BEAM-302 Topline Data Update

March 25, 2026

NASDAQ: BEAM

Beam THERAPEUTICS

Beam event participants

TOPIC	PARTICIPANT
Introduction	**Holly Manning** Vice President, Investor Relations & External Communications
Beam Overview	**John Evans** Chief Executive Officer
BEAM-302 Topline Data	**Amy Simon, M.D.** Chief Medical Officer
AATD Overview and Physician Perspective	**Jeffrey Teckman, M.D.** Saint Louis University
Next Steps for Pivotal Development & Closing Remarks	**Giuseppe Ciaramella, Ph.D.** President
Q&A	**Mr. Evans, Dr. Simon, Dr. Teckman & Dr. Ciaramella**

Cautionary note regarding forward-looking statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements include statements regarding: the therapeutic applications and potential of our technology, including with respect to AATD; our plans, and anticipated timing, to advance our programs, including risto-cel, BEAM-103, BEAM-301, BEAM-304 and BEAM-302; our plans and anticipated timing to present data from ongoing clinical trials; our anticipated regulatory interactions and filings; our current expectations and anticipated results of operations, including our expected use of capital; the sufficiency of our capital resources to fund operating expenses and capital expenditure requirements and the period in which such resources are expected to be available; and the therapeutic applications and potential of our technology, including our potential to develop lifelong, curative, precision genetic medicines for patients through base editing, including potential safety advantages, all of which are subject to known and unknown important risks, uncertainties and other factors that may cause our actual results, performance or achievements, market trends, or industry results to differ materially from those expressed or implied by such forward-looking statements. Therefore, any statements contained herein that are not statements of historical fact may be forward-looking statements and should be evaluated as such. Without limiting the foregoing, the words "anticipate," "expect," "suggest," "plan," "vision," "strategy," "possibility," "promise," "believe," "intend," "project," "forecast," "estimates," "targets," "projections," "potential," "should," "could," "would," "may," "might," "will," and the negative thereof and similar words and expressions are intended to identify forward-looking statements. Each forward-looking statement is subject to important risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement, including, without limitation, risks and uncertainties related to: our ability to develop, obtain regulatory approval for, and commercialize our product candidates, which may take longer or cost more than planned; our ability to raise additional funding, which may not be available; our ability to obtain, maintain and enforce patent and other intellectual property protection for our product candidates; that preclinical testing of our product candidates and preliminary or interim data from preclinical studies and clinical trials may not be predictive of the results or success of ongoing or later clinical trials; that initiation and enrollment of our clinical trials may take longer than expected; that our product candidates or the delivery modalities we rely on to administer them may cause serious adverse events; the uncertainty that our product candidates will receive regulatory approval necessary to initiate or continue human clinical trials, that our product candidates may experience manufacturing or supply interruptions or failures; risks related to competitive products; and the other risks and uncertainties identified under the headings "Risk Factors Summary" and "Risk Factors" and elsewhere in our annual report on Form 10-K for the year ended December 31, 2025, our quarterly reports on Form 10-Q, and in any subsequent filings with the Securities and Exchange Commission (the "SEC") which are available on the SEC's website at www.sec.gov. Additional information will be made available by our annual and quarterly reports and other filings that we make from time to time with the SEC. These forward-looking statements speak only as of the date of this presentation. Factors or events that could cause our actual results to differ may emerge from time to time, and it is not possible for us to predict all of them. We undertake no obligation to update any forward-looking statement, whether as a result of new information, future developments or otherwise, except as may be required by applicable law.

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Our vision is to provide lifelong cures
for patients suffering from serious diseases
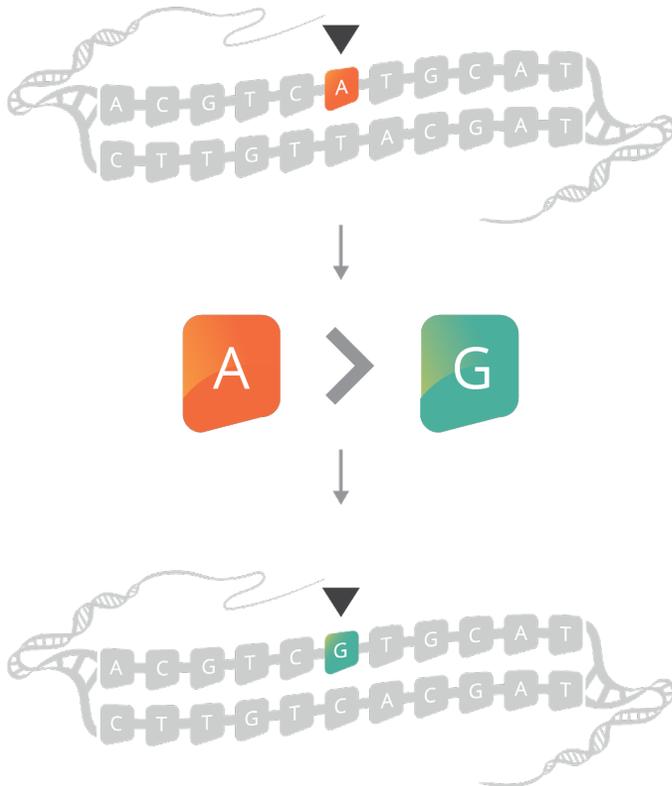
GENE EDITING FOR
rare and common diseases

POTENTIAL FOR
one-time, curative therapies

PLATFORM FOR
rapidly programmable precision medicines

Beam was founded on a simple concept with profound implications

BASE EDITING
TECHNOLOGY
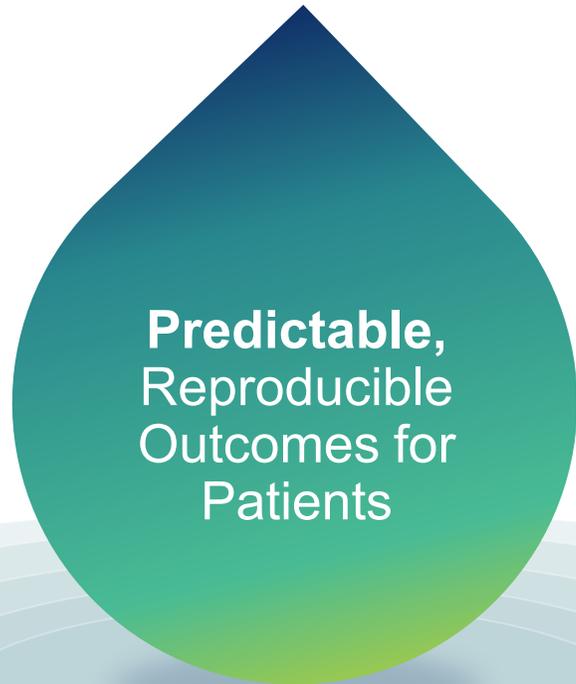
A > G

CONSISTENT gene sequence outcomes

DURABLE correction for one-time cures

LESS GENOTOXICITY than traditional gene editing

Predictable, Reproducible Outcomes for Patients

Predictability as a driver of progress:
The potential to ripple through the broader healthcare ecosystem

Predictable,
Reproducible
Outcomes for
Patients

Streamlined R&D Cycles
- Reduced development risk

Regulatory Acceleration
- Efficient regulatory pathways

Physician Confidence
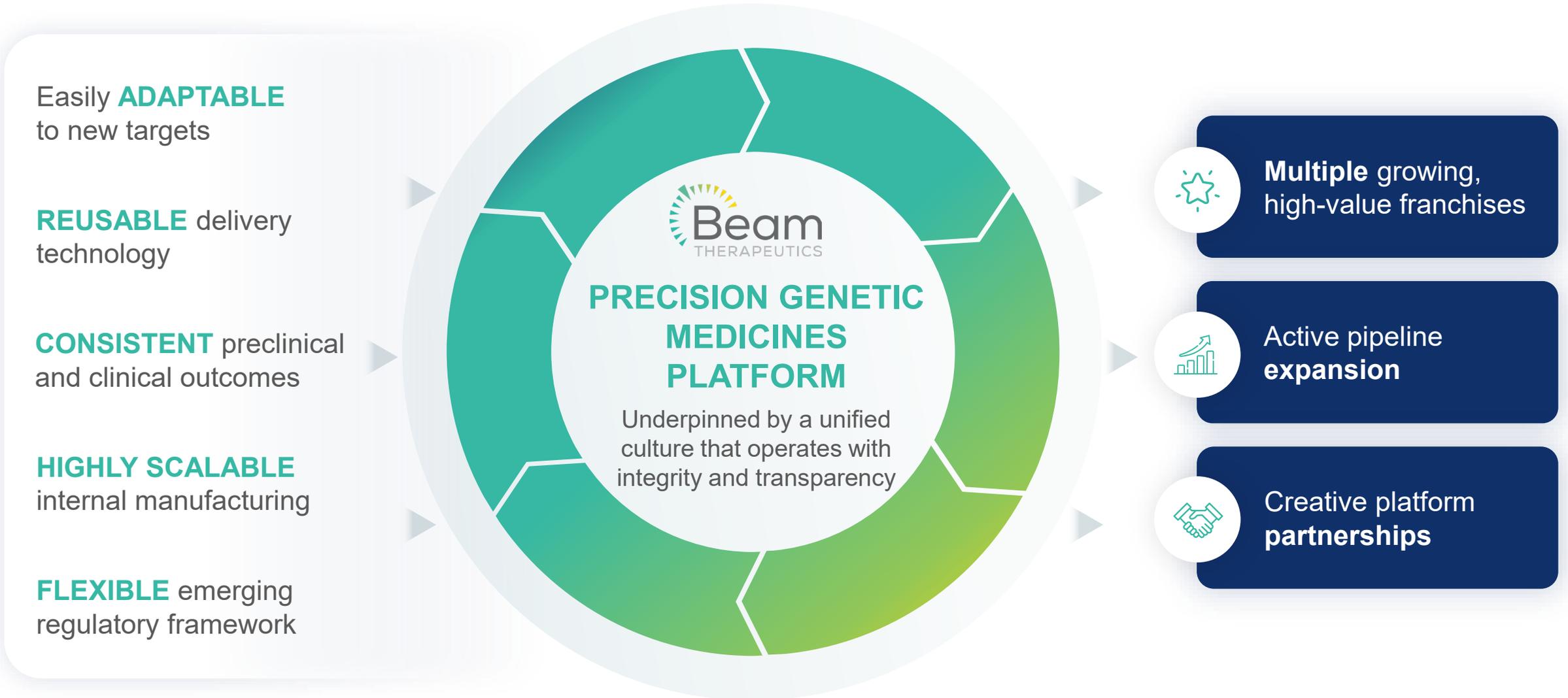- Predictable safety and durability
- Clarity in treatment decisions

Patient Experience Improvement
- Reliable therapeutic outcomes
- Enhanced quality of daily living

Payer & System Impact
- Sustainable, outcomes-aligned payer models
- Reduced lifetime healthcare utilization

The power of predictability:
Beam is building a reliable model for advancing genetic medicine

Easily **ADAPTABLE** to new targets

REUSABLE delivery technology

CONSISTENT preclinical and clinical outcomes

HIGHLY SCALABLE internal manufacturing

FLEXIBLE emerging regulatory framework

PRECISION GENETIC MEDICINES PLATFORM

Underpinned by a unified culture that operates with integrity and transparency

Multiple growing, high-value franchises

Active pipeline **expansion**

Creative platform **partnerships**

Rapidly advancing and growing a portfolio of liver-targeted *in vivo* programs for genetic diseases

Potential **best-in-class** and **first-in-class** AATD program

Strategic **pipeline expansion into PKU**

Industry-leading LNP capabilities

Platform synergies potentially enable use of novel regulatory pathways and established clinical footprint

PROGRAM	DISEASE	DELIVERY	EDITING APPROACH	RESEARCH	LEAD OPTIMIZATION	IND ENABLING	PHASE I/II	PIVOTAL
BEAM-302	Alpha-1 antitrypsin deficiency (AATD)	*In vivo* LNP	Correction of E342K mutation					
BEAM-301	Glycogen storage disease type Ia (GSDIa)	*In vivo* LNP	Correction of R83C mutation					
BEAM-304	Phenylketonuria (PKU)	*In vivo* LNP	Correction of multiple mutations					

LNP = lipid nanoparticle

Updated data from BEAM-302 Phase 1/2 trial support potential as best-in-class and first-in-class one-time treatment for AATD

Treatment with single dose 60 mg **BEAM-302 achieved durable levels of total AAT into the MZ range and a more favorable M-AAT to Z-AAT ratio with follow-up out to 12 months**

- Steady-state mean **total AAT level was 16.1 μM***, with all patients consistently >11 μM protective threshold important for lung health
- **Mutant Z-AAT reduced 84%**, important for liver function and health
- Newly produced M-AAT was **functional** and **comprised 94% of total AAT**

Strong evidence of inducibility, with **a patient reaching ~30 μM** (~95% M-AAT) during respiratory infection

Well-tolerated safety profile with transient Grade 1 transaminase elevations in single-dose cohorts

Based on strength of safety and efficacy profile of BEAM-302 in single dose cohorts, **60 mg chosen as optimal biological dose for pivotal trial**

Data cutoff February 10, 2026
*Values shown are the mean of subject-level steady-state mean values, which is defined as the period beginning on a patient's Day 28 visit and lasting until that patient's Month 12 visit (or until that patient's last visit, if earlier than twelve months).

Topline BEAM-302 Dose-escalation Clinical Data

Amy Simon, M.D., Chief Medical Officer

Severe AATD (PiZZ genotype) impacts >100,000 individuals in the U.S. with limited treatment options

Single G to A point mutation in the SERPINA1 gene
(PiZ or "Z" mutation)
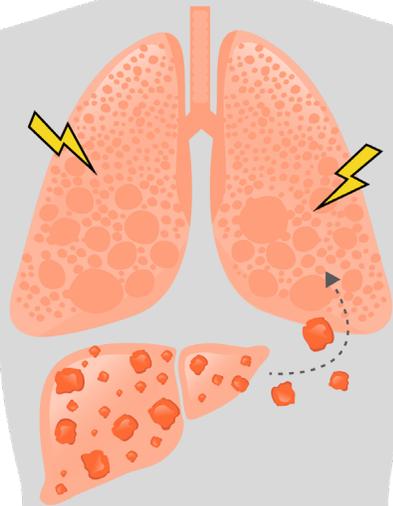
Progressive lung disease due to:

- Low and poorly functioning systemic Z-AAT levels

- Circulating Z-AAT aggregates, causes inflammation

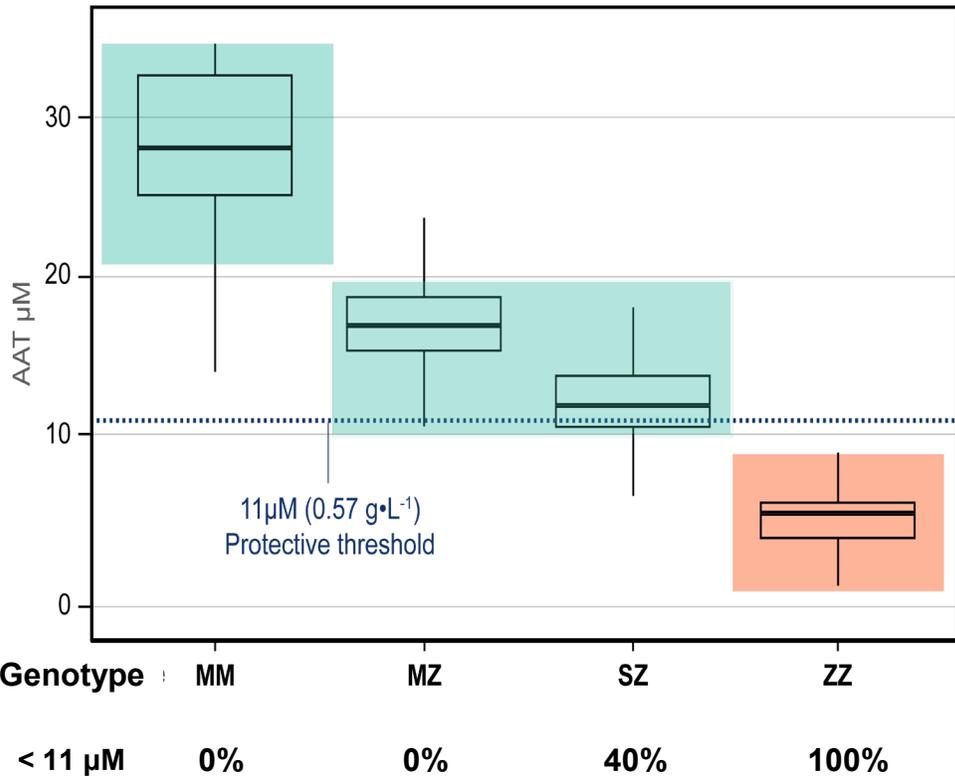✕ Routine **COPD care**

✕ IV augmentation therapy given **weekly is only approved option**

Progressive liver disease with fibrosis and cirrhosis due to:

- Aggregation and accumulation of mutant Z-AAT

✕ **Supportive care and liver transplant** for advanced disease

✕ **No approved treatments** for liver disease

AAT protective threshold >11 µM informed by clinical genetics, translates into marked risk reduction for AATD lung and/or liver disease
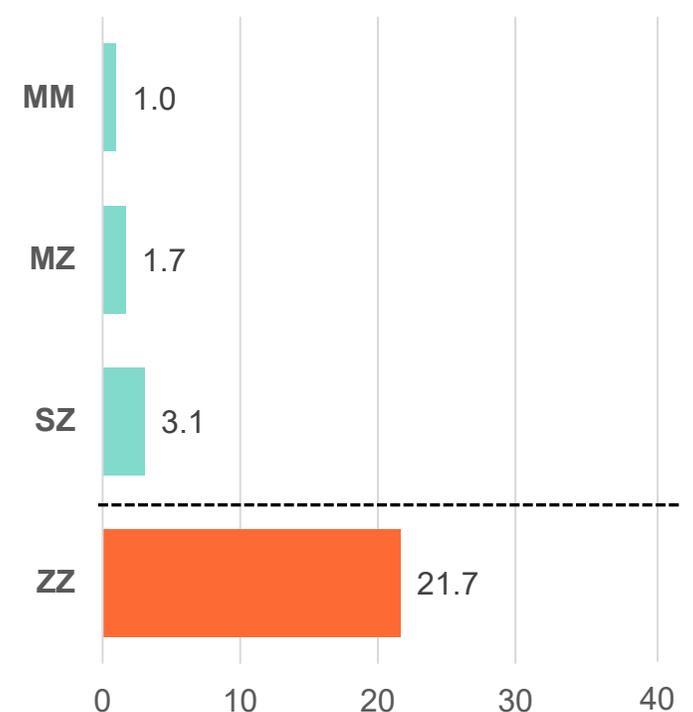

AAT Levels Across Genotypes

11µM (0.57 g•L^{-1})
Protective threshold

Genotype	MM	MZ	SZ	ZZ
< 11 µM	0%	0%	40%	100%

Emphysema Risk

- MM — 1.0
- MZ — 1.6
- SZ — 1.6
- ZZ — 35.2
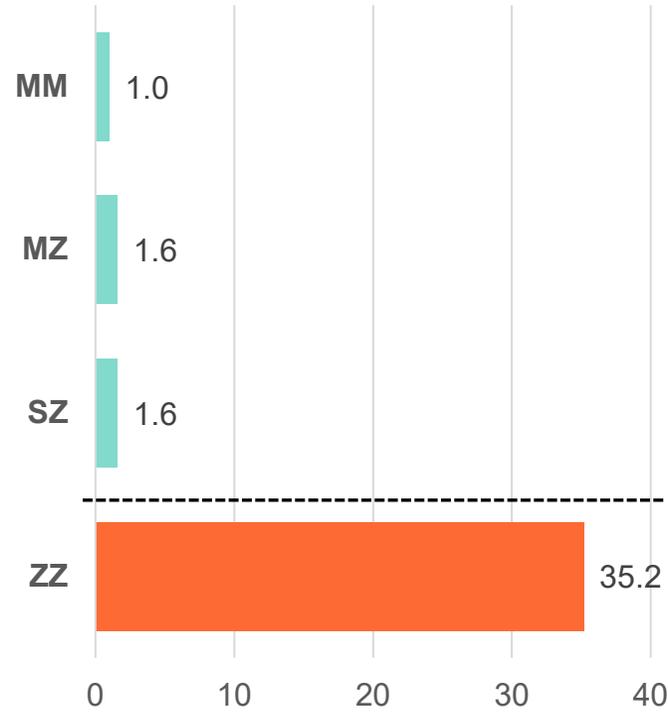
Liver Fibrosis / Cirrhosis Risk

- MM — 1.0
- MZ — 1.7
- SZ — 3.1
- ZZ — 21.7

Disease in MZ and SZ individuals requires presence of additional risk factor, e.g., smoking or obesity

BEAM-302 is a potential one-time therapy that uses base editing to directly correct the E342K mutation causing AATD

BEAM-302 BASE EDITOR

PiZ

E342K mutation

CRISPR Protein

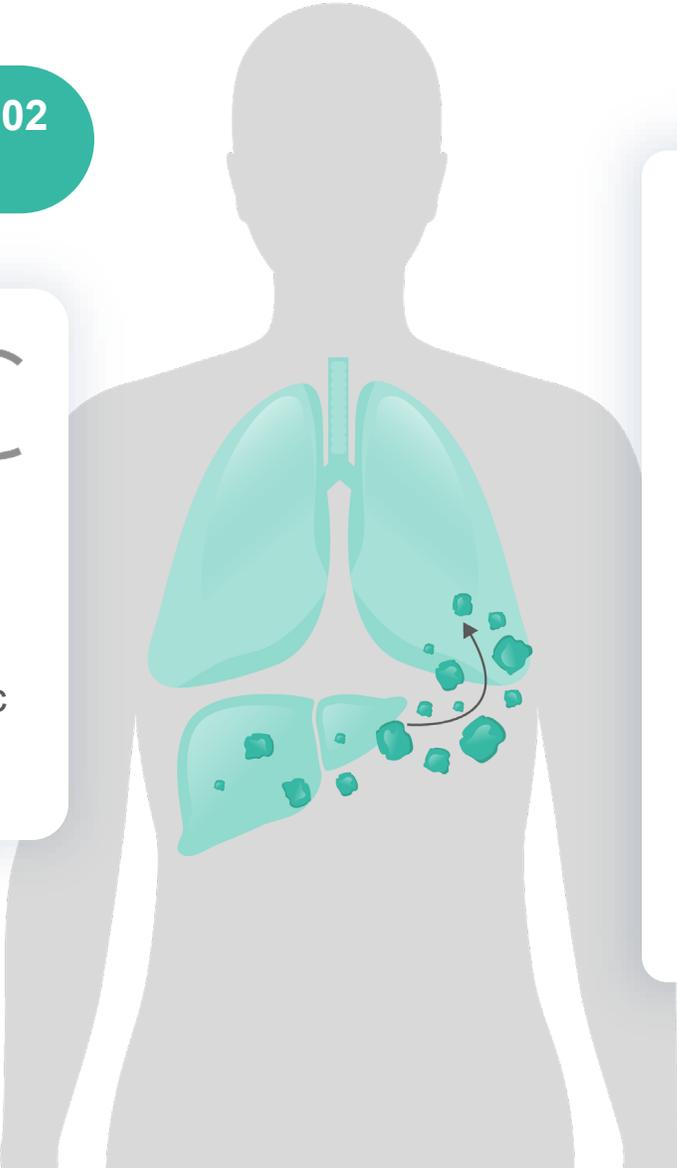Deaminase

Guide RNA

A ▸ G

PiM

Corrected SERPINA1 gene
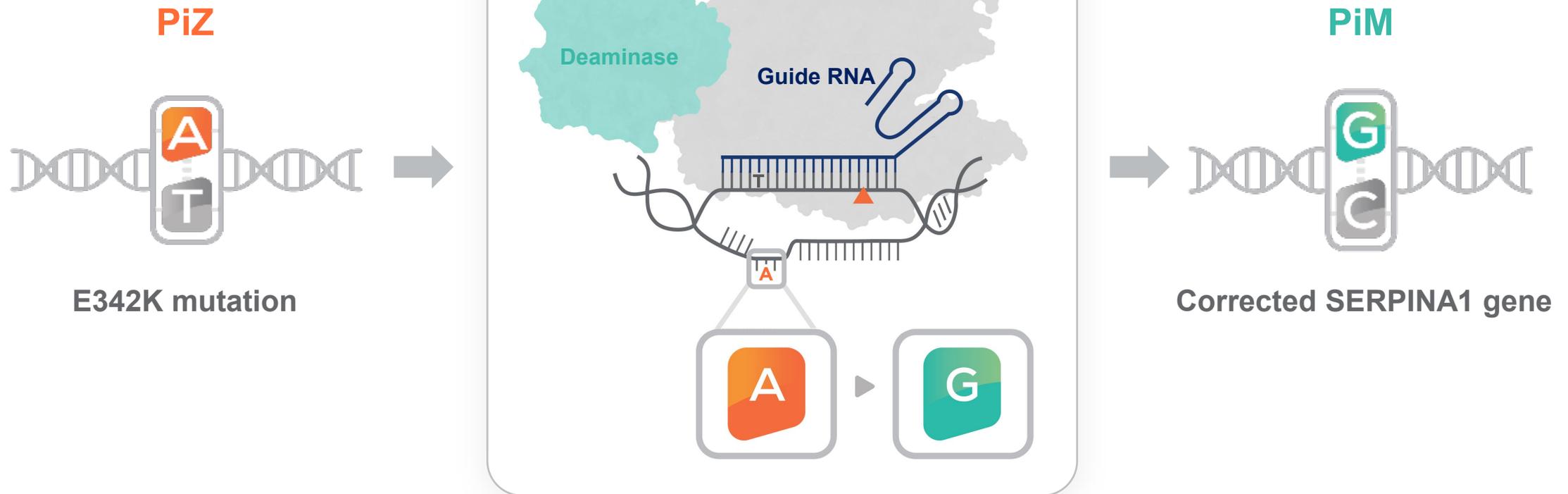
BEAM-302 has the potential to be first, one-time treatment to address full spectrum of disease manifestations of AATD

GOALS OF BEAM-302 TREATMENT

Correction at root cause of disease

Restore physiologic control of AAT

- **Liver produces M-AAT** for the first time

- Significantly **reduces Z-AAT**

- Total AAT **above 11 μM protective threshold**

- Increased total **AAT is functional**

- AAT increases with **inflammatory response**

✓ **Durable, single-course** treatment
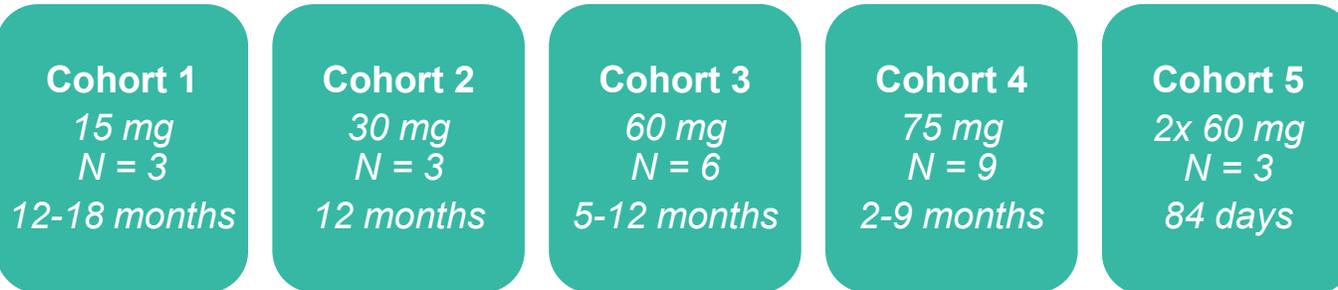
✓ **Address both lung and liver** manifestations

Significant momentum in clinical execution of BEAM-302 Phase 1/2 trial across spectrum of AATD

PART A:
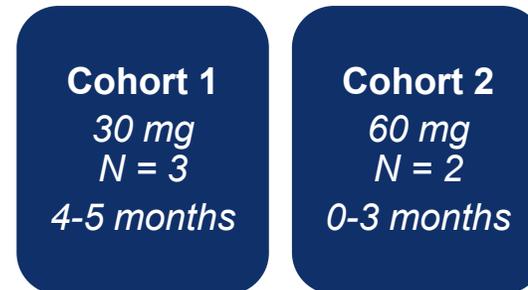
AATD-associated Lung Disease
DOSE ESCALATION

RANGE OF FOLLOW-UP

Cohort 1	**Cohort 2**	**Cohort 3**	**Cohort 4**	**Cohort 5**
15 mg	*30 mg*	*60 mg*	*75 mg*	*2x 60 mg*
N = 3	*N = 3*	*N = 6*	*N = 9*	*N = 3*
12-18 months	*12 months*	*5-12 months*	*2-9 months*	*84 days*

PART B:

AATD-associated Liver Disease with or without Lung Disease
DOSE ESCALATION

RANGE OF FOLLOW-UP

Cohort 1	**Cohort 2**
30 mg	*60 mg*
N = 3	*N = 2*
4-5 months	*0-3 months*

- Goal: assess early safety and efficacy and identify optimal dose for pivotal study

- Achieved first ever clinical proof of concept of *in vivo* base editing, leading to correction of a disease-causing mutation

- Established clinical and operational footprint to support future pivotal cohort

Single-dose BEAM-302 demonstrated a well-tolerated safety profile in 26 patients up to 75 mg, consistent across Parts A and B

PART A:

AATD-associated Lung Disease | *N=21*

PART B:

AATD-associated Liver Disease with or without Lung Disease | *N=5**

- Safety profile consistent across single-dose Part A and Part B cohorts
- No serious AEs, DLTs or ≥Grade 3 AEs observed
- All TEAEs were mild to moderate
- Grade 1 asymptomatic, transient elevations in ALT (+/- AST) observed within first 28 days
- Grade 1-Grade 2 IRRs observed; all resolved within a day

Data cutoff February 10, 2026
*Safety results for second patient dosed at 60 mg in Part B were obtained after the February 10, 2026, data cutoff.
AE: adverse events, DLT: dose-limiting toxicity; TEAE: treatment emergent adverse events; ALT: alanine aminotransferase; AST: aspartate aminotransferase; IRR: infusion related reaction

Multi-dose cohort (n=3) experienced IRRs and transaminase elevations following second dose of BEAM-302

- 2 patients experienced higher transaminase increases within days post second dose of 60 mg BEAM-302
  - Grade 4 ALT and Grade 3 AST increases occurred in one patient, which were asymptomatic and resolved without treatment
  - Grade 2 ALT increase occurred in another patient; resolved without treatment

- No clinical signs of liver dysfunction or bilirubin increases observed

- Grade 2 IRRs occurred in all patients; all resolved

BEAM-302 treatment led to durable increases in total AAT into MZ carrier range at single doses ≥60 mg

- Subject-level steady-state mean total AAT levels consistently and durably >11 µM protective threshold for all patients treated with 60 mg

- Increased total AAT in circulation was functional as demonstrated by neutrophil elastase inhibition

- Fold change from baseline was comparable between the 60 mg and 75 mg single dose cohorts, suggesting near saturation editing at doses ≥60 mg

BEAM-302 dose	N	Baseline total AAT, mean	Total AAT (steady state*), mean
15 mg	3	5.2 µM	8.4 µM
30 mg	3	5.6 µM	9.9 µM
60 mg	**6**	**6.6 µM**	**16.1 µM**
75 mg	**9**	**6.3 µM**	**14.4 µM**

Z-AAT durably reduced ~80% in highest single dose cohorts, with proportion of M-AAT greater than that of MZ genotype

Dose	N	% change in Z-AAT (steady state), mean
15 mg	3	-15%
30 mg	3	-40%
60 mg	**6**	**-84%**
75 mg	**9**	**-79%**

M-AAT comprises majority of total AAT in circulation*
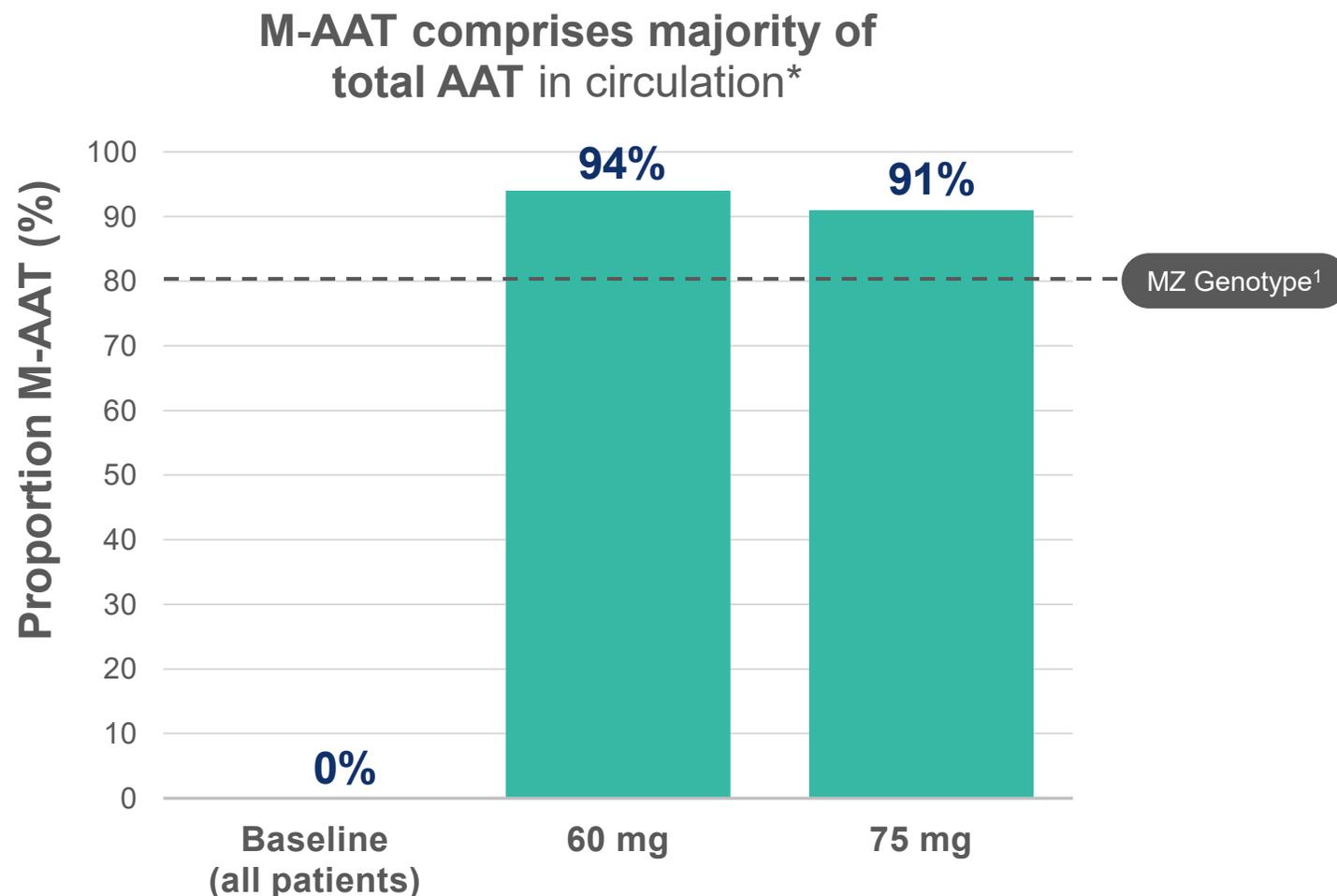
Data cutoff February 10, 2026
*Percentages for the 60 mg and 75 mg cohorts represent the mean of subject-level mean % M-AAT over the steady state.
[1]Donato et al. Respiratory Research (2015).

Part B patients with AATD-related liver disease show similar efficacy trends as Part A patients without liver disease

- Preliminary data demonstrate that increases in total AAT, % M-AAT, and reduction in % Z-AAT levels are similar between patients with liver disease and those without

BEAM-302 dose	N	Baseline total AAT, mean	Total AAT (steady state), mean	Proportion M-AAT (steady state), mean	% change in Z-AAT (steady state), mean
30 mg	3	6.8 μM	12.5 μM	75%	-51%
60 mg	1*	5.5 μM	17.2 μM	95%	-86%

Data cutoff February 10, 2026
*Second patient dosed at 60 mg in Part B was not efficacy evaluable at the time of data cutoff.

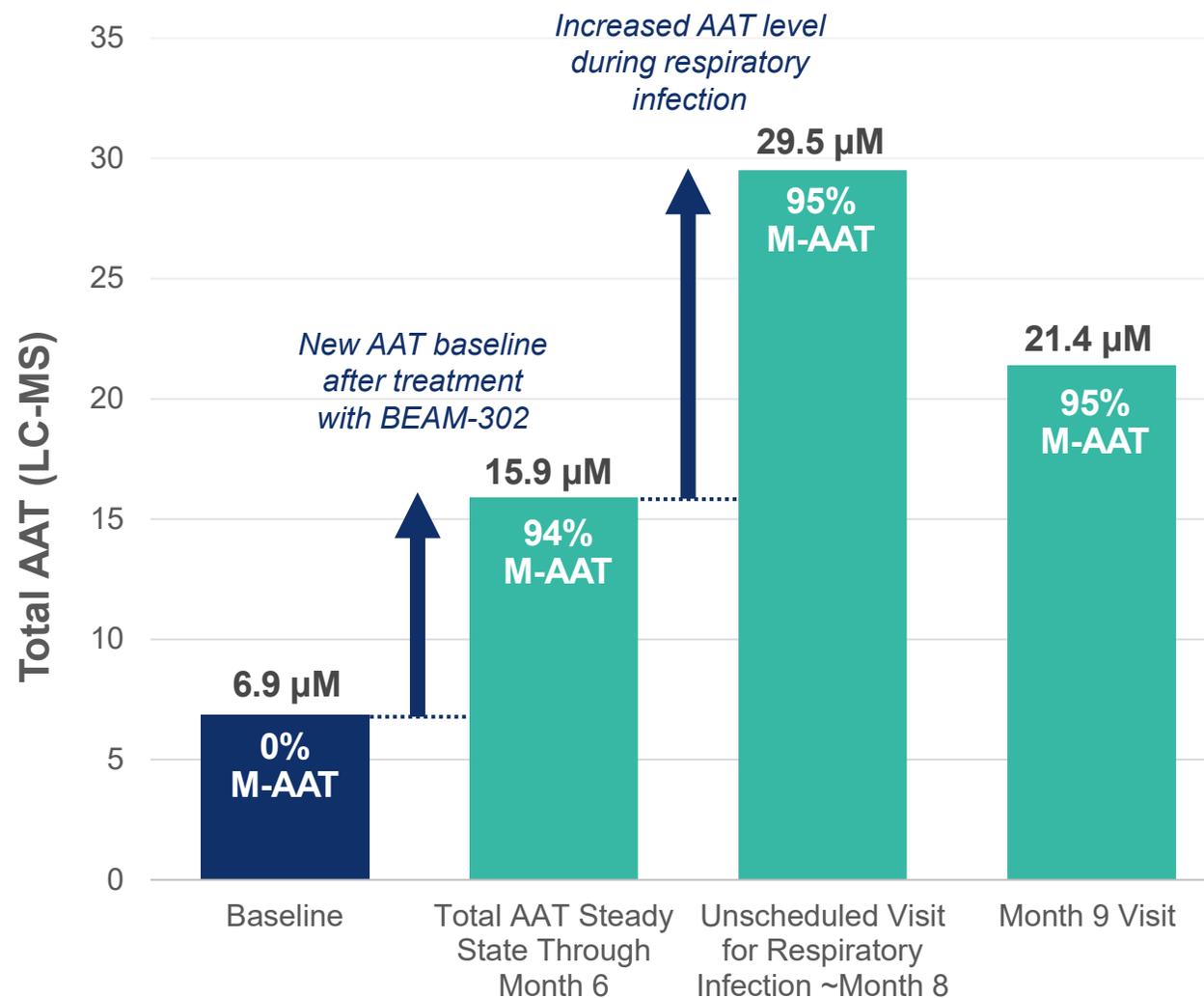# Multi-dose cohort (n=3) showed consistent efficacy profile with single 60 mg dose cohort

- Multi-dose cohort patients achieved a mean of 16.5 µM total AAT at Day 84, 28 days following the second dose of 60 mg BEAM-302
  - Baseline total AAT mean was 5.5 µM

- Z-AAT was reduced by 80% and the proportion of M-AAT was 93% following the second dose

- Early data suggest single dose of 60 mg BEAM-302 has achieved near saturation editing

Data cutoff February 10, 2026

Post-treatment AAT was inducible to ~30 µM in a patient with a respiratory infection, retained ~95% M-AAT

- During inflammation, lungs require higher AAT levels to maintain protection from tissue-damaging proteases

- Patient treated with 60 mg BEAM-302 experienced a respiratory infection in Month 8
  - Elevated CRP confirms inflammation
  - Concomitant increase in total AAT to ~30 µM
  - AAT composition of ~95% M-AAT sustained throughout event

- Demonstrates strong, dynamic production of corrected AAT upon physiologic demand

M-AAT composition was calculated as the percentage of M-AAT isoforms of the total M-AAT and Z-AAT isoforms measured by LC-MS.

Updated data from BEAM-302 Phase 1/2 trial support potential as best-in-class and first-in-class one-time treatment for AATD

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- **Mutant Z-AAT reduced 84%**, important for liver function and health
- Newly produced M-AAT was **functional** and **comprised 94% of total AAT**

Strong evidence of inducibility, with **a patient reaching ~30 µM** (~95% M-AAT) during respiratory infection
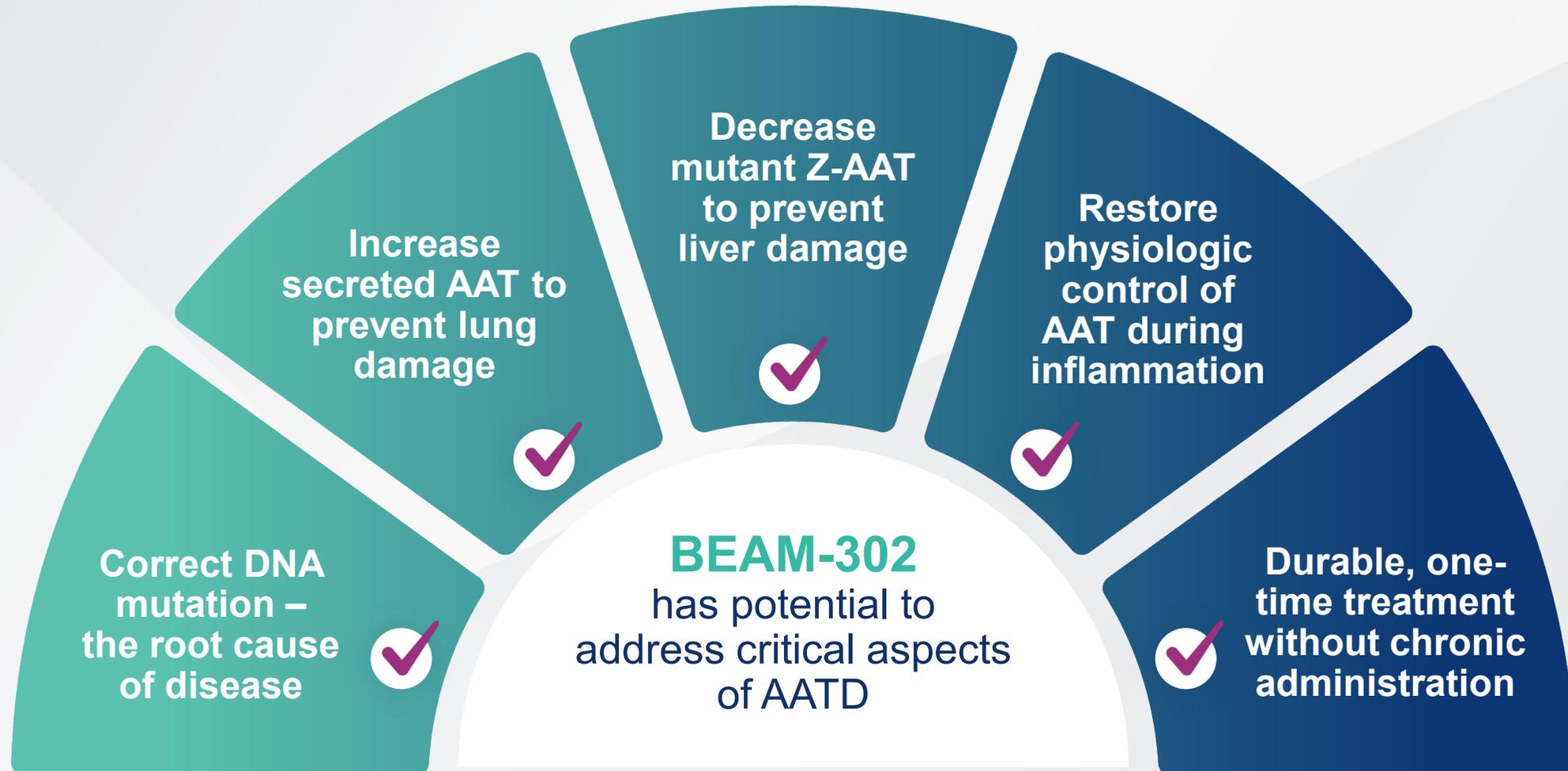
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BEAM-302 has the potential to be the first one-time treatment for both lung and liver manifestations of AATD

Increase secreted AAT to prevent lung damage

Decrease mutant Z-AAT to prevent liver damage

Restore physiologic control of AAT during inflammation

Correct DNA mutation – the root cause of disease

BEAM-302 has potential to address critical aspects of AATD

Durable, one-time treatment without chronic administration

A Clinical Perspective on AATD

Jeffrey Teckman, M.D., Saint Louis University

Background

Why alpha-1-antitrypsin deficiency now?

- Increased awareness in hepatology field of liver involvement in AATD and of "steatotic liver disease" in general, with more focus on non-invasive testing for detection and follow up

- Increased diagnosis of lung affected, but still suboptimal treatment options
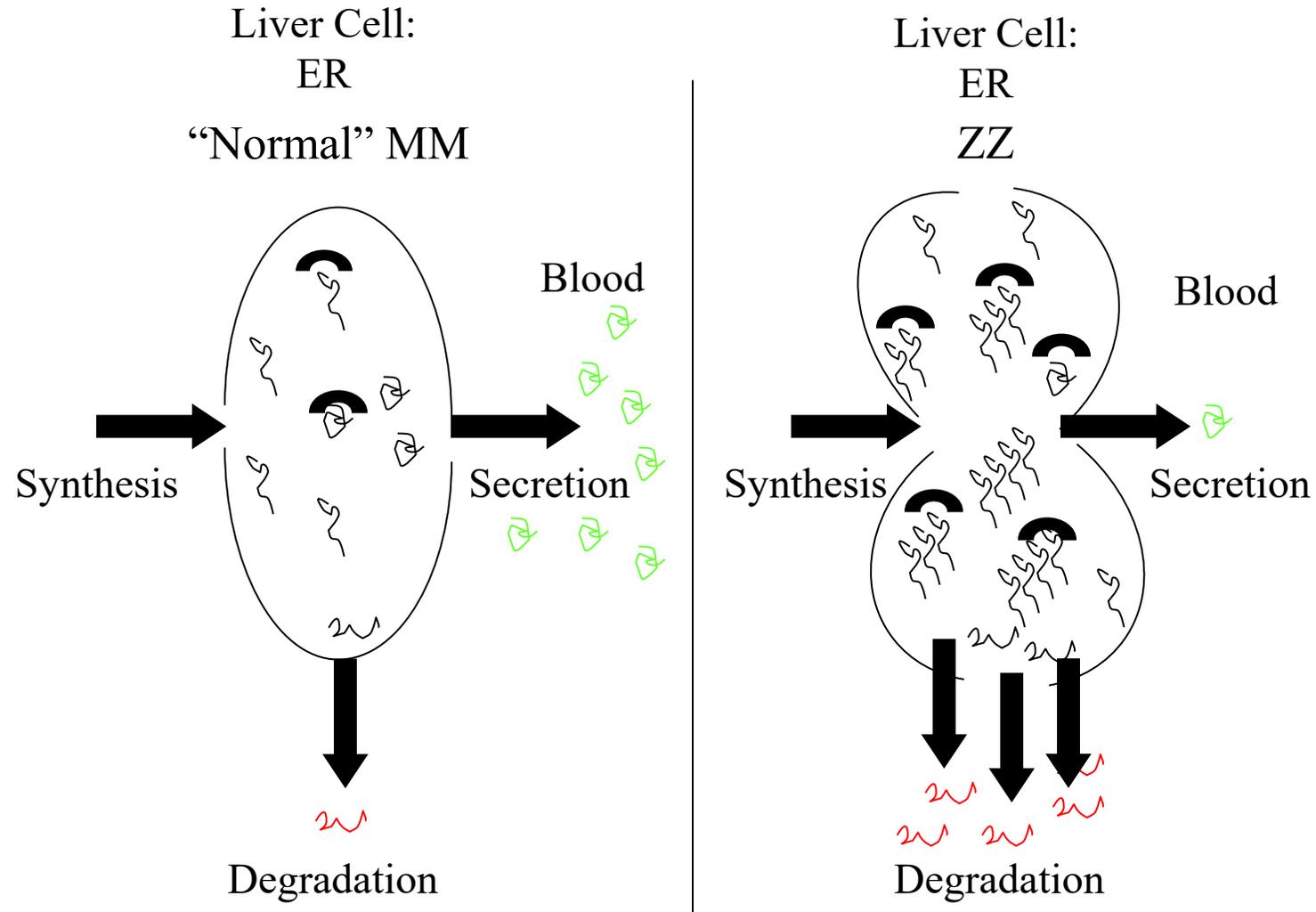
- Active and centralized patient foundation with registry and active research recruitment

- Autosomal recessive / co-dominant genetic disease.

- M - normal, wild type allele

- Z - severe disease allele

- S - intermediate severe disease allele

Background

- **Liver:** storage disease leading to fibrosis, cirrhosis and hepatocellular carcinoma

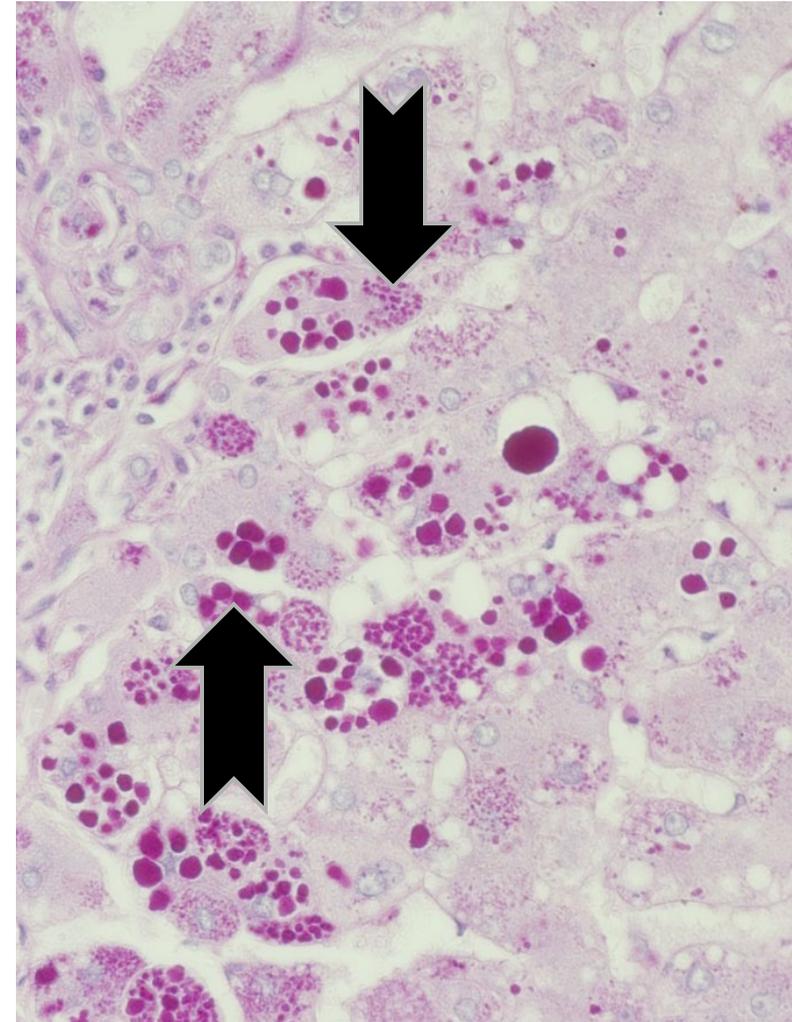- **Lung:** serum deficiency of anti-protease activity leading to lung destruction

AAT Processing in Liver

Liver Cell:
ER
"Normal" MM

Synthesis

Secretion

Blood

Degradation

Liver Cell:
ER
ZZ

Synthesis

Secretion

Blood

Degradation

Human ZZ Liver Pathology
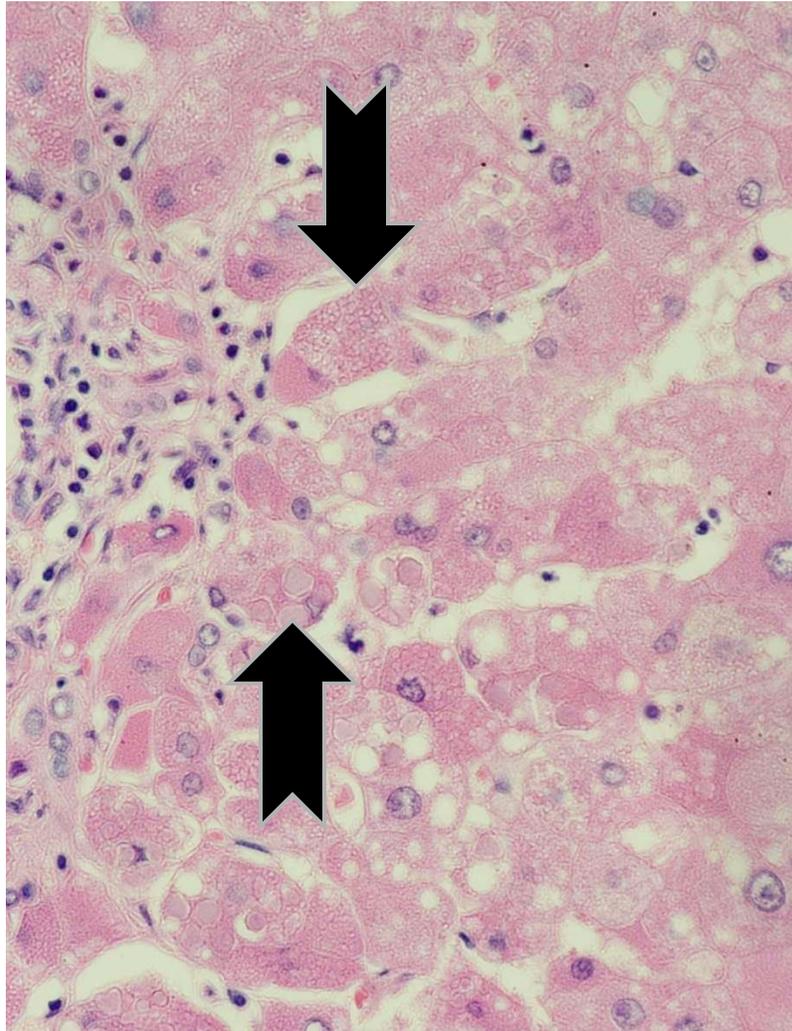
H&E

PAS with Digestion

Purple Stain: misfolded Z-AAT globules
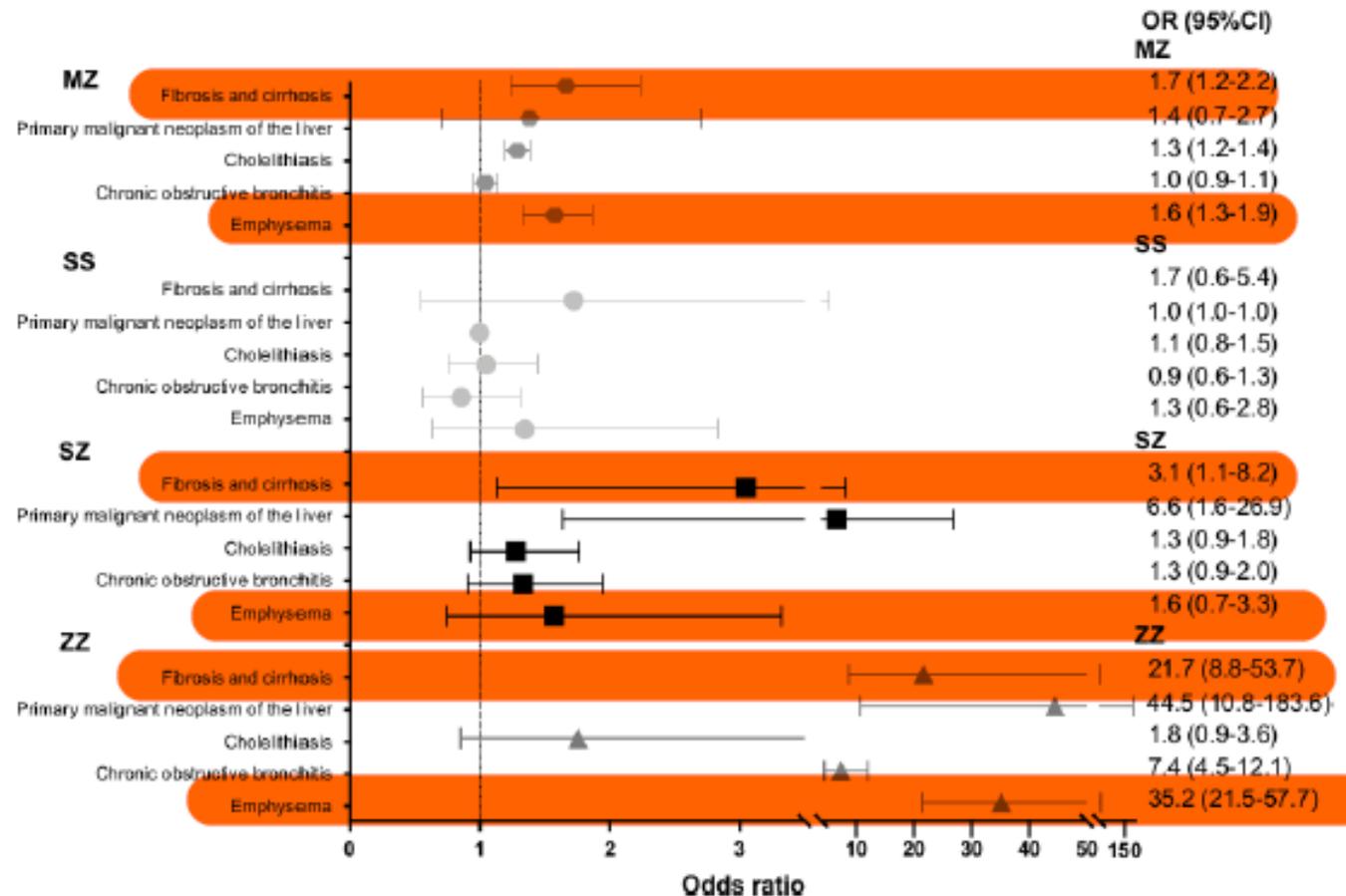
Risk of Liver Disease and Lung Disease by Genotype

Figure 5 Odds ratios (ORs) of ICD10 diagnoses in individuals heterozygous or homozygous for the Pi*Z variant (Pi*MZ/Pi*ZZ), homozygous for Pi*S variant (Pi*SS) and heterozygous for Pi*S and Pi*Z (Pi*SZ) (cohort 1). Adjusted OR (aOR) with their corresponding 95% CI are shown for Pi*MZ, Pi*SS, Pi*SZ and Pi*ZZ subjects compared with non-carriers. ORs were adjusted for age, sex, body mass index, alcohol consumption and diabetes mellitus. If in one group no cases are available, the corresponding aOR was set as 1 [1;1].

Percent ZZ individuals with medically significant or clinically recognizable liver or lung disease by age
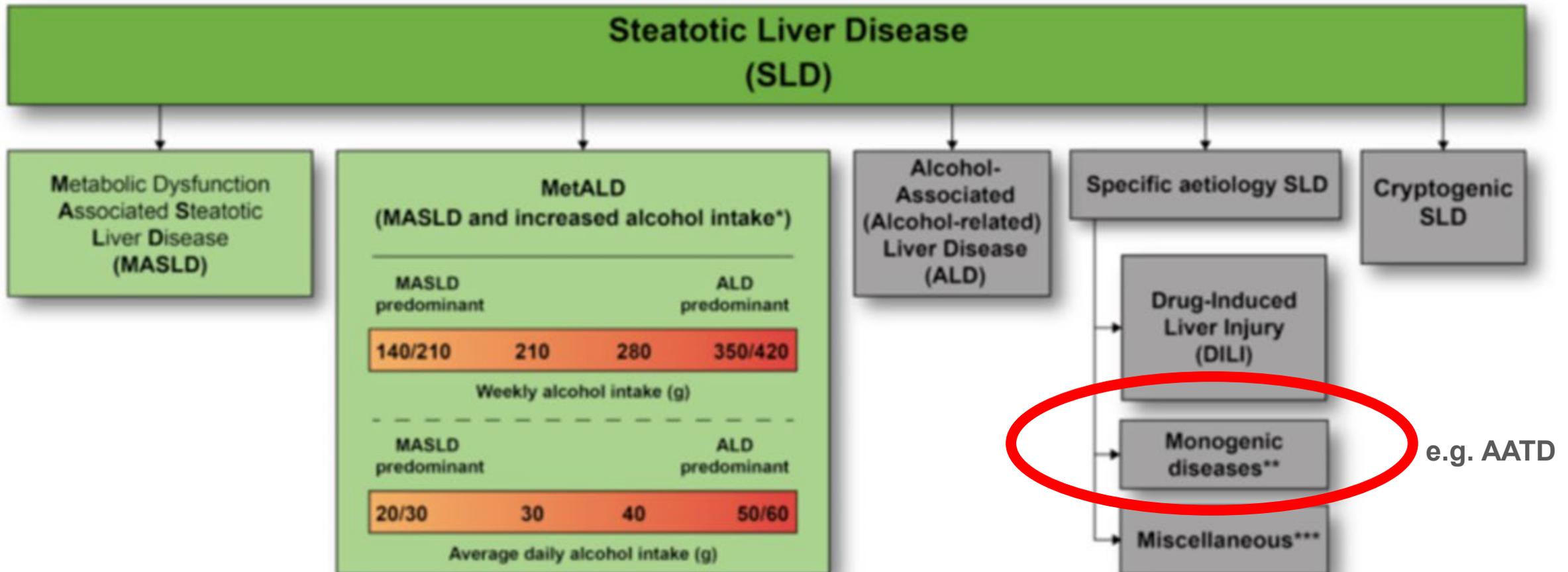
Many may be symptomatic but carrying an incorrect diagnosis
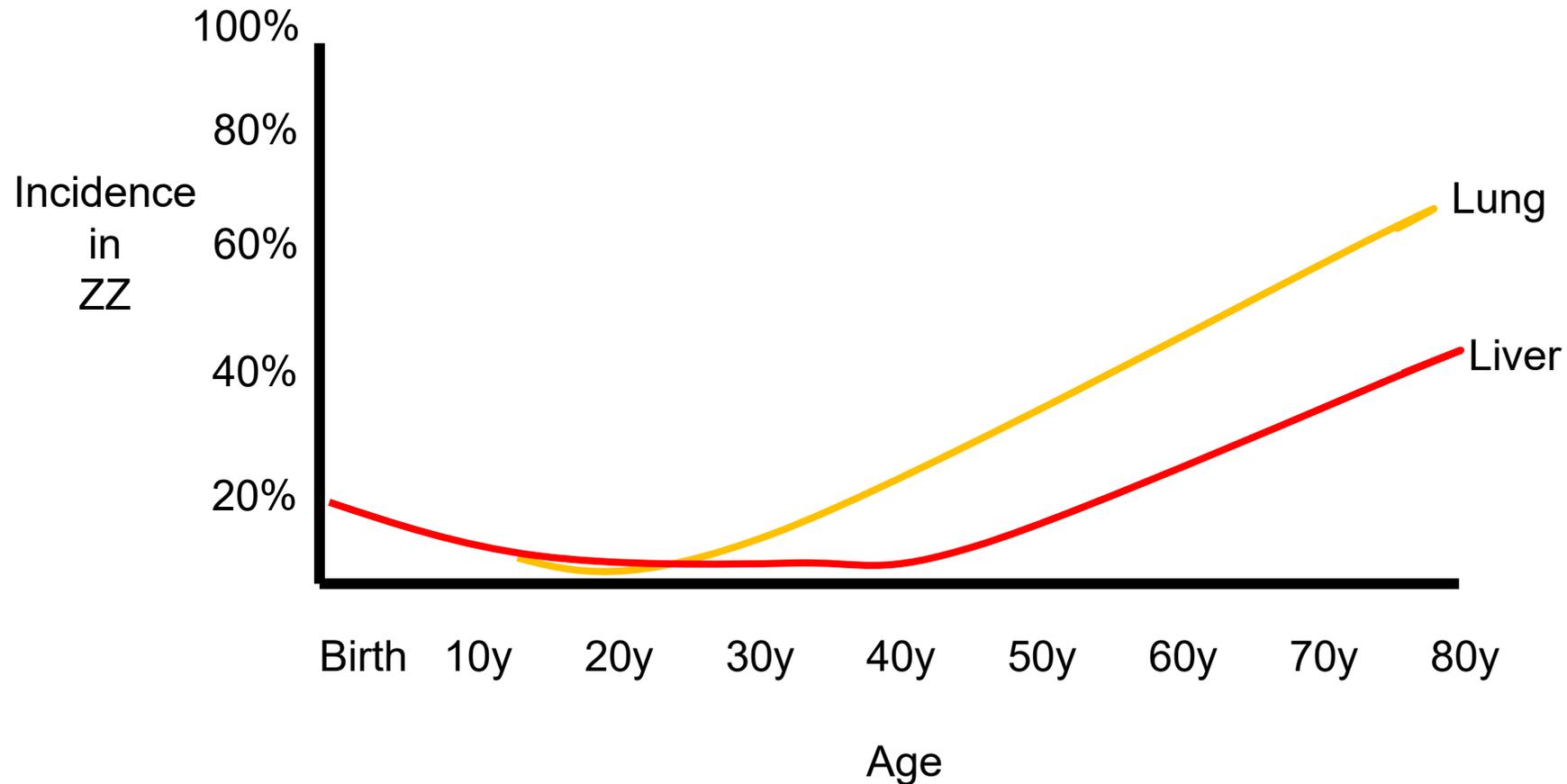
American Association for the Study of Liver Diseases; 2025

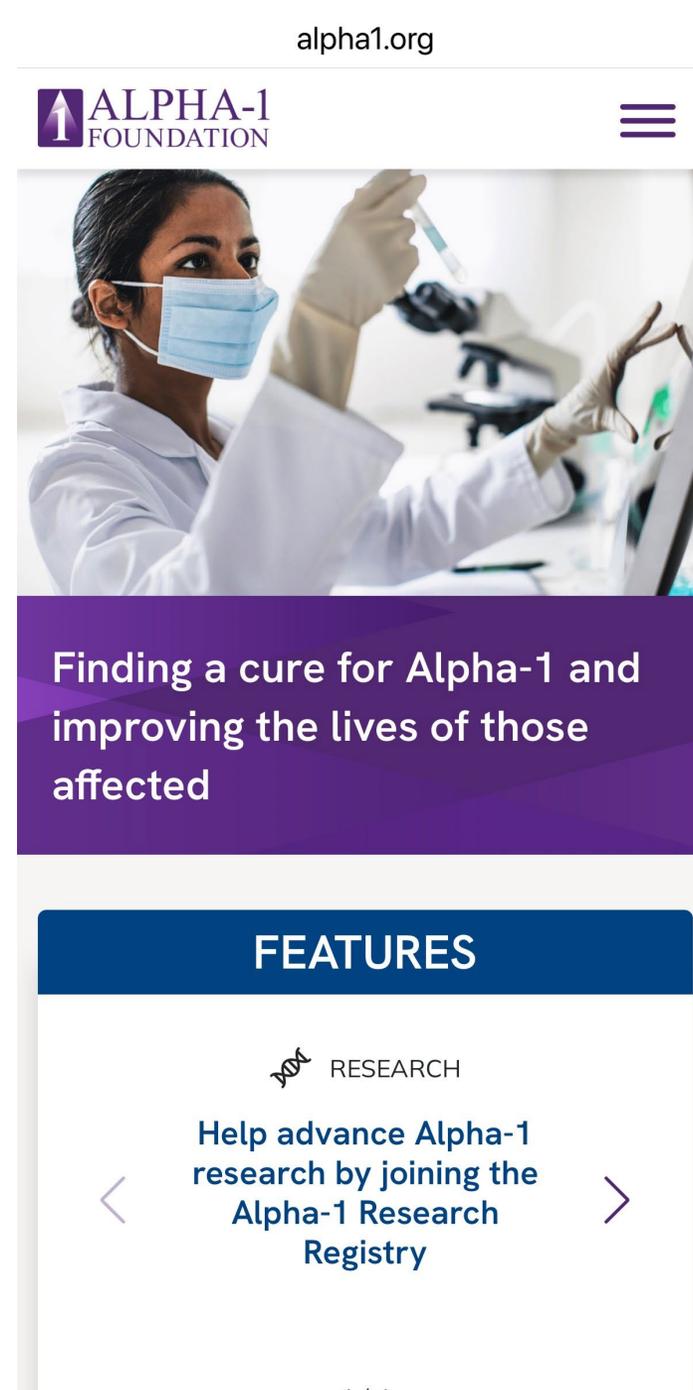## Steatotic Liver Disease Sub-classification

Current Treatment Options

- **Liver:** Nothing approved. Supportive care and/or Liver Transplant.

- **Lung:** IV weekly protein replacement that does not return patients to wild type and does not help the liver.

- www.alpha1.org

- Research registry

- Support groups

- Information

Key takeaways

- **Clinical presentation of AATD**
  - AAT levels and disease manifestations are correlated across genotypes
  - Z-AAT has a role as a bad actor in both liver manifestations and in the lungs (polymer in circulation can result in lung inflammation/injury)

- **Underdiagnosed, but increasing disease understanding and awareness**
  - Natural history studies are providing important longitudinal information on both the lung and liver manifestations associated with AATD across individuals' lifespan
  - Initiatives to improve awareness and diagnosis (Alpha-1F, AlphaDetect, etc.)

- **Unmet need based on current management of AATD**
  - No single therapy that can treat both lung and liver disease manifestations
  - High interest and patient receptivity to one-time novel therapies, such as DNA editing

Next Steps to Enable Pivotal Development of BEAM-302

Giuseppe Ciaramella, Ph.D., President

Beam intends to pursue accelerated approval pathway for BEAM-302 based on FDA feedback to date

Q1 2025

- ✓ Clinical proof of concept
- ✓ U.S. IND clearance

Q2 2025

- ✓ RMAT Designation
- ✓ Orphan Drug Designation

Q4 2025   FDA alignment

- Alignment reached with FDA on a potential accelerated approval pathway for BEAM-302 in AATD

- Primary endpoint expected to be based on AAT biomarkers evaluated over 12 months

- Anticipate enrolling approximately 50 additional patients in expansion of open-label Phase 1/2 trial

- Ongoing dialogue with FDA on confirmatory trial design

- Accepted to FDA CMC Development and Readiness Pilot (CDRP) program

IND = investigational new drug; RMAT Designation = Regenerative Medicine Advanced Therapy Designation

Enrollment in 60 mg Part A and Part B cohorts to continue while operationalizing pivotal cohort for planned initiation in 2H 2026

PART A:
AATD-associated Lung Disease

DOSE ESCALATION & EXPANSION

- Enrolling additional patients in 60 mg expansion
- Dosing started at U.S. sites

PART B:
AATD-associated Liver Disease with or without Lung Disease

DOSE ESCALATION

- Continuing to enroll 60 mg cohort

PART C: PIVOTAL COHORT
AATD-associated Lung Disease with or without Liver Disease

- Plan to initiate pivotal cohort in second half of 2026
- Protocol amendment in process
- Plan to utilize existing extensive global site network: 12+ sites in 6 countries
- U.S. sites now activated

Beam's long-term commitment to leading innovation for the AATD community

- Internal Beam lifecycle management through ongoing R&D efforts

- Member of C-Path's Critical Path for AATD (CPA-1) consortium in collaboration with FDA to accelerate AATD research by identifying clinical efficacy endpoints

- Collaboration with Alpha-1 Foundation and Alpha-1 Europe Alliance to educate about gene editing and obtain critical input on clinical trial design and patient experience

Beam is well positioned to realize the power of predictability in 2026 through key anticipated milestones

Pursue Path to Approval for Lead Programs

- Initiate pivotal cohort for BEAM-302 in 2H 2026
- Present detailed and updated BEAM-302 data at medical congress in 2026
- Plan to submit risto-cel BLA as early as YE 2026

Advance and Expand Pipeline

- File IND for BEAM-304 in 2026
- Report initial BEAM-301 data by YE 2026
- Complete BEAM-103 healthy volunteer study in 1H 2026
- Advance *in vivo* HSC editing program

Maintain Financial Strength

- $1.25 billion in cash as of Dec. 31, 2025*
- Expected runway into mid-2029 through anticipated risto-cel launch, execution of BEAM-302 pivotal development plan and clinical proof of concept for BEAM-304**
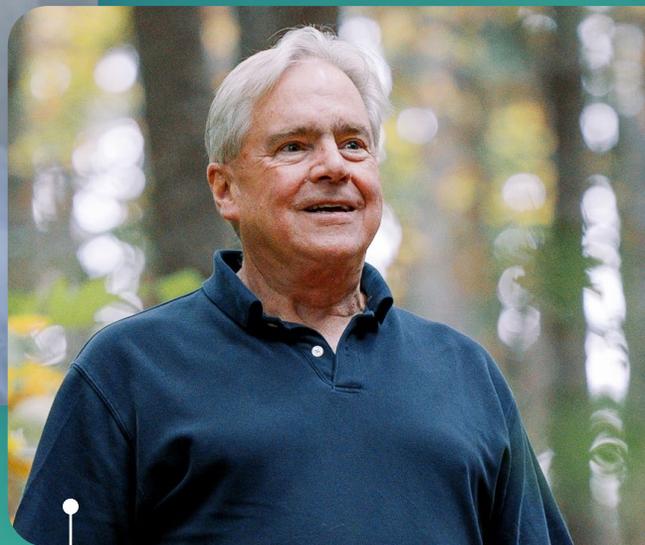
*Inclusive of cash, cash equivalents, and marketable securities.
**Inclusive of expected $200 million minimum draw from Sixth Street facility.

THANK YOU

Kyle
LIVING WITH
SICKLE CELL DISEASE

Dan
LIVING WITH
ALPHA-1 ANTITRYPSIN DEFICIENCY

Alyssa and Gayle
LIVING WITH
GLYCOGEN STORAGE DISEASE TYPE IA