WVE-006: RNA Editing for AATD

Interim RestorAATion-2 Clinical Data

ATS International Conference 2026 | Investor Webcast

May 18, 2026

Forward-looking statements

This document contains forward-looking statements. All statements other than statements of historical facts contained in this document, including statements regarding possible or assumed future results of operations, preclinical and clinical studies, business strategies, research and development plans, collaborations and partnerships, regulatory activities and timing thereof, competitive position, potential growth opportunities, use of proceeds and the effects of competition are forward-looking statements. These statements involve known and unknown risks, uncertainties and other important factors that may cause the actual results, performance or achievements of Wave Life Sciences Ltd. (the "Company") to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. In some cases, you can identify forward-looking statements by terms such as "may," "will," "should," "expect," "plan," "aim," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions. The forward-looking statements in this presentation are only predictions. The Company has based these forward-looking statements largely on its current expectations and projections about future events and financial trends that it believes may affect the Company's business, financial condition and results of operations. These forward-looking statements speak only as of the date of this presentation and are subject to a number of risks, uncertainties and assumptions, including those listed under Risk Factors in the Company's Form 10-K and other filings with the SEC, some of which cannot be predicted or quantified and some of which are beyond the Company's control. The events and circumstances reflected in the Company's forward-looking statements may not be achieved or occur, and actual results could differ materially from those projected in the forward-looking statements. Moreover, the Company operates in a dynamic industry and economy. New risk factors and uncertainties may emerge from time to time, and it is not possible for management to predict all risk factors and uncertainties that the Company may face. Except as required by applicable law, the Company does not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

Today's agenda

Opening remarks
Paul Bolno, MD, MBA
President and CEO

Clinician perspective on AATD and treatment gaps
D. Kyle Hogarth, MD, FCCP
Professor of Medicine at The University of Chicago

RestorAATion-2 clinical data
Chris Wright MD, PhD
Chief Medical Officer

Closing remarks
Paul Bolno, MD, MBA
President and CEO

Q&A

Opening remarks

Paul Bolno, MD, MBA

President & CEO

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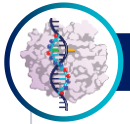
WVE-006, RNA editing for AATD: Produces wild-type M-AAT protein, reduces Z-AAT protein, restores dynamic acute phase response



RNA editing for AATD

- **Significant unmet need remains in AATD**
  - Approximately 200,000 individuals living with Pi*ZZ AATD in US and Europe
  - Weekly IV augmentation therapy is the only treatment option for AATD lung disease
  - No approved therapies for AATD liver disease
- **Wave's RNA editing offers a differentiated approach**
  - RNA editing aims to address the root cause of both lung and liver manifestations of AATD and restore dynamic production of AAT with a safe and convenient therapy
  - No risk of permanent bystander edits, indels, off-target edits (including cancer-associated genes) which have been connected to DNA editing[1]
- **WVE-006**
  - WVE-006 is a GalNAc-conjugated, subcutaneously delivered, RNA editing oligonucleotide (AIMer) for AATD that utilizes Wave's proprietary best-in-class chemistry

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1. Packer, et al. (2024) Methods and Compositions for Treating Alpha-1 Antitrypsin Deficiency (International Patent Application PCT/US2024/034189) https://patentimages.storage.googleapis.com/f7/f0/9a/f88f452755cb16/WO2024259364A2.pdf

Treatment with WVE-006 achieves Pi*MZ profile across doses: delivering potential liver and lung benefits with a safe and convenient therapy

		Protecting the liver	Protecting the lung	
WVE-006	**Total AAT**	**Reduce Z-AAT**	**MZ-like dynamic AAT production**	**Generate healthy, wild-type M-AAT**
200 mg biweekly	**11.9** µM	**71%** reduction	**Three** acute phase responses CRP increases correlated with AAT increases	**64%** M-AAT of total AAT
400 mg monthly	**13.6** µM	**68%** reduction		**59%** M-AAT of total AAT

Safe and convenient

- Generally safe and well tolerated
- Subcutaneous delivery, with potential for self-administration
- Potential for monthly dosing

Regulatory feedback on accelerated approval pathway for WVE-006 expected mid-2026

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Circulating M-AAT, Z-AAT, and total (M + Z) AAT protein in the serum were measured by highly selective and sensitive LC-MS/MS assays (LLOQ: 0.096 µM (M), 0.029 µM (Z)) and reported as mean participant MAD maximums. In a natural history cohort of individuals with M/Z genotype using the same LC-MS/MS assay in RestorAATion-2, M-AAT% of total AAT mean was 64% (range 57%-71%).

Guest speaker: D. Kyle Hogarth, MD, FCCP

Professor of Medicine and Director of the Alpha-1 Clinical Resource Center University of Chicago

- D. Kyle Hogarth, MD, FCCP, is a Professor of Medicine in the Section of Pulmonary and Critical Care Medicine at The University of Chicago. He is the Director of Bronchoscopy and is heavily involved in the field of advanced bronchoscopy and interventional pulmonary with numerous publications. He also runs the Alpha One Antitrypsin Deficiency Clinical Resource Center, one of the largest in the Midwest with over 250 patients.
- Dr. Hogarth received his medical degree at Case Western Reserve University School of Medicine in Cleveland, Ohio. He then completed a residency in Internal Medicine and a fellowship in Pulmonary and Critical Care at The University of Chicago.
- He has been published in the New England Journal of Medicine, Chest, ERJ and many other journals. He previously served on the editorial board of CHEST, where he was the section editor for the podcast section and giants in chest medicine series. He helped write the 2016 Alpha One Antitrypsin deficiency Clinical Practice Guidelines. He has won numerous clinical awards and teaching awards from his University. He is a founding member and a Past President of the Society for Advanced Bronchoscopy.

Professor of Medicine, Section of Pulmonary and Critical Care Medicine
University of Chicago
Director of Bronchoscopy
Director of the Alpha One Antitrypsin Deficiency Clinical Resource Center
Director of the Pulmonary Rehab

Clinician perspective on AATD and treatment gaps

D. Kyle Hogarth, MD, FCCP

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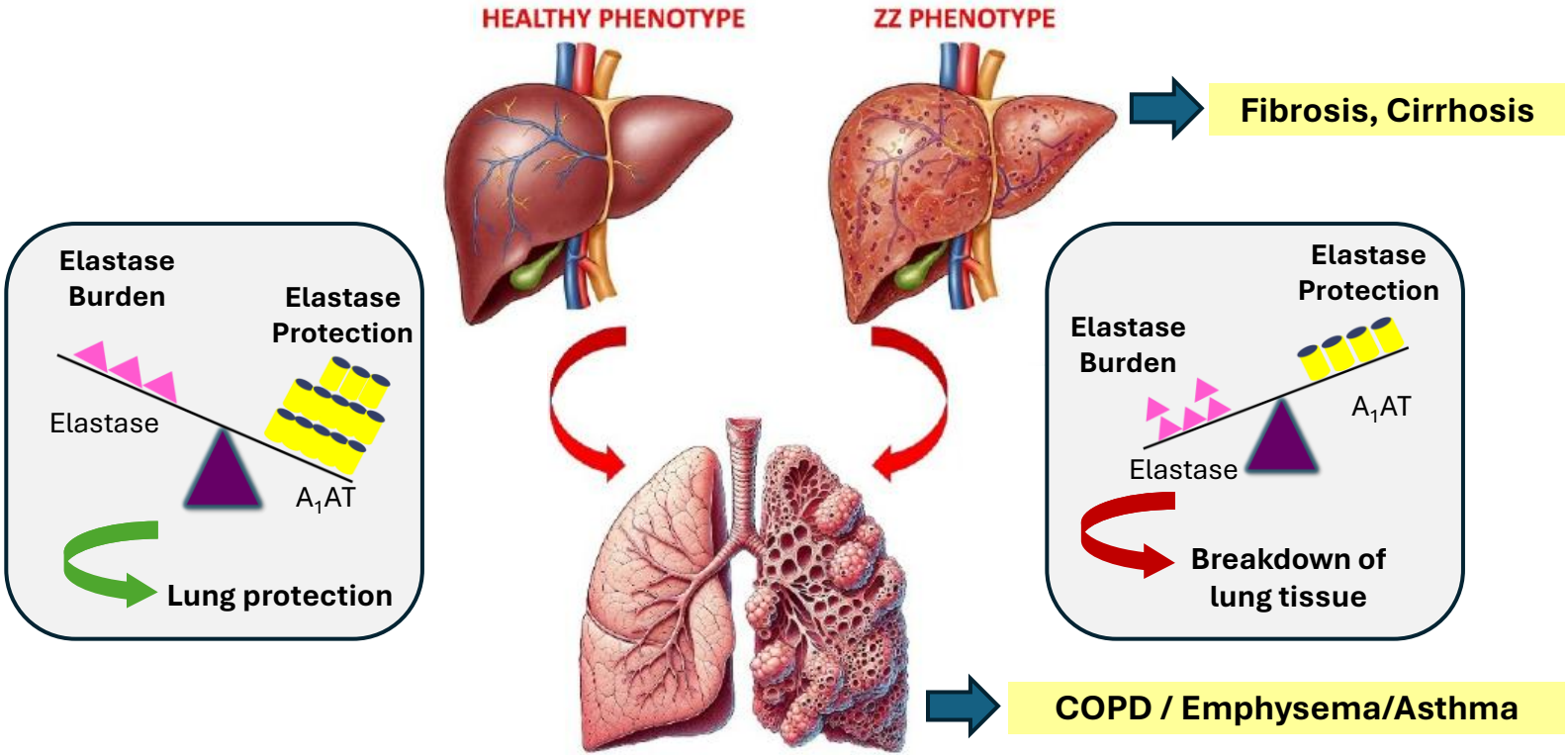
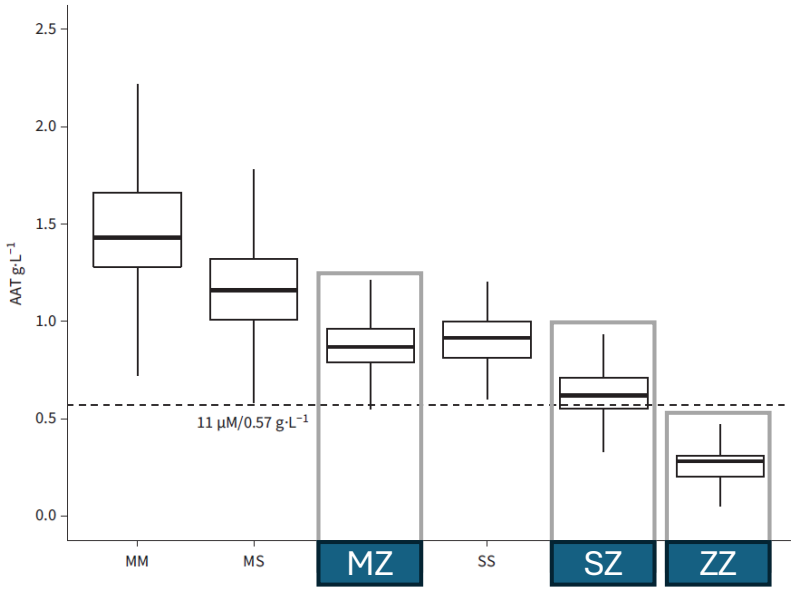
Alpha-1 antitrypsin deficiency (AATD) results from the SERPINA1 Z mutation and can lead to both liver and lung disease



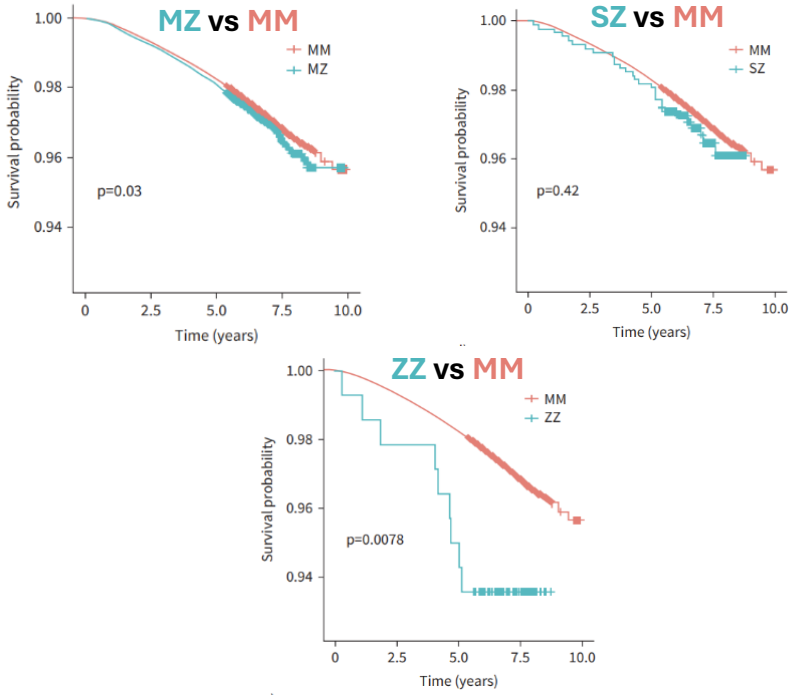
Adapted from Grignano, *et al. Int. J. Mol. Sci.* 2025, 26(11):5085

AATD genotypes have varying levels of AAT and disease risk, with Pi*ZZ at significantly greater risk for poorer outcomes

Common AATD genotypes and associated AAT levels

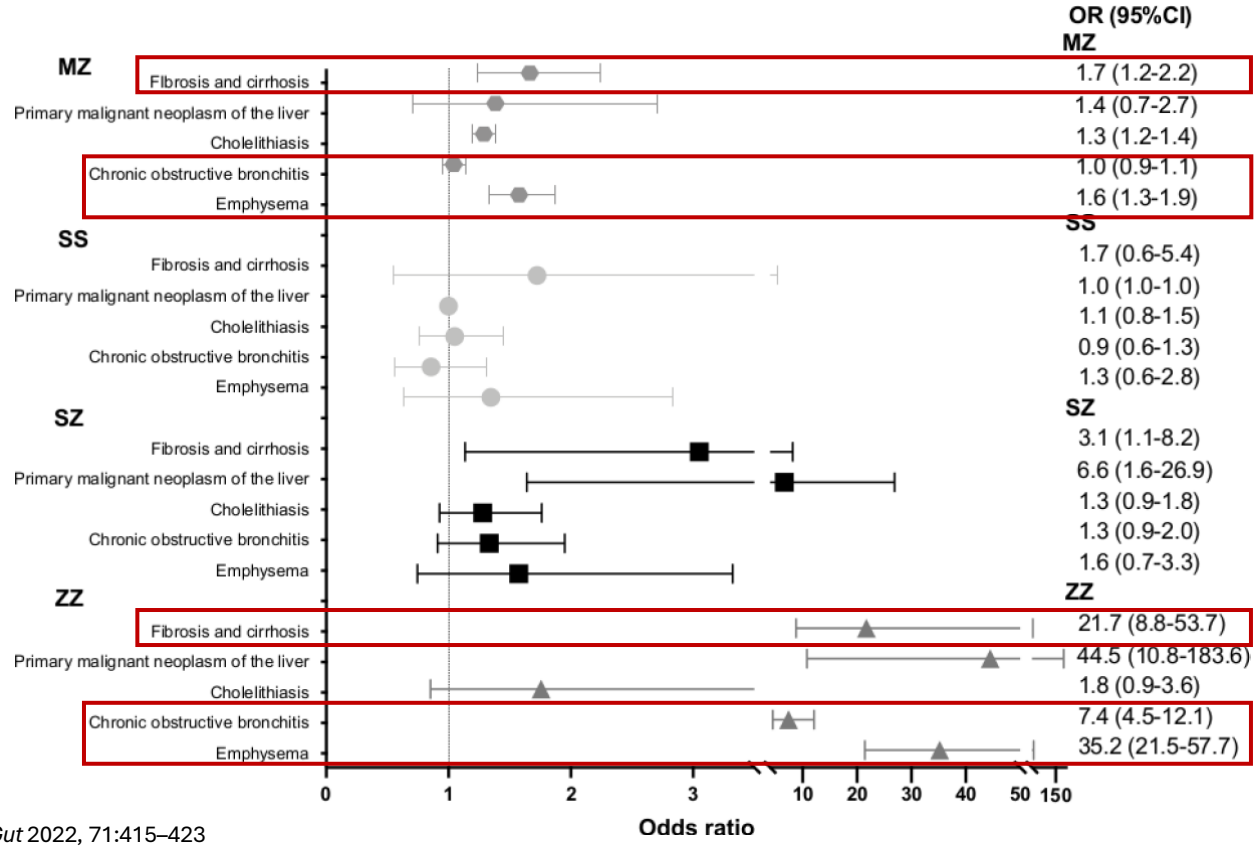


Survival curves of all-cause mortality by SERPINA1 genotype in UK Biobank



Franciosi, *et al*. *Eur Respir J* 2022, 59: 2101410

Individuals with Pi*ZZ genotype have significantly higher risk of lung and liver disease vs. those with Pi*MZ genotype



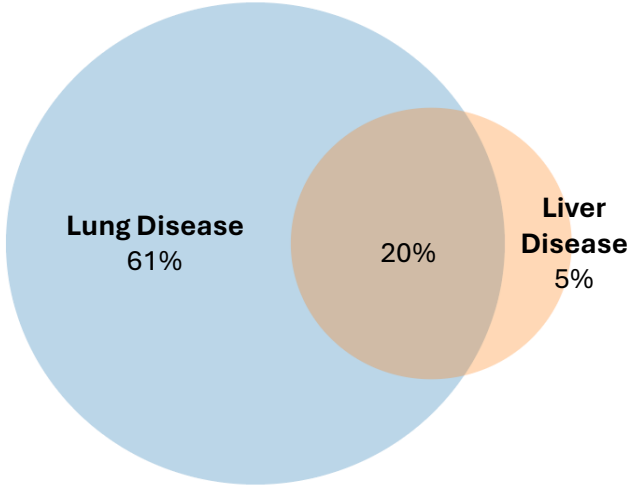
Fromme M, *et al. Gut* 2022, 71:415–423

All Pi*ZZ individuals are at risk for liver and lung disease, with diagnosis rates expected to increase with greater disease awareness and screening

AATD is highly underdiagnosed today

- ~200K Pi*ZZ individuals in US and Europe,[1] but only ~10% are diagnosed
- Average age of diagnosis of AATD lung disease is **46 years**[2]
- Average age of adult-onset liver disease is **61 years**[3]

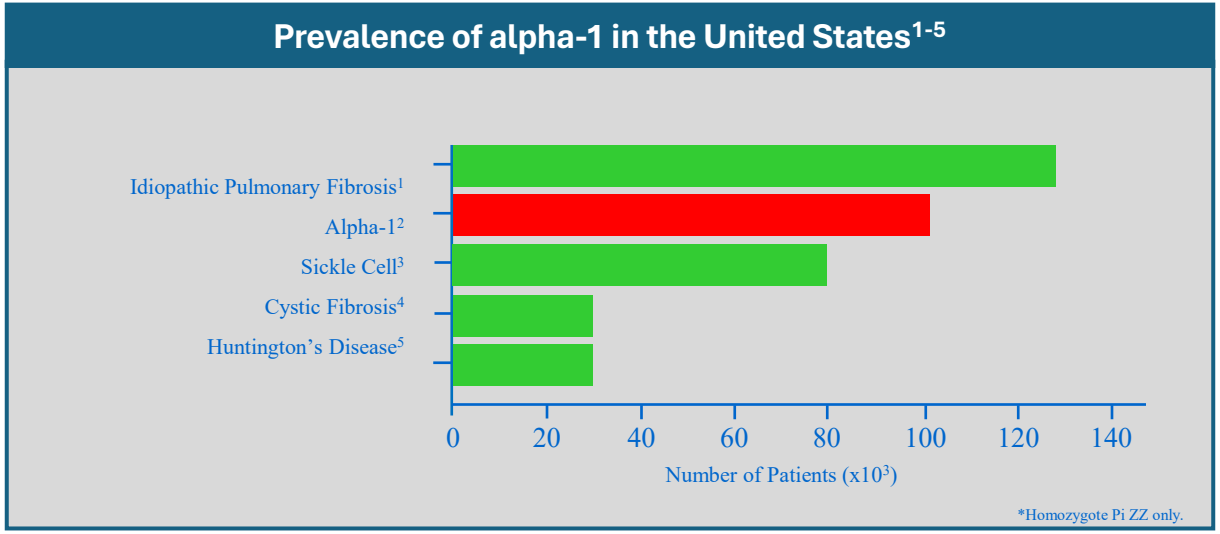
Frequency of Lung or Liver Disease of Pi*ZZ Patients at Diagnosis*



**From Alpha One International Registry (n=3,405)*[4]
(13% reported no disease at diagnosis)

1. Blanco, *et al*., *Int J Chron Obstruct Pulmon Dis* 2017, 12:561-69; 2. Strnad, *et al*. *N Engl J Med* 2020, 382:1443-55; 3. Tanash and Piitulainen *J Gastroenterol* 2019, 54:541-548; 4. McElvaney, *et al.*, *Eur Respir J* 2020, 55:1902410

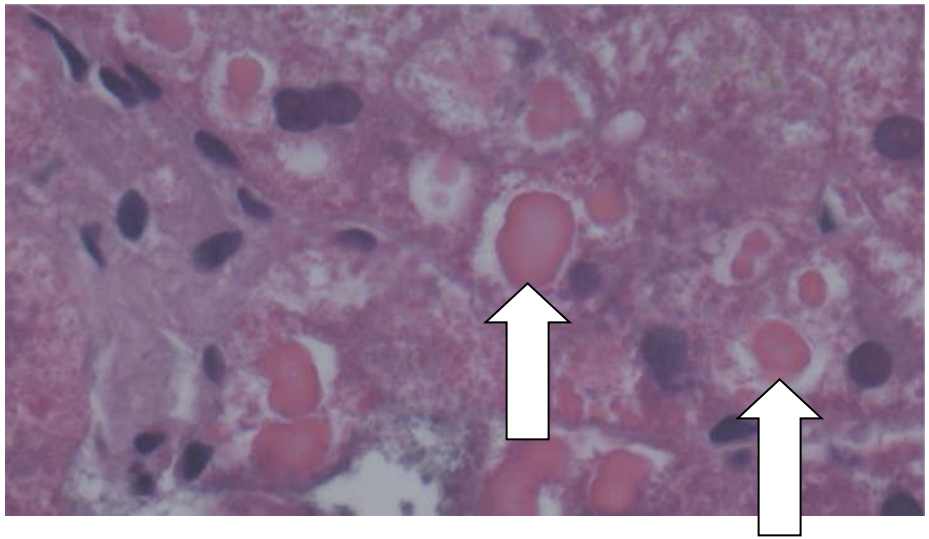
Genetic COPD: More common than previously thought



- Not a rare disorder, JUST RARELY TESTED FOR.[6]
  - Up to 90% of those with alpha-1 are undiagnosed[2]
  - In a registry study of more than 1000 patients, the average interval between onset of symptoms and diagnosis was 8.3 years[2]

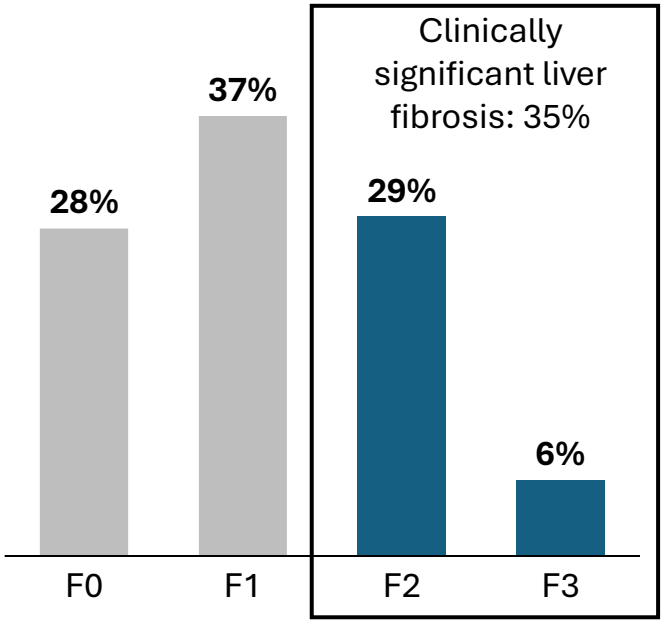
1. http://www.coalitionforpf.org/cpf_faq.php. Accessed October 4, 2011. 2. Campos MA, et al. *Chest*. 2005;128(3):1179-1186.
3. http://sicklecelldisorder.com/index.php/the-disease.html. Accessed October 4, 2011. 4. http://www.cff.org/AboutCF/Faqs/#What_is_cystic_fibrosis? Accessed October 4, 2011.
5. http://www.hdsa.org/about/our-mission/what-is-hd.html. Accessed October 4, 2011. 6. de Serres FJ. *Environ Health Perspect*. 2003;111(16):1851-1854.

Liver fibrosis in AATD is common and often asymptomatic in early stages



Build up of alpha1 Z within liver cells

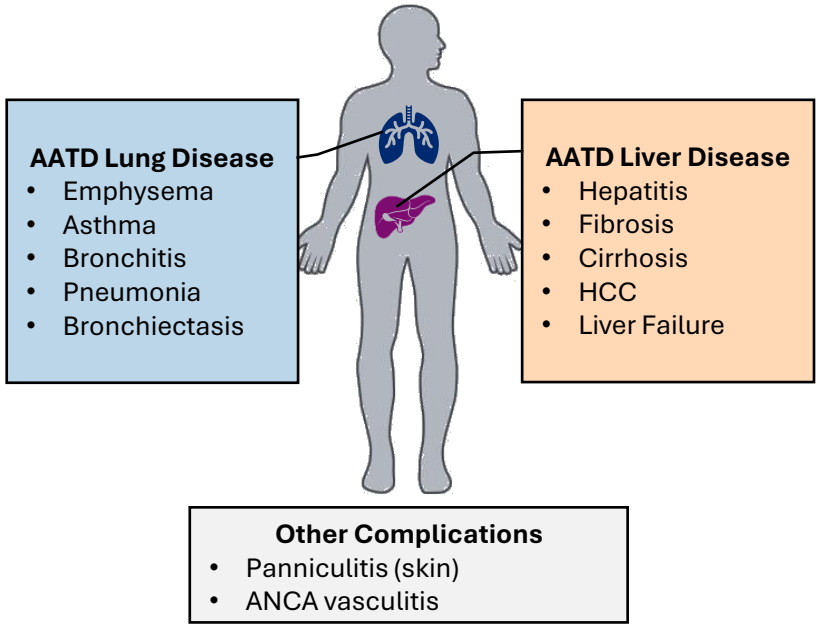
Fibrosis as Assessed by Liver Biopsy in Pi*ZZ AATD Cohort (n=94 adults)



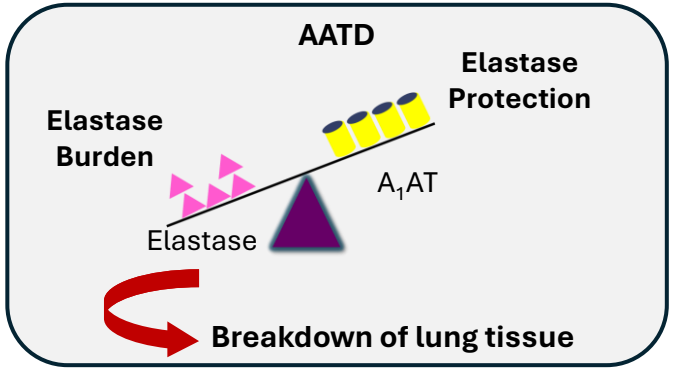
Clark, *et al. J Hepatology* 2018, 69:1357

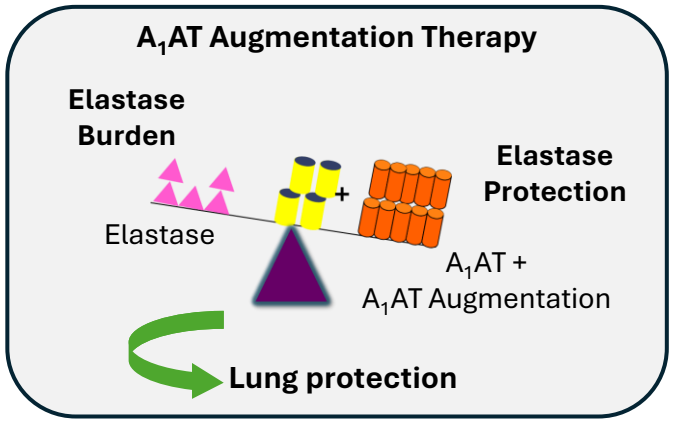
AATD significantly impacts patient quality of life and has limited treatment options

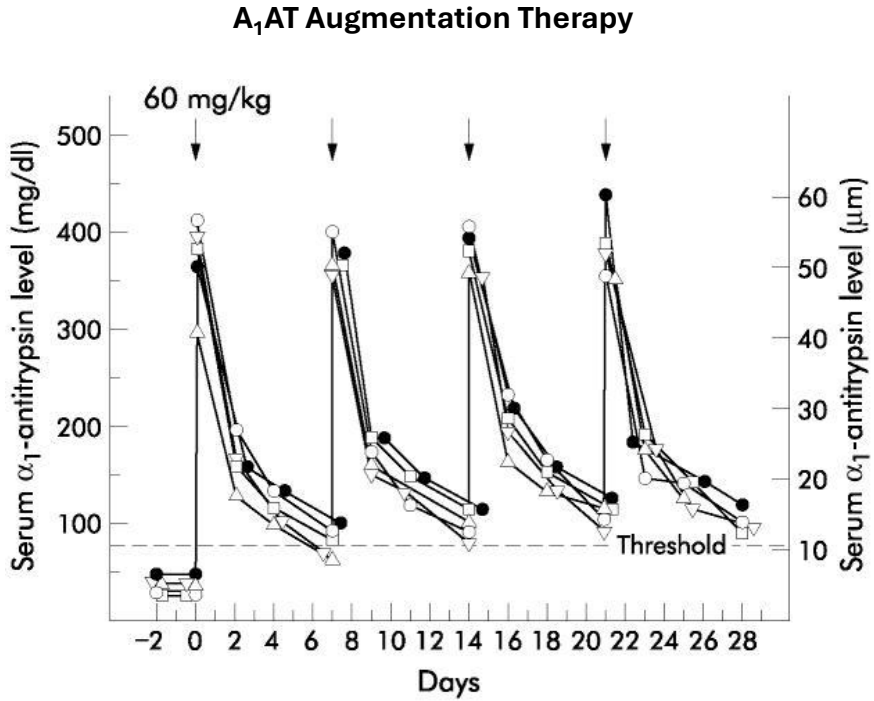
- **Augmentation therapy** is only treatment option for AATD lung disease and requires weekly IV infusions
- **No treatment for AATD liver disease**, other than liver transplant



Augmentation therapy increases AAT levels with weekly IV infusions but does not address AATD liver disease



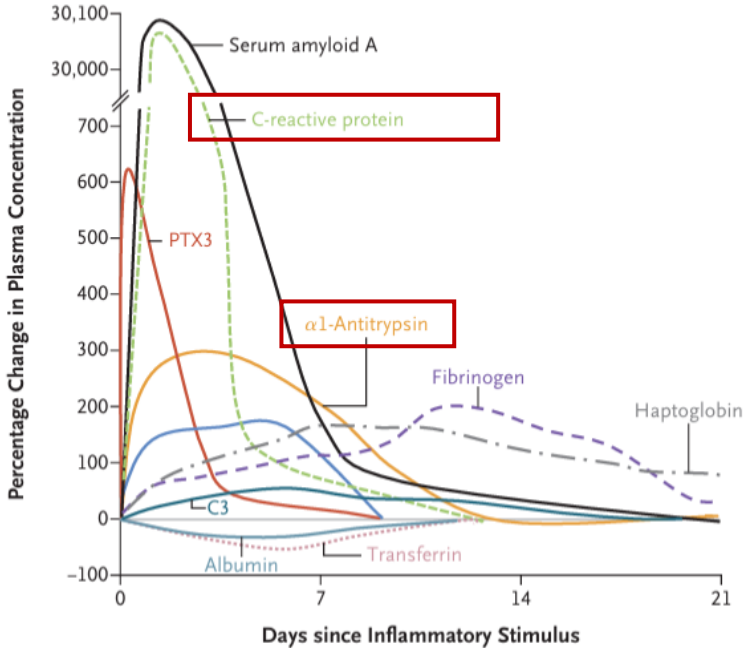




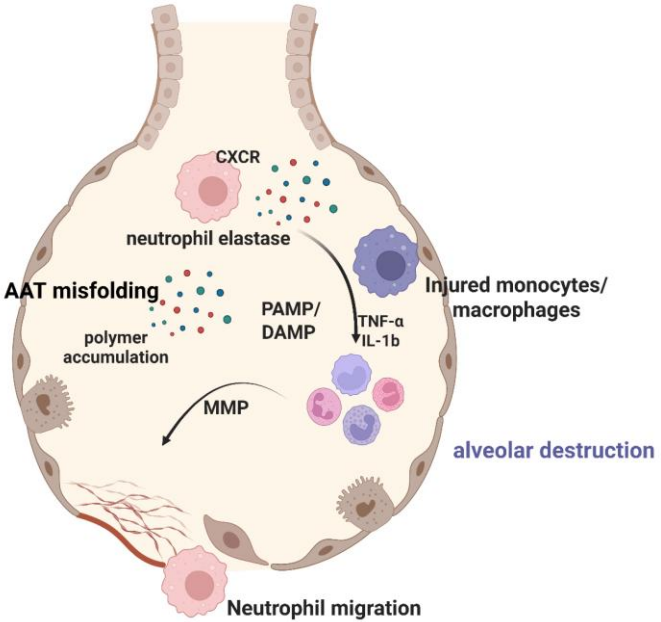
Thorax 2004;59 708–712
Wewers in *NEJM* 1987;316:1055

Alpha-1 antitrypsin is a key player in the acute phase response

In healthy individuals, AAT levels increase rapidly following an inflammatory stimulus

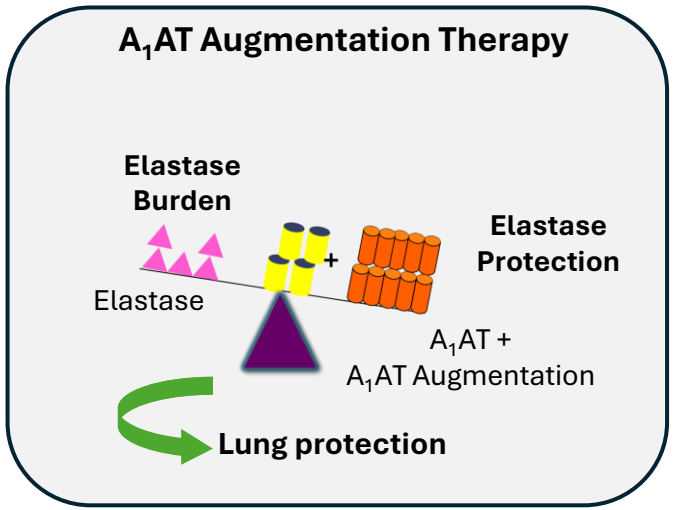


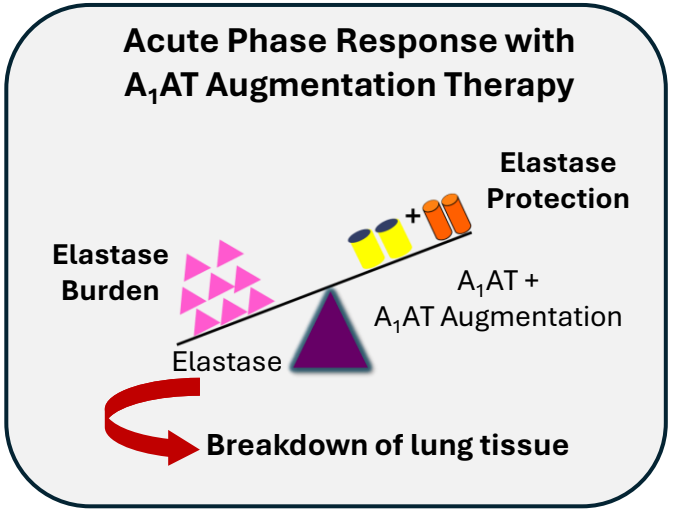
Pathophysiology of AATD-Associated Alveolar Injury

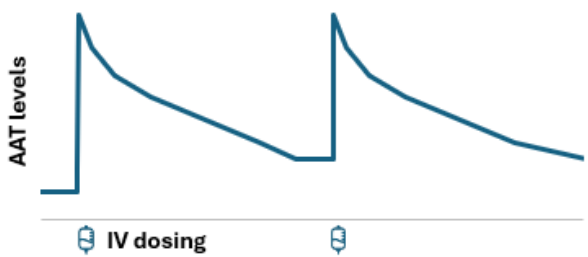


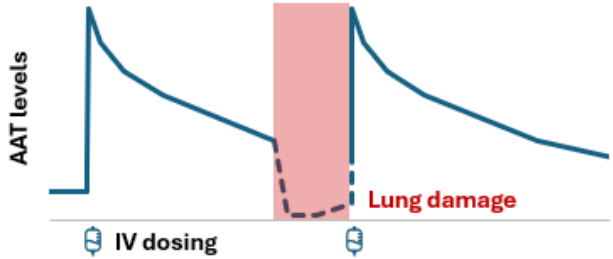
Mantovani and Garlanda. *N Engl J Med* 2023, 388:439-452; Yang and Kim. *Int. J. Mol. Sci.* 2025, 26:8504

During acute phase responses, A1AT is consumed and can leave lungs at risk for proteolytic damage, even with augmentation therapy









Select emerging treatment options

Lung Only

Recombinant Augmentation Therapy

Liver Only

AAT siRNA

Lung and Liver Disease

RNA Editing

DNA Base Editing

WVE-006: RNA editing for AATD

Chris Wright, MD, PhD
Chief Medical Officer

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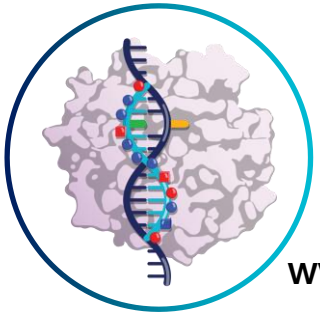
WVE-006 corrects the SERPINA1 transcript to convert the Z mutation to wild-type, restore M-AAT production

WVE-006 (RNA editing)

G to **A** mutation

Z-AAT Misfolded Protein



WVE-006

A to **I(G)** RNA edit

Wild-type M-AAT Protein

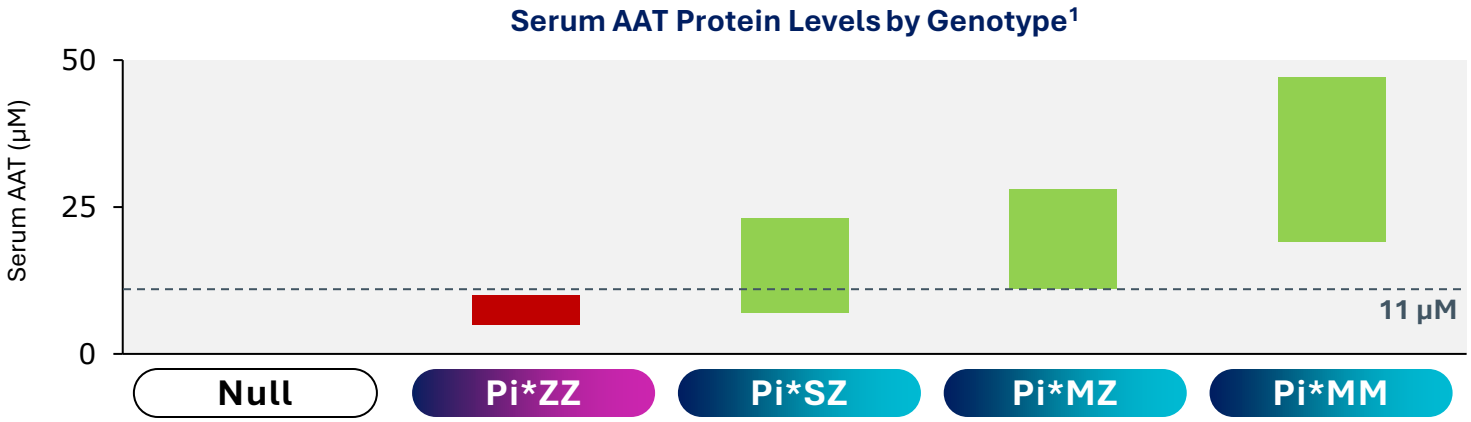
- Proprietary chemistry
- Highly specific (no bystander edits)
- Subcutaneous delivery (GalNAc)
- Infrequent dosing

Reduce Z-AAT protein aggregation in liver

Restore circulating M-AAT and physiological AAT protein production

Strnad et al., 2020 *N Engl J Med* 382:1443-55; Stoller et al., 1993 Alpha-1 Antitrypsin Deficiency GeneReviews. M-AAT: Wild-type alpha-1 antitrypsin protein Z-AAT: mutant alpha-1 antitrypsin protein

Treatment target: Heterozygous MZ genotype with M-AAT >50% AAT and a dynamic CRP response has low risk of liver and lung disease



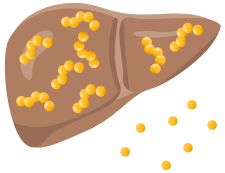
Serum AAT composition	No AAT	100% Z-AAT	S-AAT and Z-AAT	Mean 64% M-AAT (range: 57%-71%)[2]	100% M-AAT
Risk of lung disease[3]	Very high	High	Low	Low	Normal
Risk of liver disease[3]	Normal	High	Low	Low	Normal
AAT levels increase with acute phase response[4]	N/A	No	Yes	Yes	Yes

1. Range of observed serum AAT levels from Bornhorst, et al. 2013 CHEST 143:1000-1008; 2. Percent M-AAT of total AAT from a natural history cohort of individuals with M/Z genotype using the same LC-MS/MS assay in RestorAATion-2; 3. Brode, et al. 2012 CMAJ 184:1365-1371; 4. Sanders, et al. 2018 COPD 15:10-16

RNA editing expected to deliver even greater impact over time with continued treatment and improved liver health

Pi*ZZ

Liver disease

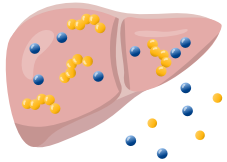


High Z-AAT aggregation and liver inflammation

WVE-006

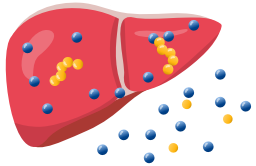
Pi*MZ-like

Reduce Z-AAT to prevent liver damage



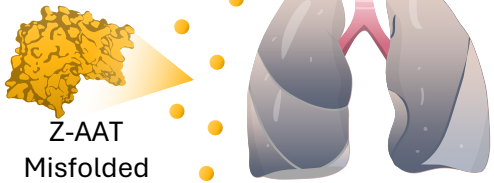
~50% M-AAT produced in liver

Z-aggregate clearance over time



Increasing hepatocyte health

Lung disease

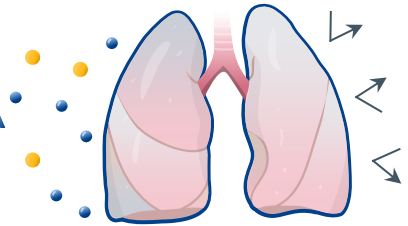


Z-AAT Misfolded Protein

100% Z-AAT, 0% M-AAT

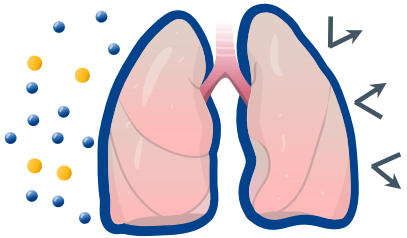
Wild-type M-AAT Protein

Restore M-AAT to prevent lung damage



>50% M-AAT

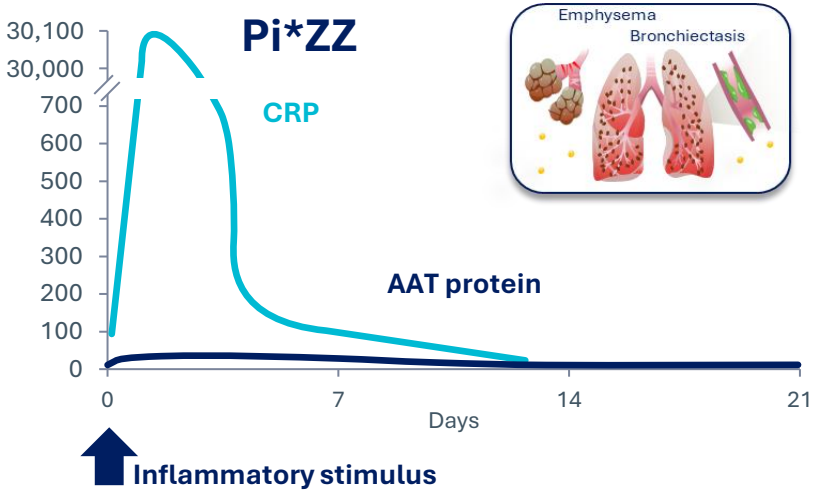
AAT levels responsive to CRP



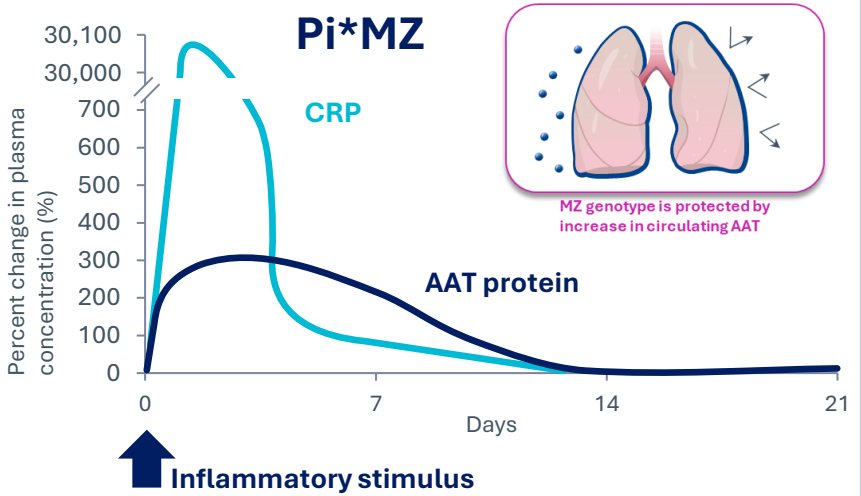
Potential for higher levels of serum AAT

Pi*ZZ individuals have an impaired serum AAT increase during acute-phase responses (APRs), when they need it most

Lung damage occurs during exacerbations, when more AAT protein is needed for protection



AAT protein has protective functions and is produced during acute phase response



RNA editing has potential to restore dynamic AAT response to inflammation

Left: Mantovani A, Garlanda C. *N Engl J Med*, 2023;388:439-452; Right: Sanders et al., *J COPD*, 2018

WVE-006 restores dynamic production of AAT protein as part of APRs to protect lungs from damage

APRs require a rapid rise in AAT levels to prevent lung damage

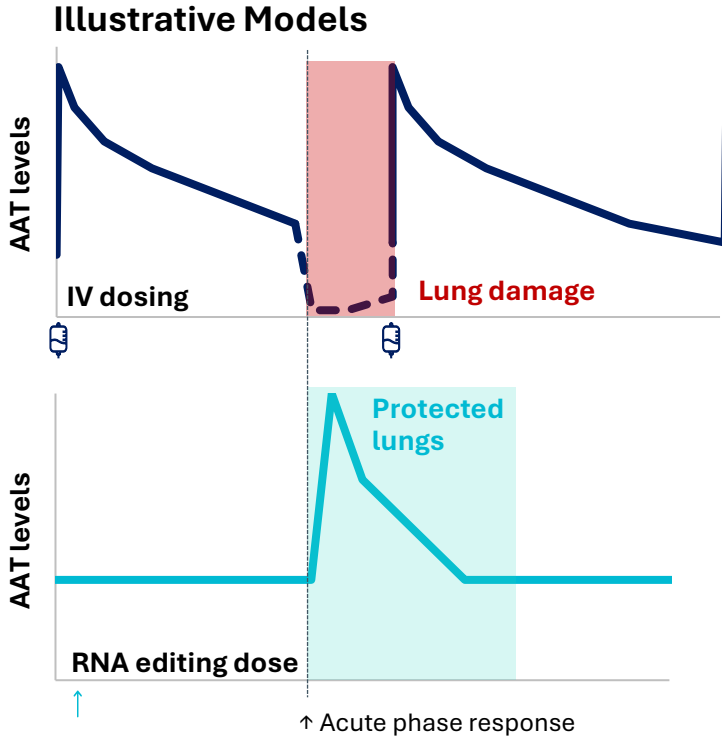
- Even with augmentation therapy, in ZZ individuals, exacerbations occur on average 1–2x yearly
- These exacerbations could deplete AAT levels within hours[1]
- In healthy (including MZ) individuals, AAT levels rise quickly (24–72 hours) after stimulus

Standard of care (augmentation therapy) may leave patients at risk

- Exogenous AAT levels risk **depletion** before next scheduled IV dose

WVE-006 treatment response is dynamic

- Endogenous AAT levels increase during acute phase response



Right: Illustrative model of impact of acute phase response

1. Liou & Campbell, *Journal of Immunology* 1996; Thulborn et al., *Respiratory Research* 2019; Beatty et al., *Journal of Biological Chemistry* 1980.

RestorAATion-2 clinical update

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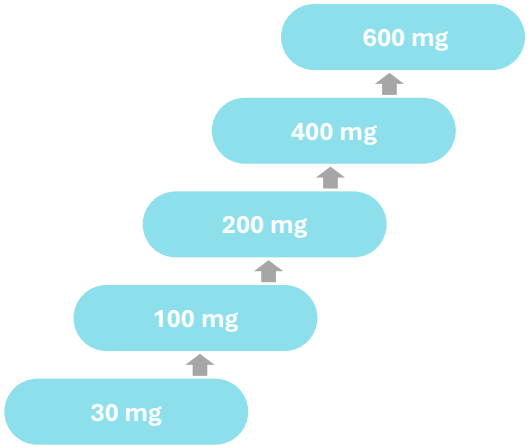
RestorAATion-2: 200 mg biweekly and 400 mg monthly cohorts complete; 600 mg monthly ongoing

RESTORAATION

RestorAATion-1: Healthy Volunteers → **RestorAATion-2: AATD Patients**

SAD → MAD Multi-dosing complete



SAD	MAD
600 mg Single dose	600 mg x 4 doses Monthly
400 mg Single dose	400 mg x 4 doses Monthly
200 mg Single dose	200 mg x 7 doses Biweekly

Study key objectives

Safety and tolerability	Pharmacokinetics	Serum M-AAT levels

HV: healthy volunteer; SAD: single-ascending dose; MAD: multi-ascending dose

Baseline characteristics were similar across the cohorts and included participants with liver disease

Key Inclusion Criteria

- Pi*ZZ genotype
- 18 to 70 years of age
- Healthy or mild to moderate AATD-induced lung disease and/or stable mild AATD-induced liver disease
- Non-smoker for at least 1 year prior to screening

RestorAATion-2 Baseline Participant Characteristics	200 mg N=8 n (%)	400 mg N=8 n (%)	600 mg N=8 n (%)
Age at consent (years) (mean (SD))	51.4 (12.0)	49.4 (18.1)	51.3 (15.5)
Gender, N (%) Male Female	 2 (25.0) 6 (75.0)	 4 (50.0) 4 (50.0)	 6 (75.0) 2 (25.0)
Weight (kg), Median (min, max)	79.6 (64.8, 100.0)	84.0 (65.6, 97.5)	78.5 (64.4, 96.5)
Years since AATD diagnosis (mean)	15.9	16.2	10.9
Baseline Day 1 FEV1 (mean (L) (min, max))	3.2 (2.2, 5.8)	2.9 (1.3, 5.1)	3.6 (2.4, 5.0)
Baseline Liver Elastography (N, F0-2 score; % of participants)	F0: 8 (100.0)	F0: 5 (62.5) F1: 2 (25.0) F2: 1 (12.5)	F0: 5 (62.5) F1: 2 (25.0) F2: 1 (12.5)

RestorAATion-2: WVE-006 continues to be generally safe and well tolerated through 600 mg

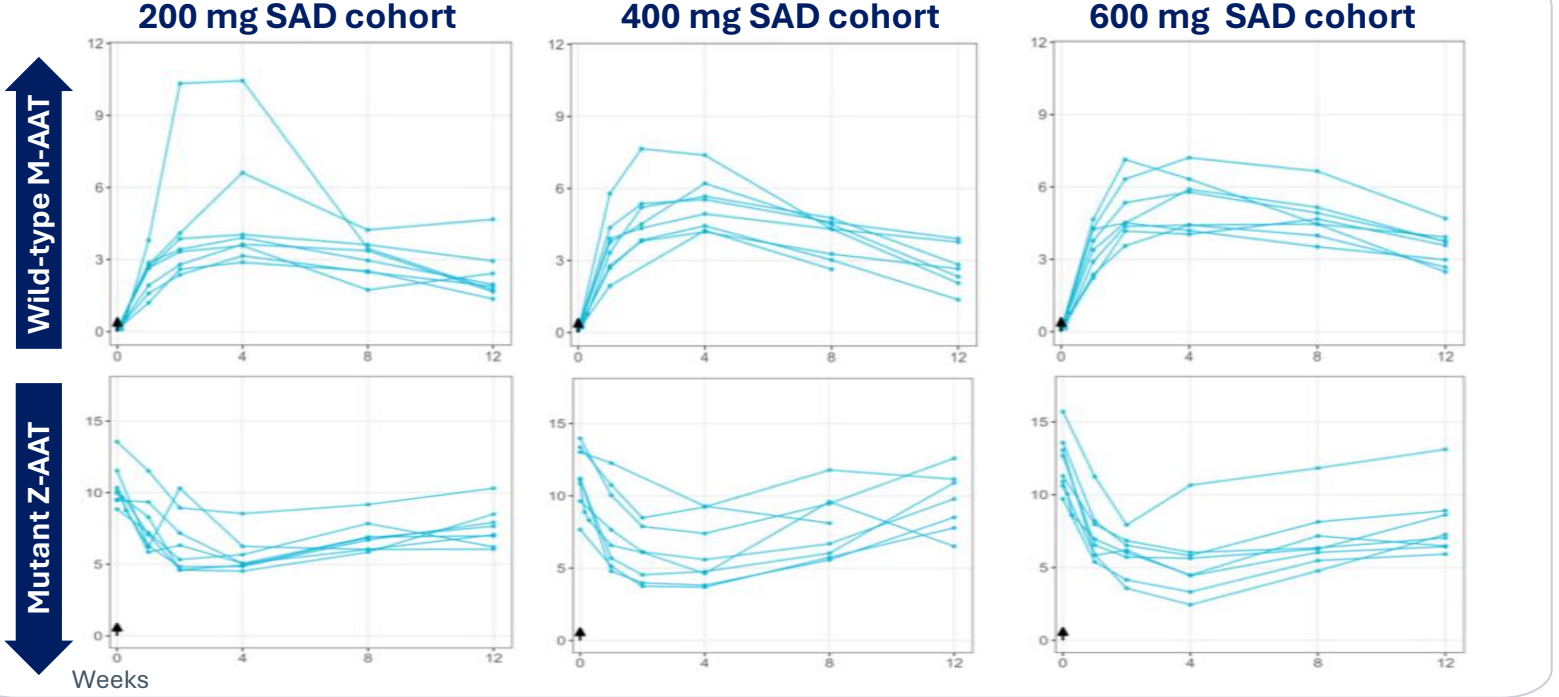
TEAE Category	200 mg SAD N=8 n (%)	200 mg MAD N=8 n (%)	400 mg SAD N=8 n (%)	400 mg MAD N=9 n(%)	600 mg SAD N=8 n(%)
Any TEAE	6 (75.0)	6 (75.0)	7 (87.5)	7 (77.8)	8 (100.0)
Mild	2 (25.0)	0	1 (12.5)	3 (33.3)	6 (75.0)
Moderate	4 (50.0)	6 (75.0)	6 (75.0)	4 (44.4)	2 (25.0)
Severe	0	0	0	0	0
Any drug-related TEAE	1 (12.5)	3 (37.5)	2 (25.0)	5 (55.6)	5 (62.5)
Mild	1 (12.5)	3 (37.5)	0	2 (22.2)	4 (50.0)
Moderate	0	0	2 (25.0)	3 (33.3)	1 (12.5)
Severe	0	0	0	0	0
Any serious TEAE	0	0	0	0	0
Any TEAE leading to discontinuation	0	0	1 (12.5)	0	0
Any TEAE leading to death	0	0	0	0	0

- No SAEs and all TEAEs were mild to moderate in severity
- No treatment-related, clinically relevant changes in labs (including liver function tests), ECG, or vital signs

Data cut as of April 3, 2026.

Rapid and consistent increases in M-AAT and reductions in Z-AAT following single dose of WVE-006

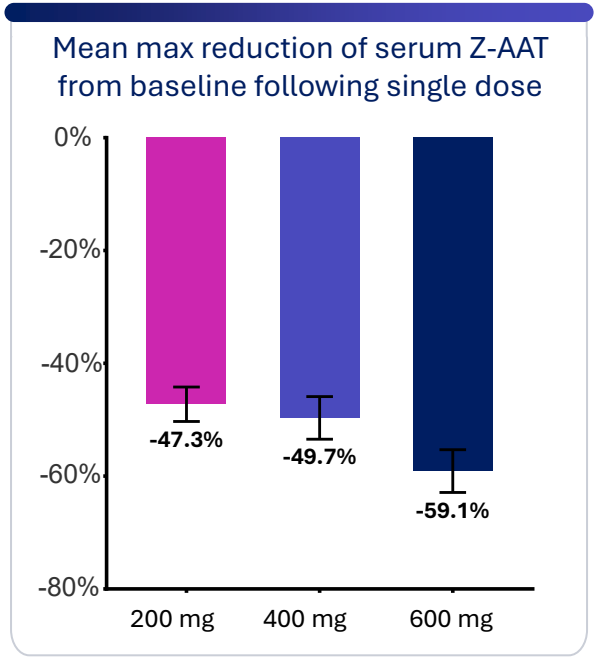
Increases in neutrophil elastase inhibition from baseline confirmed production of functional AAT



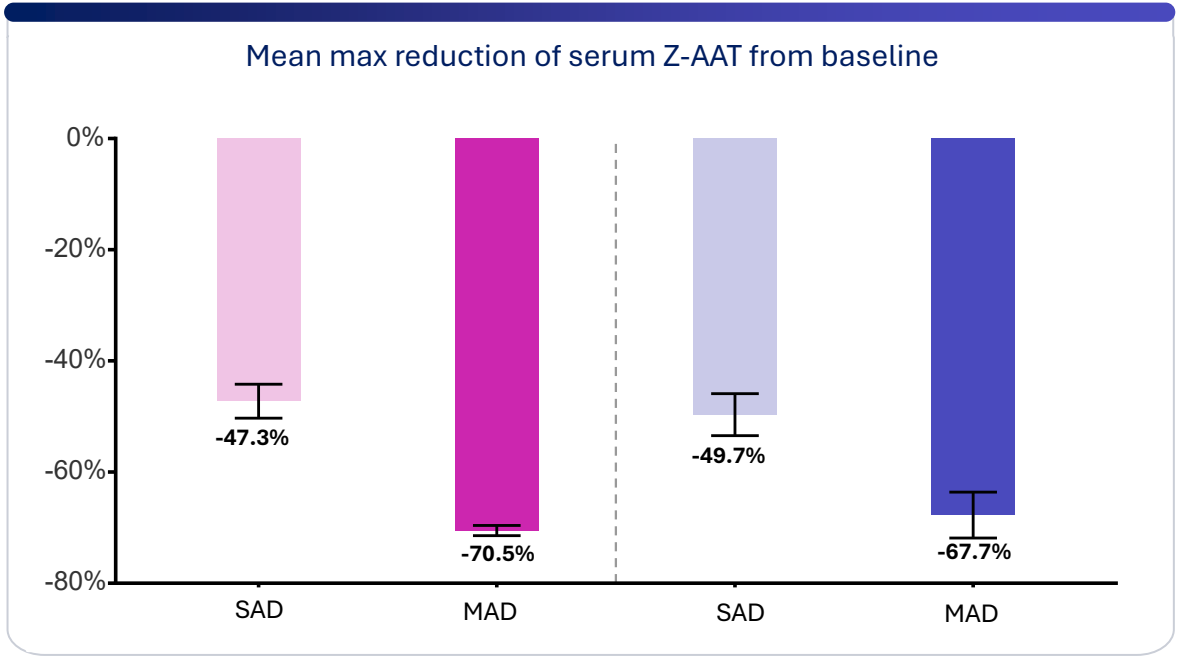
M and Z-AAT protein in the serum were measured by highly selective and sensitive LC-MS/MS assays. f-AAT was measured using a neutrophil elastase inhibition assay. Arrows indicate scheduled dose administration times of WVE-006. In a natural history cohort of individuals with Z/Z genotype using the same LC-MS/MS assay in RestorAATion-2, Z-AAT mean was 13.1μM (range 8.2-21.7μM).

Up to 71% reduction in circulating, mutant Z-AAT supporting potential improvement in liver health

SAD cohorts: Dose-dependent Z-AAT reductions



MAD cohorts: Similar reductions with extended dosing interval

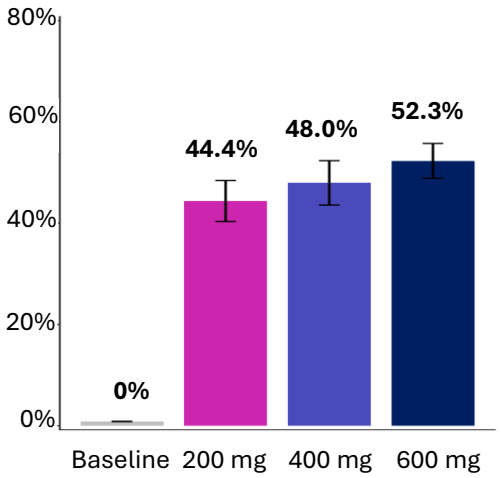


Error bars represent the standard error of the mean (SE).

WVE-006 generated circulating wild-type M-AAT within the MZ range (up to 64%) with no bystander edits, supporting potential improvement in lung health

SAD cohorts: Dose-dependent M-AAT restoration

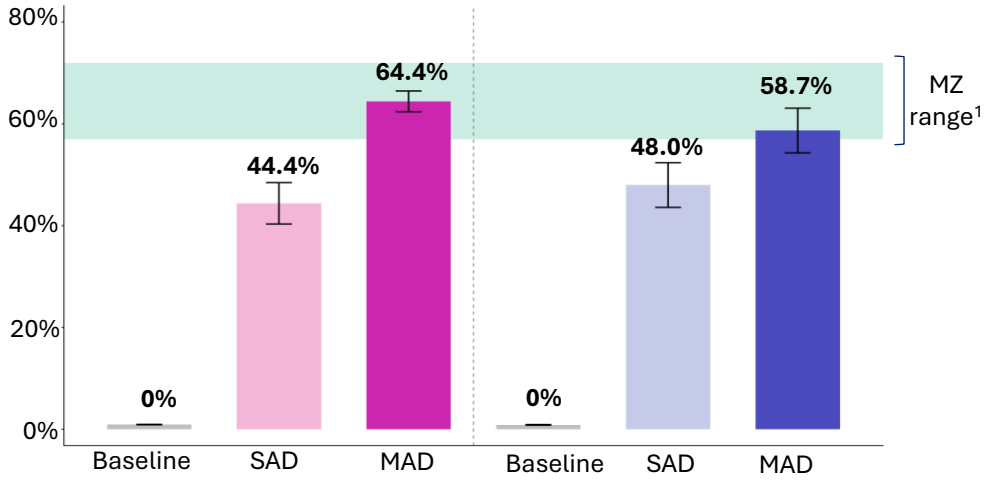
Mean max M-AAT (% of total AAT) following a single dose



MAD cohorts: Similar M-AAT% with extended dosing interval

Biweekly (200 mg x 7 doses) Monthly (400 mg x 4 doses)

Mean max M-AAT (% of total AAT)



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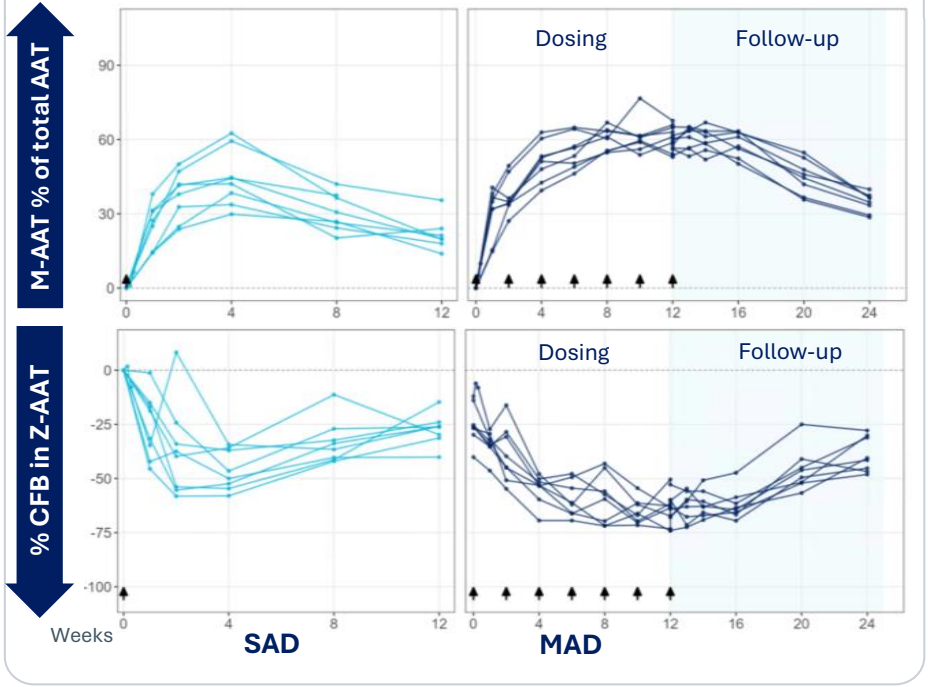
1. In a natural history cohort of individuals with M/Z genotype using the same LC-MS/MS assay in RestorAATion-2, M-AAT% of total AAT mean was 64% (range 57%-71%).
At baseline, serum M-AAT values were below the LLOQ (0.096 µM); the LLOQ value was used for baseline calculations.
Error bars represent the standard error of the mean (SE).

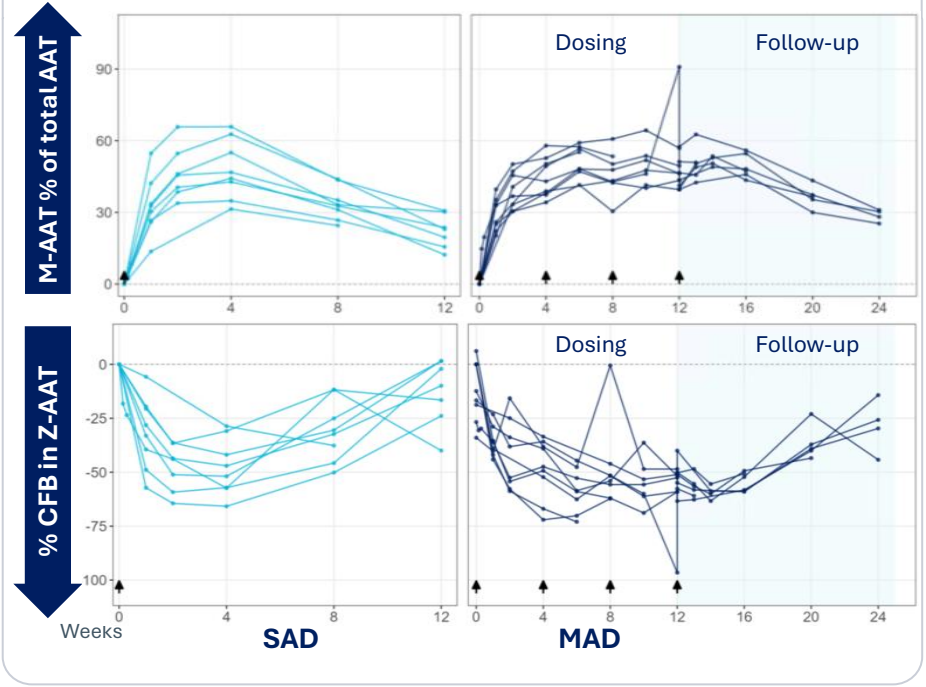
Consistent and durable generation of M-AAT and reductions of Z-AAT support monthly dosing

Editing sustained out to at least three months post last dose

200 mg cohort: Single and biweekly doses



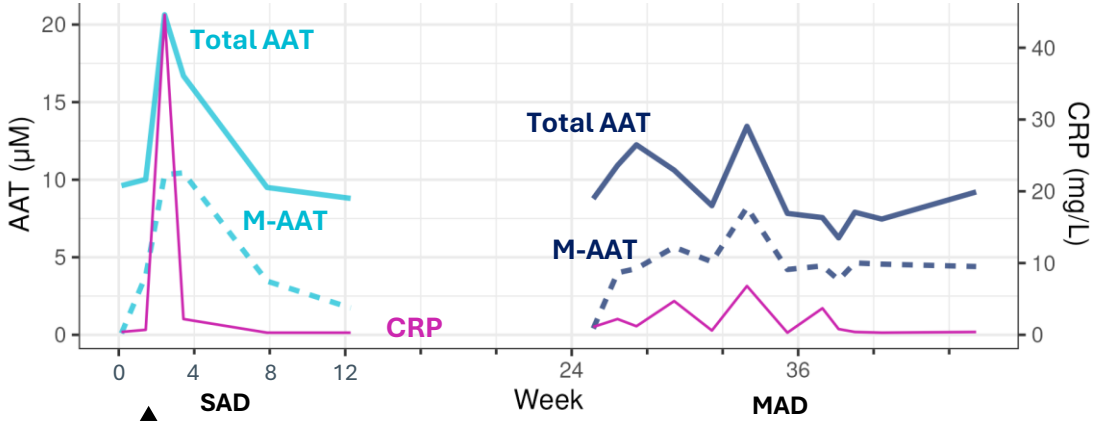
400 mg cohort: Single and monthly doses



Circulating M-AAT, Z-AAT, and total (M + Z) AAT protein in the serum were measured by highly selective and sensitive LC-MS/MS assays. %CFB: percent change from baseline. Arrows indicate scheduled dose administration times of WVE-006.

WVE-006 continues to demonstrate dynamic increases of AAT during acute phase responses

Total AAT reached 20.6 µM two weeks post single 200 mg dose during acute phase response



▲ Acute phase response due to a kidney stone

CRP levels are correlated with AAT elevations across RestorAATion-2

- Two additional acute phase responses (URI-common cold) accompanied by 57.8% and 59.8% increases in AAT from pre-event[1]
- CRP increases are strongly correlated with AAT increases (r=0.73, p<0.001, n=19) [2,3]

Across RestorAATion-2 even low elevations in CRP are associated with increased production of AAT

CRP: C-reactive protein; URI: upper respiratory infection
1 – The most recent non-missing assessment obtained prior to the first observation where CRP exceeded the upper limit of normal (ULN);
2 –Pearson correlation of log-transformed fold changes from pre-event (p < 0.001);
3 – N=19 defined as transitions from normal (≤ ULN) to elevated (> ULN) CRP

WVE-006 continues to demonstrate potential as a best-in-class treatment option

- **Robust and durable RNA editing:**
  - Reductions of circulating, mutant Z-AAT of up to 71% support potential improvement in liver health
  - Restoration of M-AAT of up to 64% (no bystanders) of total, in line with MZ individuals
  - Sustained editing supports monthly dosing
- **Dynamic AAT production:**
  - WVE-006 restores dynamic production of AAT during acute phase responses
  - Three acute phase responses observed; CRP increases correlated with AAT increases
- **Safe and convenient:**
  - Treatment with WVE-006 continues to be safe and well tolerated across all doses, no liver toxicities to date

WVE-006 next steps

Regulatory feedback

- Engaging regulators on accelerated approval with feedback expected mid-2026

RESTORAATION

Complete RestorAATion-2 study

- Data from 600 mg monthly dose cohort expected in 2H 2026

Continue AATD community engagement

- Actively collaborating with global advocacy partners to increase awareness, shorten the diagnostic journey and incorporate community feedback in study protocols, consent forms, and educational materials

Alpha-1 Europe Alliance

ALFA-1 NORDEN

ALPHA-1 Association of Australia

Alpha 1 UK

ALPHA-1 ASSOCIATION NEW ZEALAND

Alpha1 Deutschland e.V.

AlphaNet Canada

ALPHA-1 FOUNDATION

Closing remarks

Paul Bolno, MD, MBA

President & CEO

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WVE-006 has the potential to transform AATD treatment

Reduce Z-AAT to prevent liver disease

Provide healthy, wild-type M-AAT to protect lung

Enable dynamic AAT production during acute phase response

WVE-006 for AATD

Infrequent subcutaneous dosing; potential for self-administration

Generally safe and well tolerated

No bystander edits or other permanent DNA modifications

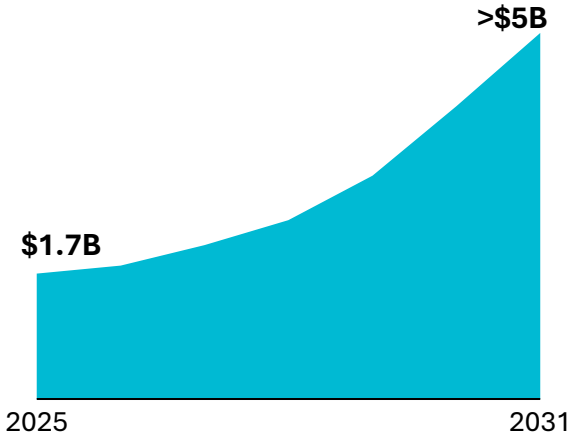
WVE-006 aims to support a differentiated value proposition for Pi*ZZ individuals, HCPs, and payers

AATD is a growing market estimated to reach >$5B globally by 2031

Current Pi*ZZ AATD market

- **~$1.7B global market**
- ~200,000 Pi*ZZ individuals in US and Europe, but only ~10% are diagnosed
- No therapies for AATD liver disease
- Only weekly, IV augmentation therapy for AATD lung disease

AATD market projected to grow to >$5B globally



Availability of new therapies expected to drive:

- Increased AATD awareness and diagnosis
- Increased treatment rate and potentially earlier treatment
- Better coverage outside US vs. augmentation therapy (which is not currently reimbursed in many countries)

WVE-006 offers a differentiated treatment approach for AATD compared to current standard of care and other therapies in clinical development

	Protecting the liver	Protecting the lung		Safe and convenient	
	Reduce Z-AAT	Enable dynamic AAT production	Provide healthy, wild-type M-AAT	Infrequent SC dosing	Safe & well-tolerated
WVE-006 *(investigational)*	✓	✓	**100% wild-type M-AAT**	**SC, Q4W**	✓
Approved augmentation therapy[1]	✗	✗	100% wild-type M-AAT	IV, weekly	✓
Recombinant augmentation therapy[2] *(investigational)*	✗	✗	100% Fc-dimerized AAT	IV, Q3-4W	✓
DNA base editing *(investigational)*	✓	✓	Less than 50% wild-type M-AAT[4]	IV, single dose (uncertain durability, redosing may not be possible)	Liver enzyme elevations[3]; Requires LNPs; Risk of permanent off-target effects, including in cancer-associated genes[5]
AAT siRNA[6] *(investigational)*	✓	✗	✗	SC, Q3M	Potential exacerbation of lung disease due to AAT knockdown[6]

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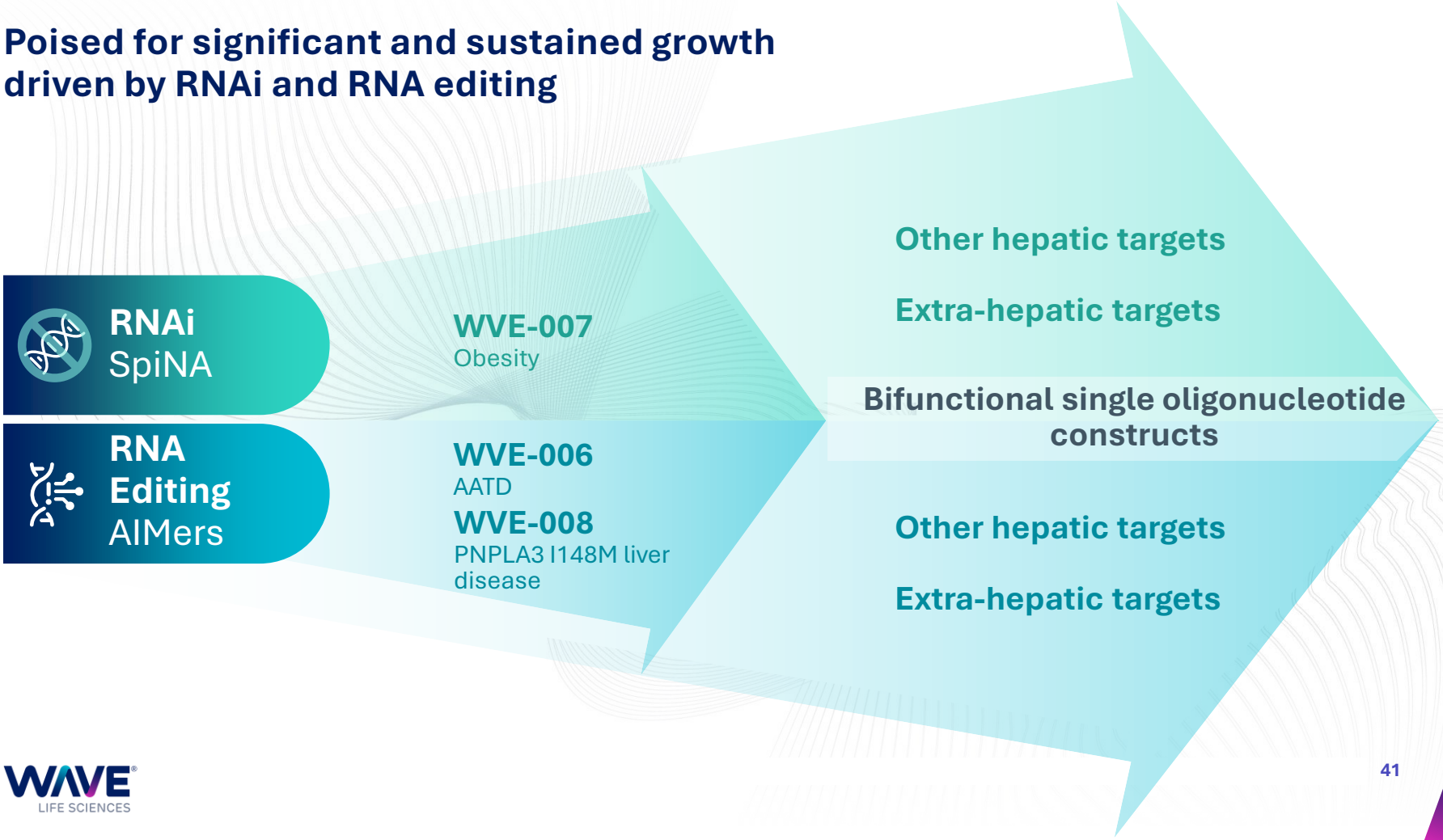
SC: subcutaneous; IV: intravenous. 1. Prolastin label; 2. Barjaktarevic, et al. Efdoralprin Alfa vs. Plasma-derived Alpha-1 Proteinase Inhibitor (pdA1PI) Augmentation Therapy in Adults With Alpha-1 Antitrypsin Deficiency (AATD) Emphysema: Results From Phase 2 ElevAATe Trial [Conference abstract] ATS 2026; 3. BEAM-302 Topline Data Update, March 25, 2026; 4. Banerjee, et al. BEAM-302 decreases hepatic aggregates of mutant AAT and increases circulating functional AAT in rodent models of Alpha-1 Antitrypsin Deficiency [Poster] 2023 ESGCT Congress; 5. Packer, et al. (2024) Methods and Compositions for Treating Alpha-1 Antitrypsin Deficiency (International Patent Application PCT/US2024/034189). https://patentimages.storage.googleapis.com/f7/f0/9a/f88f452755cb16/WO2024259364A2.pdf ; 6. Strnad, et al. 2022 N Engl J Med 387:514-524.

Poised for significant and sustained growth driven by RNAi and RNA editing



Thank you to the participants, families, clinicians, and study site staff who are participating in this study

Q&A

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For questions contact:
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