-002	Submit IND application to FDA and initiate potential Phase 2b/3 Clinical Trial[2]
EARLY-STAGE PIPELINE						
AREA OF FOCUS	PRODUCT CANDIDATE	SUBJECTS STUDIED	Q1 2020	Q2 2020	Q3 2020[1]	Q4 2020[1]
BLOOD	AXA4010	Sickle Cell Disease				Report top-line data from Cohort 1 of AXA4010-001

AXA1665 and AXA4010 are being investigated in Non-IND Clinical Studies. Please refer to slides 3 for further detail.
1. Timing based on current expectations and subject to risks associated with the COVID-19 pandemic.
2. We believe that this has the potential to serve as a registrational Clinical Trial, contingent upon final data readout from ongoing Clinical Study and allowance by the FDA.

Our thanks to the subjects and investigators who participated in AXA1125-003

axcella

Participating Sites:

Arizona Liver Health
Chandler & Tucson, AZ

Bioclinical Research
Orlando, FL

Cataline Research Institute
Montclair, CA

Doctors Hospital at Renaissance
Edinburg, TX

Excel Medical Clinical Trials
Boca Raton, FL

Florida Research Institute
Lakewood Ranch, FL

Gastro One
Germantown, TN

Kansas City Research Institute
Kansas City, MO

Liver Center of Texas
Dallas, TX

National Research Institute
Los Angeles, Huntington Park & Panorama City, CA

Pinnacle Clinical Research
San Antonio & Austin, TX

Southern Therapy & Advanced Res.
Jackson, MS

Texas Digestive Disease Consultants
Fort Worth, TX