Half-year update

(There has been a lot going on!)

Dear Alpha-1 UK Members,

As we reach the midpoint of 2025, it's a great time to reflect on the progress we've made so far. The first half of the year has been filled with momentum, learning, and meaningful collaboration. From key project milestones to new ways of working, we've made significant strides—and there's still so much more to come. Thank you to everyone for your continued commitment, energy, and focus as we build on this progress together.

Commemorating 63 Years Since the Discovery of Alpha-1 Antitrypsin Deficiency (AATD)

🧬 What is Alpha-1 Antitrypsin Deficiency?

Alpha-1 Antitrypsin Deficiency (AATD) is an inherited condition where the body produces too little of a protein called **alpha-1 antitrypsin (AAT)**. AAT helps protect the lungs from damage. Without enough of it, people are more likely to develop serious lung and sometimes liver problems.

🔍 A Short History of Alpha-1

- **1963** – Discovered by Swedish scientists Carl-Bertil Laurell and Sten Eriksson, who linked low AAT levels to lung disease (emphysema).
- **1969** – A connection to liver disease was identified in the United States.
- Today, AATD is recognised as one of the **most common inherited conditions**, comparable in prevalence to cystic fibrosis.

📖 Want to find out more? Read a previous newsletter here.

Why Awareness Matters

Despite being common, AATD often goes undiagnosed or is misdiagnosed. Raising awareness helps:

- Patients get diagnosed earlier.
- Families understand their genetic risk.
- Clinicians offer better care.

In April this year, **Alpha-1 UK** collaborated with **Alpha-1 Europe Alliance** to launch **"🧬 The Viking Legacy"** campaign for European Alpha-1 Awareness Day. Inspired by the genetic roots of AATD, the campaign raised awareness through videos, social media posts in 24 languages, and a multilingual website. It encouraged early diagnosis and united patients, healthcare professionals, and policymakers across Europe.

The **Alpha-1 Europe Alliance** is a non-profit, patient-driven umbrella organisation based in Brussels that represents national Alpha-1 Antitrypsin Deficiency (AATD) patient associations (including us) across over 14 European countries. Its mission is to advocate for timely diagnosis, personalised care, holistic therapies, increased awareness, and robust research, working to improve and extend the lives of those affected by Alpha-1 in Europe, and ultimately find a cure.

📽️ Missed it? Then watch it here.

On April 4–5, **Alpha-1 UK** was proud to be represented by our Trustee, **Tanya Jones**, at the **7th Global Research Conference and 10th Patient Congress** in Lisbon, Portugal. Hosted by the Alpha-1 Foundation and supported by the Alpha-1 Europe Alliance, this landmark event brought together participants from over 25 countries—including researchers, healthcare professionals, industry leaders, and patient advocates. It was an inspiring opportunity to share knowledge, foster collaboration, and accelerate progress in Alpha-1 care.

You can read more about the event via the Alpha-1 Europe Alliance here.

Exciting Trial News!!

🧪 Current Treatment Options around the world

Augmentation Therapy (Replacement Therapy)

- Developed in the **1980s**, this treatment boosts AAT levels using protein extracted from donated human blood.
- Administered via intravenous (IV) infusion.
- Approved in **20+ countries** , including the US, Canada, Germany, Spain, France, and Italy.
- **Not yet available in the UK**, Sweden, or Australia due to pending clinical approval.
- Not suitable for:
  People with partial deficiency (e.g. MZ genotype)
  Liver-only AATD patients

Inhaled AAT Therapy

- Delivers AAT directly to the lungs through inhalation.
- Early trials show promising results.
- Currently under **FDA and EU Orphan Drug review**.

🧬 The Future: Gene & Stem Cell Therapies

- Researchers have used **stem cells from skin** to recreate healthy liver cells with corrected AAT genes.
- It could offer a future alternative to liver transplant **without lifelong immune suppression**.
- Still in early stages, but a major step toward personalised treatment.

RESTORAATION

THE WVE-006 TRIAL

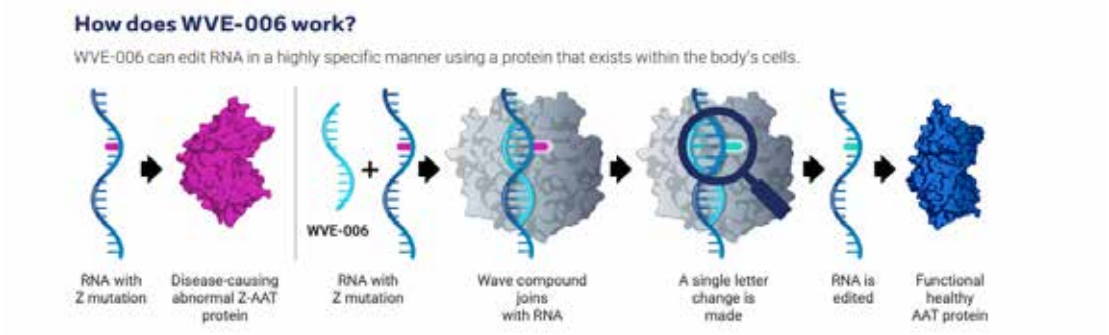
WVE-006 is an investigational therapy made up of single strands of RNA that are designed to address lung and/or liver disease for those living with AATD. It aims to increase the amount of active alpha-1 antitrypsin (AAT) protein in the body and decrease the amount of accumulated AAT protein in the liver.

The WVE-006 clinical program, called “RestorAATion,” is enrolling individuals with ZZ AATD who inherited two genes that cause AATD, one from each parent. We are currently enrolling individuals with ZZ AATD who do not have symptoms or have mild to moderate lung disease and/or mild liver disease

If you are interested in finding out more, you can on these sites:

🔗 www.clinicaltrials.gov

🔗 www.aatdclinicaltrial.com



🌍 Looking Ahead

The science behind AATD has advanced significantly since its discovery. With ongoing breakthroughs in RNA therapy, gene editing, and stem cell research, the future holds real promise for improved quality of life for those living with Alpha-1.

As part of this progress, **Alpha-1 UK** continues to work on behalf of the patient community in an advisory capacity with pharmaceutical companies, healthcare professionals, and policymakers. By ensuring the patient voice is heard at every stage of drug development and care planning, Alpha-1 UK helps to shape more informed, compassionate, and effective health strategies. The charity also plays a key role in raising awareness, funding and promoting research, and providing support and advocacy for individuals and families affected by AATD across the UK.

🎄 Spread Christmas Cheer & Support Alpha-1 UK! 🎄

We know it’s early, but did you know we have beautiful **Alpha-1 UK Christmas cards** that are now available?

Each pack contains 10 cards with envelopes and costs just **£3.95** plus P&P. By purchasing, you’re helping raise vital awareness and support for those affected by Alpha-1 Antitrypsin Deficiency.

✨ Order yours today and make a difference this festive season!

👉 email Tanya@alpha1.org.uk

📣 Fundraising thank-you shout outs

A huge THANK YOU to everyone for your continued support — whether you've been running marathons, baking cakes, or even dressing up as hot dogs (yes, really!), we see you and we appreciate every single effort. Nothing goes unnoticed, and it's thanks to *you* that we can continue raising awareness and supporting those affected by Alpha-1.

Want to see more of the amazing things our community is up to? Head over to our Facebook page to stay inspired and keep up to date with all the incredible stories and people making a difference every day! 📘 Facebook

Perhaps you could help raise funds to enable us to continue our work? Whether £5 or £500, all donations will be put to good use, providing information, equipment and support for all Alpha-1 patients. In addition, we aim to promote better awareness and understanding of AATD-related diseases throughout the medical profession and the general public. To donate or set up your fundraising page, please use **JustGiving**. It's easy (completely free) and only takes 60 seconds to get up and running.

Look out for UK Alpha-1 Awareness month

November marks **Alpha-1 UK Awareness Month** — a time for unity, awareness and advocacy. Throughout the month we will to shine a spotlight on Alpha-1 Antitrypsin Deficiency, raise awareness of the challenges faced by our community, and push for better recognition, diagnosis, and care.

Here in the UK, we're proud to stand alongside our European partners in driving change. Whether it’s through sharing patient stories, raising funds, or engaging with healthcare professionals and policymakers, this month is all about showing the strength and spirit of the Alpha-1 community.

💜 Keep an eye on our social media throughout November as we share inspiring voices, important messages, and ways you can get involved. Together, we are louder — and stronger.

🧡 How You Can Support Us

You can make a big difference by simply following, liking, and sharing our posts on social media—it's a quick and powerful way to help raise awareness of Alpha-1 and support our work. Spreading the word helps us reach more people affected by AATD and connect them with the information, support, and community they need. To stay updated, join the conversation and connect with us online:

🌐 www.alpha1.org.uk 📘 Facebook | 📸 Instagram | 🕊️ X | 💼 LinkedIn

Perhaps you could help us raise funds to continue our work? Whether it's £5 or £500, every donation makes a difference. Your support helps us provide information and assistance to Alpha-1 patients across the UK.

We also work to increase awareness and understanding of AATD-related conditions within the medical community and the general public.

To donate or set up your own fundraising page, please use **JustGiving** – it's easy, completely free, and takes just 60 seconds to get started!

Kind regards,
Alpha-1 Trustees and Committee