

Vol. 22, No. 3 • Winter 2025

A MAGAZINE OF THE ALPHA-1 FOUNDATION

ALPHA-1-TO-ONE

Practical advice, personal experiences and pertinent news for people touched by ALPHA-1

30th Anniversary
Commemorative Issue

Alpha-1-to-One Magazine: Alpha-1 Foundation's 30th Anniversary Commemorative Issue

INNOVATION

30th
1995
2025

INVESTMENT

INSIGHT

IMPACT

ALPHA-1
FOUNDATION

cones
for a
CURE



Letter from Leadership



The Alpha-1 Foundation (A1F) is proud to commemorate its 30th anniversary in 2025. This milestone celebrates and honors the legacy of those who have impacted the Alpha-1 community, especially the Alphas and their families who A1F serves.

For three decades, with the insight of the Alpha-1 community, A1F has designed and launched innovative, patient-focused programs and services supporting Alphas along their journey; including a solid infrastructure to promote pioneering scientific research, and the establishment of collaborative partnerships to advance the development of novel therapies. The essence of A1F's work has resulted in improving the quality of life for those affected by Alpha-1 Antitrypsin Deficiency (Alpha-1).

This year, A1F shares a vision framed by four powerful words that define its work: Insight, Innovation, Investment, and Impact.

INSIGHT: Discovery begins with questions, and insight drives the path toward answers. Over the years, A1F driven research has deepened the understanding of Alpha-1, shedding light on critical pathways and unlocking doors to new possibilities. A1F has expanded its knowledge base significantly,

ALPHA-1-TO-ONE

Practical advice, personal experiences and pertinent news for people touched by Alpha-1, their families and friends.

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building a stronger foundation for the next steps in Alpha-1 research. With each step forward, A1F gains insight that propels the Alpha-1 community closer to lasting solutions.

INNOVATION: A1F is known for tackling challenges with bold, innovative solutions and innovation is the heart of progress. From pioneering studies on Alpha-1 to cutting-edge trials, the team constantly seeks novel solutions that push boundaries and challenge the status quo. A1F has expanded the Therapeutic Development Network (TDN), bringing the Alpha-1 community closer to creating new, accessible treatments and life-changing outcomes. A1F's innovative approach to clinical trial recruitment, utilizing the Alpha-1 Research Registry to prioritize individuals who are willing and able to take part in clinical studies to move impactful research forward.

INVESTMENT: A1F's progress has been fueled by the investment of its supporters; people that believe in the transformative power of philanthropy. Continued investment in A1F is critical to sustain the organization's expanded efforts, allowing A1F to engage new scientists, fund innovative studies, provide education and support to the Alpha-1

community, and provide hope for countless families.

IMPACT: Your continued support resonates through every breakthrough A1F achieves and the life of every Alpha A1F touches. Together, we are creating a legacy of hope, possibility, and progress; one that will resonate for generations to come.

A1F is excited to bring you this special 30th Anniversary edition of the Alpha-1-to-One Magazine, highlighting the milestones and achievements of this outstanding organization and recognizing the last 30 years of **Insight, Innovation, Investment, and Impact**, as the journey continues towards a cure for Alpha-1. Please use the QR codes located throughout this year's volume to learn more about A1F programs and services and how to engage with the Alpha-1 community.

Sincerely,



Scott Santarella

President & CEO
Alpha-1 Foundation

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The Alpha-1 Foundation is committed to finding a cure for Alpha-1 Antitrypsin Deficiency (Alpha-1) and to improving the lives of people affected by Alpha-1 worldwide.

*Diagnosed with Alpha-1 Antitrypsin Deficiency
+Diagnosed Family Member

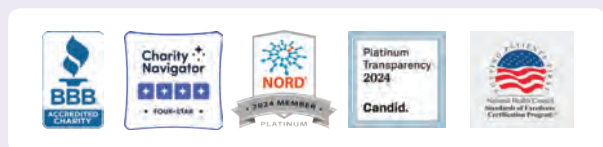


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Alpha-1 Foundation



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The Alpha-1 Foundation Commemorates its 30th Anniversary



“

We—all of us Alphas—had to empower ourselves as a community to find resources to fund research because no one else was doing it.

—John W. Walsh, Co-founder of A1F

”

The National Heart, Lung, and Blood Institute (NHLBI) of the National Institutes of Health (NIH) was nearing the conclusion of its seven-year study on Alpha-1 Antitrypsin Deficiency (Alpha-1), definitively establishing augmentation therapy as an effective treatment. Though not a cure, the therapy provided stability for patient care. As the study ended, it became clear that the future of research and the hope for a cure would depend on Alpha-1 patients taking the lead in building the resources and infrastructure needed to drive progress.

On January 31, 1995, the Alpha-1 Foundation (A1F) was incorporated as a not-for-profit organization by its three Alpha-1 patient co-founders, John W. Walsh, Sandy Lindsey, and Susan Stanley, to find a cure for Alpha-1. Three decades later, A1F is proud to commemorate its 30th Anniversary.

The **insight** of John W. Walsh, Sandy Lindsey, and Susan Stanley, three determined “Alphas” recognized that no further resources were going to be allocated toward Alpha-1 research after the NHLBI study, hence the establishment of A1F with the goal to fund research for a cure. Walsh enlisted world-renowned experts to serve on the first Board of Directors and advisory committees, and to chair scientific workshops and conferences. While the founders have since passed away, with

Susan Stanley passing in 2000, Sandy Lindsey in 2002, and John W. Walsh in 2017, their legacy and work continues to live on.

A1F has been committed to finding a cure for Alpha-1 while improving the lives of those affected worldwide since inception. In pursuit of this mission, A1F has devoted substantial resources to addressing the social and scientific challenges of Alpha-1. The founders set out to establish an innovative scientific infrastructure that would support a powerful grant program aimed at investing in evidenced-based research to find a cure for Alpha-1.

“The Alpha-1 Foundation leads the way worldwide. It is a prime example of how a patient-oriented organization can also be very much research oriented,” shares Prof. Gerry McElvaney, past A1F Board Member and Professor of Medicine at RCSI Education & Research Centre in Ireland.

Walsh’s leadership and passion helped shape A1F into the incredibly successful organization it is today by establishing a broad scope of **innovative** programs including the Alpha-1 Research Registry, the Alpha-1 DNA & Tissue Bank, the Alpha-1 Grants Award program, International Scientific Conferences, and the Gordon L. Snider (GLS) Critical Issues Workshops.

A unique and innovative aspect of A1F is that it started and has continued to be a patient-driven and directed organization; most of the A1F Board of Directors are either diagnosed with Alpha-1 or have a family member diagnosed with Alpha-1. “The thing the Alpha-1 Foundation does so well is it takes the input of the Alpha-1 community to help form the questions we need the answers to. From sitting on the board, the discussion centers around: What are we missing and how can we help? Which is the sign of a very active organization that is trying to do the right thing,” said Virginia Clark, MD, Physician Director for the A1F Board of Directors.

Founded in 1995 by the same individuals who started A1F, AlphaNet was established to provide health maintenance and support services to Alpha-1 patients with a clear mission of coordinating and consolidating low-cost health services, designed exclusively for individuals with Alpha-1, while generating a consistent source of review to support the research mission of A1F. Recognizing the need for **investment** into research, Walsh created this model unlike any rare disease communities had ever seen, launching the current structure of AlphaNet, with “Alphas serving Alphas” through a unique patient-driven business model with customized patient care and innovative disease management, all while funding research for a cure.

The A1F and AlphaNet model began to attract attention and funding, including a \$1 million grant from the Health Foundation of South Florida, that facilitated the 1998 move of Dr. Mark Brantly from the NIH to the University of Florida.

With the help of the Alpha-1 community, A1F has designed and launched new programs and developed a solid infrastructure to promote research and the development of new therapies



2024 A1F Staff



for improving the quality of life for those diagnosed with Alpha-1. It has fostered collaborations with investigators throughout North America, Europe, the Middle East, and Australia, working closely with the National Institutes of Health (NIH), the U.S. Food and Drug Administration (FDA), the pharmaceutical industry, and Alpha-1 patients to expedite development of improved therapies.

AIF's achievements in its first 30 years can be attributed to the ability to bring all constituents and stakeholders to the table with a patient-focused approach to

accelerate innovative approaches to this disease. "The effort the Alpha-1 Foundation has made to connect the scientists, the doctors, and the patients makes the work feel more rewarding," shares Joseph Kaserman, MD, Assistant Professor of Medicine at Boston University.

Recognized for good operating practices, accountability, and responsible governance, AIF has received the National Health Council's Full Compliance Certification and the Charity Seal from the Better Business Bureau's Wise Giving Alliance. These are the highest accreditations

attainable in the not-for-profit and voluntary health sectors. AIF has also received an Exceptional 4-Star rating by Charity Navigator, the highest rating given by that organization. GuideStar has also awarded the Platinum Seal of Transparency, their highest level of recognition, to AIF.

AIF has spent 30 years working for **YOU, the Alpha-1 community**, to improve the lives of Alphas and to find a cure for Alpha-1. It has invested over \$100 million in Alpha-1 research at 130 institutions around the world, spurring countless innovation and impacts. ↑



INSIGHT

Every discovery begins with a question, and insight drives the path toward answers. Over the years, our research has deepened the understanding of Alpha-1, shedding light on critical pathways and unlocking doors to new possibilities. This year we've expanded our knowledge base significantly and funded more first year research grants than ever before, building a stronger foundation for the next steps in our research.



INNOVATION

Innovation is the heart of progress. From pioneering studies on Alpha-1 to cutting-edge trials, our team constantly seeks novel solutions that push boundaries and challenge the status quo. This year we've expanded our Therapeutic Development Network bringing us closer to creating paths to new, accessible treatments and life-changing outcomes for Alphas.

INVESTMENT

Our progress has been fueled by the investment of our supporters; people like you who believe in the transformative power of research. Continued investment in the Alpha-1 Foundation is critical to sustain our efforts, allowing us to engage new scientists, fund innovative studies in Alpha-1, provide education and support to the Alpha-1 community and provide hope for countless families.

IMPACT

The impact of your support resonates through every breakthrough we achieve and every life of every Alpha we touch. Because of you, we are not only conducting research and improving the lives of Alphas, but also building a legacy of hope, one that creates ripple effects in our community and beyond. With your continued support, we are poised to achieve even greater milestones in the coming years, and we are excited to see what the future holds.

Every dollar, every act of generosity, fuels our shared vision of a healthier, better world for every Alpha. Thank you for your unwavering commitment and for being part of the **Insight, Innovation, Investment, and Impact** that defines our work. With your support, let's make this milestone year a turning point for those we serve.

To donate, please visit alpha1.org/donate or scan the QR code below:



A Network of Alpha-1 Specialists Providing Insight for Alpha-1 Patients: A1F Clinical Resource Centers (CRC)



Alpha-1 Foundation (AIF) Clinical Resource Centers (CRCs) are an integrated network of research institutions and physicians specializing in Alpha-1 Antitrypsin Deficiency (Alpha-1) treatment, education, and care. This network was established following the conclusion of the National Institutes of Health (NIH) Study, the NIH Registry, which served as the foundation for the CRCs' formation. The program initially began as the NIH Registry of Patients with Alpha-1 between 1990 and 1996. During this period, 1,129 patients with severe Alpha-1 were enrolled, significantly contributing to the growing network of investigators with specialized expertise in managing Alpha-1. Centers with high enrollment numbers in the NIH Registry were later designated as CRCs by AIF.

Prior to becoming AIF CRCs, these centers were already academic institutions. However, as interest from private pulmonary and hepatology practices grew, a more formal and rigorous screening process was established. This process included a bi-annual review and renewal of the CRC designation to ensure that the centers were maintaining high standards of care and research.

Today, the AIF CRC network includes 98 sites and 110 physicians, with an ongoing effort to expand AIF's Liver CRC footprint in both the adult and pediatric spaces. This expansion is part of AIF's commitment to providing comprehensive care that addresses the full spectrum of needs for individuals living with Alpha-1.

As part of this commitment to high-quality care, Dr. Bruce Trapnell, past AIF Scientific Director, emphasized the crucial role CRCs play in providing specialized care for lung and liver diseases.





CRCs provide comprehensive care to Alphas, including specialized care for lung disease and liver disease. In addition, CRCs provide patients with the resources and information needed to manage their Alpha-1. All CRCs go through a rigorous designation process and are required to adhere to the highest standard of care and best practices. CRCs also play a critical role in helping AIF recruit patients for the Alpha-1 Research Registry.

—Dr. Bruce Trapnell,
past AIF Scientific Director



A key focus for AIF is the development of multi-disciplinary teams within each CRC. Recognizing the complexities of Alpha-1, AIF aims to provide more integrated and coordinated care. These teams are made up of specialists from various disciplines, including pulmonologists, hepatologists, nutritionists, and mental health professionals. By working together, these teams create personalized care plans that address the full range of patient needs, from medical treatment to emotional and psychological support.

Dr. Robert Sandhaus and Dr. James Donohue emphasized the commitment to strengthening the CRCs and ensuring that more individuals can benefit from their expertise while co-chairing the CRC Program. They highlighted AIF’s dedication to further enhancing the CRCs to better serve Alphas and the broader research community. This includes increasing the utilization of the CRC Access Program, designed to ensure more Alphas can access the expertise and support provided by CRCs.

A critical focus is the development of multi-disciplinary teams within each CRC, incorporating specialists from various disciplines. These teams aim to provide integrated and coordinated care


for Alphas, addressing the full spectrum of patient needs, from medical treatment to emotional and psychological support.

Dr. Bradley Drummond, CRC co-chair, also highlighted AIF’s forward-looking approach to expanding and strengthening the CRC network. In a recent presentation he shared, “The CRC really becomes that hub where you can enter into all of the Alpha-1 specialty care in one place. There’s so much collaboration that happens; there’s so much communication that becomes a central hub where you can get excellent care, engage in research, and hear about all the different things that are happening in the Alpha-1 space.”

In addition to its clinical efforts, AIF is committed to ongoing professional development through its Continuing Medical Education (CME) program. This accredited program, developed in partnership with the Cleveland Clinic, aims to help healthcare providers deepen their understanding of Alpha-1 and improve the care they provide to individuals living with Alpha-1.

The CME program is supported by a generous gift from the Stoller family, in memory of Alfred and Norma Stoller, who were passionate about advancing education in the healthcare field. Their gift plays a crucial role in enabling AIF to offer this important educational resource to healthcare professionals.

Upon completion of the program, participants can earn American Medical Association (AMA) Physician’s Recognition Award (PRA) Category 1 Credit, ensuring that they are equipped with the latest knowledge to provide the best care for Alpha-1 patients.

Lastly, the AIF CRC Access Program is a critical initiative that aims to enhance access to expert care for individuals with Alpha-1. Through this program, AIF ensures that more Alphas can benefit from the specialized expertise available at CRCs. For more information, including access to the program details, visit the map and access program information through the QR code below. 



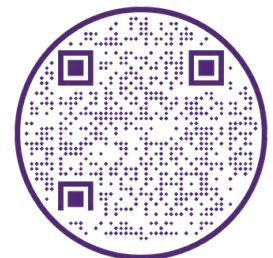


Alpha-1 Foundation Continuing Medical Education Series

The Alpha-1 Foundation and the Cleveland Clinic are partners on a healthcare professional medical education series! Here are some highlights of the program. To take the course, visit: alf.org/CME

- This educational series is designed to aid in the knowledge, diagnostic, and treatment competence needs of healthcare providers to diagnose, treat, and provide better care for Alpha-1 patients.
- The professional education program targets various types of healthcare professionals, including pulmonologists, hepatologists, pediatric lung and liver physicians, liver and lung transplant physicians, nurse practitioners, physician assistants, genetics counselors, respiratory therapists, and internal medicine/general medicine physicians.
- Each video, as part of this series, includes a patient testimonial about their Alpha-1 experience.
- This online program has been approved for *AMA PRA Category 1 Credits™*
- The Foundation has partnered with Dr. Jamie Stoller and the Cleveland Clinic, which is accrediting the program to develop this new healthcare professional, medical education program.

A big thank you to patients, Dr. Stoller and his Cleveland Clinic team, members of the Foundation's Medical and Scientific Advisory Committee, several board of directors members, and Cleveland Clinic clinical staff who volunteered to record videos to help us create a robust Alpha-1 Foundation Continuing Medical Education Series.



Early Innovation in Alpha-1 Detection and Research Programs



The genesis of the Alpha-1 Foundation's (A1F) mission to find a cure for Alpha-1 Antitrypsin Deficiency (Alpha-1) emerged from the insight of A1F's patient founders who understood the pathway to a cure would start with raising funds to support Alpha-1 specific research. Thanks to a relationship built during the original Alpha-1 longitudinal study between a patient and a clinician, a unique partnership was born 30 years ago. John W. Walsh's doctor at the National Heart, Lung, and Blood Institute (NHLBI), Mark Brantly, MD, accepted John's challenge to help create the premier research grant funding program to support research dedicated to improving the understanding of and investigate potential therapies for Alpha-1, with the ultimate goal of someday finding a cure.

Dr. Brantly went to work as a pulmonary fellow at the NHLBI in 1983. Ronald Crystal, MD, who headed the famous pulmonary branch, hired him to work on a little-known genetic condition called Alpha-1. In 1989, The NHLBI Alpha-1 Registry began its seven-year longitudinal study to understand the natural history of Alpha-1. The NHLBI study ran from 1989-1996 with 1,129 Alphas enrolled. Brantly was a steering committee member, ran the central phenotyping and was a principal investigator that saw over 120 Alphas.

The NHLBI Alpha-1 Registry, which started right after the approval of the first augmentation product, is where the Alpha-1 community got its official start. "We met each other at clinical centers, got to know each other, kept in touch and formed support groups. We learned the importance of participating in clinical research and we mobilized ourselves around that theme," said Walsh.

In 1995, Walsh co-founded A1F and AlphaNet. In 1996, The NHLBI Alpha-1 Registry ended, and results showed improved survival and slower rate of decline in lung function for those on augmentation therapy. Augmentation therapy was made possible through the Orphan Drug Act, a 1983 U.S. law intended to help bring drugs to rare disease populations. In 1997, The Alpha-1 Registry was established to facilitate enrollment in research studies to continue research in the disease space.

In 1998, A1F saw the need for an Alpha-1 research and testing lab. Walsh and others enticed Dr. Brantly to relocate to the University of Florida (UF) to focus on Alpha-1 research and A1F helped to fund the new Alpha-1 research program.

Dr. Brantly was appointed as a member of the A1F Board of Directors and began discussing with scientific advisors about the Alpha-1 community's research needs; a research registry, a DNA and tissue bank, a reference lab specializing in Alpha-1, and a high-output diagnostics lab.

The A1F DNA and Tissue Bank was established in 2000 by A1F as a repository for clinical data and tissue samples for alpha-1 deficient individuals and their families. The bank was funded by a grant from Fundacion Leopoldo Fernandes Pujals as a part of a matching grant initiative with the State of Florida.

Reflecting on the early days in his UF lab, Dr. Brantly said, "We needed an easy method to take blood and not have to worry about storage while we were getting it to the lab. I fell upon trying to replicate the dry blood spot methodology (from newborn baby diagnosis). The Alpha-1 Foundation created an endowment for me, so I had some funding, and I cobbled together some additional funds to hire PhD scientists to help with the work."

This effort began the combination of a research and detection program that continued in Brantly's lab until 2014, when the detection program moved

to the privately owned GeneAidix. A1F's Alpha-1 Coded Testing (ACT) study and the A1F DNA Tissue Bank also were operated under the direction of Dr. Brantly at UF.

Research in Alpha-1 is needed to better understand the disease, to discern

what causes emphysema and/or liver disease in only some people with the condition, and to improve current treatments. Participation in research and clinical trials is crucial. In Dr. Brantly's lab, they developed a team of scientists, nurses and administrators to conduct a number of clinical trials and other research projects for individuals who have Alpha-1 and their family members.

The abundance of ongoing clinical trials investigating a diverse array of potential treatment pathways for Alpha-1 is a credit to the efficacy of A1F's pipeline in bringing therapeutic discoveries from benchtop to bedside.

Dr. Brantly's dedicated and active research in Alpha-1 for over 30 years has increased national awareness of Alpha-1 and its effects on deficient individuals. He was influential in expanding the State of Florida detection program and nationwide





Alpha-1 research and patient care over the past 30 years has been an asset to the Alpha-1 community and he promises to be a mentor and participate in Alpha-1 in any way that he can until a cure is found.

Dr. Brantly was a professor of medicine and chief of molecular genetics and microbiology at the University of Florida Department of Medicine, Division of Pulmonary, Critical Care and Sleep Medicine. He also served as the associate director of UF's Clinical Research Program. He was also the endowed AIF research professor at UF. Dr. Brantly served as the AIF Scientific Director from 2019 to 2022. He received the John W. Walsh Public Advisory Roundtable (PAR) Award for Excellence from the American Thoracic Society (ATS) in 2018.

“My impact on detection is just a drop in the bucket compared to what’s been done in the past. In my laboratory, developing some new techniques to do diagnosis, we have screened more than 1 million individuals for Alpha-1, so we have been able to identify around 5,000 Alpha-1 antitrypsin deficient individuals through the lab and it has been my privilege to actually work with some of those patients later on in research studies,” shared Mark Brantly, MD, AIF Board Member.

As we celebrate the 30th Anniversary, AIF extends thanks to Dr. Brantly for his outstanding leadership and his dedication to serving Alphas, the community, and the Foundation. We are grateful for his commitment to innovation and evidenced-based science initiatives to move the mission and the vision of John W. Walsh forward. His tireless dedication to the scientific infrastructure is evident in his continued focus and a career devoted to finding a cure for Alpha-1. 📌

screening programs. Dr. Brantly has countless publications focused on Alpha-1 research. He has been the Principal Investigator on research studies and clinical trials. Dr. Brantly has educated the Alpha-1 community on all topics related to Alpha-1 including lung health, clinical trials, research updates, detection, testing and more.

Dr. Brantly retired from his career at UF in September 2024. He remains an active member of the AIF Board of Directors dedicated to the mission to find a cure for Alpha-1. His commitment to

1995 Research Agenda

- Determine Research Priorities
- Establish workshops focusing on single-issue topics
- Explore curative strategies
- Research Prevention & Correction of Lung Destruction
- Develop Strategies to advance therapy in Alpha-1

VS.

2025 Research Agenda

- Identify Alphas
- Eliminating barriers for Alphas— Ethical, Social & Legal Issues
- Developing & Renewing the Alpha-1 Research Workforce
- Developing & Evaluating Novel Therapeutic Approaches
- Defining the Epidemiology & Natural History of Alpha-1

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AT HOME
WITH NURSE



AT INFUSION
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AT CLINIC

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GLASSIA is a medicine containing human Alpha₁-Proteinase Inhibitor (Alpha₁-PI) that is used to treat adults with lung disease (emphysema) because of severe Alpha₁-antitrypsin (Alpha₁) deficiency. GLASSIA is not meant to be used as a therapy for lung disease other than severe Alpha₁ deficiency. Effects of GLASSIA on worsening lung function and emphysema progression have not been proven in clinical trials. Long-term effects of Alpha₁ replacement and maintenance therapy have not been studied.

IMPORTANT SAFETY INFORMATION

What is the most important information I need to know about GLASSIA?

- GLASSIA can cause severe allergic reactions including hives, swelling in the mouth or throat, itching, tightness in the chest, trouble breathing, wheezing, faintness or low blood pressure
- If you will be taking GLASSIA outside a healthcare setting, ask your healthcare provider (HCP) about an epinephrine pen and/or other supportive care for certain severe allergic reactions.

Who should not use GLASSIA?

Do not use GLASSIA if you:

- Have immunoglobulin A (IgA) deficiency with antibodies to IgA
- Have a severe allergic reaction to human Alpha₁-PI products.

IMPORTANT SAFETY INFORMATION (continued)

What are the possible or reasonably likely side effects of GLASSIA?

If any of the following problems occur contact your healthcare provider (HCP) or call emergency services right away:

- Worsening or flare-up of your chronic obstructive pulmonary disease (COPD)
- Hives, swelling in the mouth or throat, itching, chest tightness, trouble breathing, wheezing, fainting or dizziness. These could be signs of a serious allergic reaction.

The most common side effects that may occur are headache and upper respiratory tract infections.

Other possible side effects of GLASSIA include:

- Cough
- Sinus infection
- Chest discomfort
- Dizziness
- Increased liver enzymes
- Shortness of breath
- Nausea
- Fatigue

These are not all the possible side effects. Tell your HCP about any side effect that bothers you or that does not go away. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

Please see the Important Facts About GLASSIA on the next page.

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Patient Information

GLASSIA (Alpha₁-Proteinase Inhibitor (Human)) Injection, For Intravenous Use

The following summarizes important information about GLASSIA (pronounced glass-see-ä). Please read it carefully before using this medicine. This information does not take the place of talking with your healthcare professional, and it does not include all of the important information about GLASSIA. If you have any questions after reading this, ask your healthcare professional.

What is the most important information that I should know about GLASSIA?

Severe allergic reactions can occur with GLASSIA. Your doctor will inform you about signs of allergic reactions which include hives, swelling in the mouth or throat, itching, tightness in the chest, trouble breathing, wheezing, faintness, low blood pressure, or serious allergic reaction.

If you have any of these reactions, discontinue use of the product and contact your physician and/or seek immediate emergency care, depending on the severity of the reaction.

If you or your caregiver will be administering GLASSIA outside a healthcare setting, ask your doctor about an epinephrine pen and/or other supportive care for certain severe allergic reactions.

Ask your doctor to make sure you receive training on how and when to use any prescribed supportive care medicine and keep it close at hand when administering GLASSIA.

What is GLASSIA?

GLASSIA is a liquid medicine containing human Alpha₁-Proteinase Inhibitor (Alpha₁-PI) also known as alpha₁-antitrypsin (AAT), which is purified from human blood. The main purpose of infusing GLASSIA is to increase the levels of the AAT protein in your blood and lungs. AAT protein protects the lung tissue by blocking certain enzyme-caused damage. Such damage can lead to severe lung disease, such as emphysema.

Limitations of Use:

- The effects of increasing the AAT protein levels with GLASSIA or any other Alpha₁-PI product on worsening pulmonary function and progression of emphysema have not been proven in clinical trials.
- The long-term effects of AAT replacement and maintenance therapy with GLASSIA have not been studied.
- GLASSIA is not intended as a therapy in individuals with lung disease other than severe Alpha₁-PI deficiency.

Who should not take GLASSIA?

You should not use GLASSIA if you:

- Have immunoglobulin A (IgA) deficiency with antibodies to IgA
- Have had a severe allergic reaction to human Alpha₁-PI products

How should I take GLASSIA?

- GLASSIA is given directly into the bloodstream.
- You can get GLASSIA at your healthcare professional's office, clinic, hospital, or delivered directly to your home by a healthcare professional from a limited network of specialty pharmacy providers.
- Your healthcare professional will decide if self-infusion in your home is right for you. You should be trained on how to do infusions by your healthcare professional.

What should I tell my healthcare professional before I start using GLASSIA?

Before starting GLASSIA, tell your healthcare professional if you:

- Have IgA deficiency with antibodies to IgA.
- Have a history of severe allergic reactions to Alpha₁-PI products.

What are the possible or reasonably likely side effects of GLASSIA?

- A possible side effect to GLASSIA is worsening or flare-up of your chronic obstructive pulmonary disease (COPD) in which your breathing gets worse than usual.
- Call your healthcare professional or go to your emergency department right away if you get: Hives, swelling in the mouth or throat, itching, chest tightness, trouble breathing, wheezing, fainting or dizziness. These could be signs of a serious allergic reaction.
- The most common side effects are headache and upper respiratory tract infections. Other possible side effects of GLASSIA include: cough, sinus infection, chest discomfort, dizziness, increased liver enzymes, shortness of breath, nausea, and fatigue.

These are not all of the possible side effects for GLASSIA. You can ask your healthcare professional for information that is provided to healthcare professionals. Talk to your healthcare professional about any side effects that bother you or that don't go away.

How do I store GLASSIA?

Store GLASSIA refrigerated or at room temperature.

- You can store GLASSIA in the refrigerator (36°F to 46°F [2°C to 8°C]). Do not freeze.
- You can store GLASSIA at room temperature (up to 77°F [25°C]) for up to one month. You must use GLASSIA within one month once you remove it from the refrigerator. Do not re-refrigerate GLASSIA once the product has been stored at room temperature.
- Keep the GLASSIA vial in the box until you are ready to administer the product.

Check the expiration date on the carton and vial label. Do not use GLASSIA after the expiration date.

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Manufactured by:

Takeda Pharmaceuticals U.S.A., Inc.

Lexington, MA 02421 USA

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Revised: September 2023

GLA378

US-GLA-0459v1.0 03/24



Bringing the Brightest Minds Together to Advance Alpha-1 Research: The Gordon L. Snider (GLS) Critical Issues Workshop Series

Scientific meetings and conferences organized by the Alpha-1 Foundation (A1F) are designed to answer important questions about Alpha-1 Antitrypsin Deficiency (Alpha-1) and identify new areas of research, questions, important answers, and a resulting publication. One of the unique aspects of A1F is its long history of convening experts in a field to address critical issues.

The Gordon L. Snider (GLS) Critical Issues Workshops are single-topic workshops that facilitate exchange between experts to answer important questions or stimulate additional investigation and often result in collaborative, field advancing initiatives. The series is named after the late Dr. Gordon L. Snider, one of the most influential researchers in Alpha-1. Dr. Snider not only made seminal scientific contributions that have clarified the pathogenesis of Alpha-1 but also laid the groundwork for A1F's research program. In particular, he stressed the importance of encouraging young investigators to pursue a career in Alpha-1 research. Dr. Snider served on the A1F Board of Directors, where he helped guide not only scientific research but also the focus on the whole organization.



He was a pioneer in emphysema research, a past president of the American Thoracic Society (ATS), chair of the department of medicine at the Boston VA Hospital, and author of hundreds of scientific papers, opinion pieces, and editorials. His work bridged basic science and the clinical aspects of the disease. I think of Gordon as one of the first translational researchers in pulmonary diseases.

—Mark Brantly, MD,
A1F Board Member



History of the Gordon L. Snider (GLS) Critical Issues Workshop Series:

1999

- Aerosolized Therapies for Alpha-1 Antitrypsin Deficiency:
- Screening and Detection

2001

- Computed Tomographic Scan Workshop
- Inflammatory Markers

2002

- Environmental, Occupational, and Genetic Risk
- Models of Emphysema: Speeding the Pace of Progress
- Stem Cell Therapies in Reparative Medicine

2003

- The Impact of Genetic Testing: Ethical, Legal, and Social Issues

2004

- Chronic Bronchial Injury in Humans & Models

2008

- Quantitative Chest Tomography in COPD Research

2010

- The Promise and Challenge of GINA: Is it Time for Newborn Screening for Alpha-1?

2011

- New Formulations and Applications of Alpha-1 Antitrypsin

2014

- Clinical Trial Design for Alpha-1 Antitrypsin Deficiency: A Model for Rare Diseases

2015

- Can Cystic Fibrosis (CF) and Alpha-1 Antitrypsin Deficiency (AATD) Inform COPD?

2016

- Ethical Issues Related to Clinical Research and Rare Diseases

2017

- MZ Carrier State in Alpha-1 Antitrypsin Deficiency

2018

- The Alpha-1 Foundation Approach to the COPD National Action Plan

2019

- Detection of Alpha-1 Antitrypsin Deficiency: The Past, Present and Future

2020

- Biomarkers and Endpoints for Drug Development in Alpha-1 Antitrypsin Deficiency
- The Promise of Gene-based Interventions in Alpha-1 Antitrypsin Deficiency

2021

- Exacerbations and Pulmonary Comorbidities
- CT Lung Density Estimates in Alpha-1 Antitrypsin Deficiency: State of the Art
- Liver Biomarkers and the Path to Alpha-1 Liver Therapies

2022

- The Alveolar Macrophage: Role in Lung Destruction and AATD

2023

- Z Variant Heterozygosity in Alpha-1 Antitrypsin Deficiency: Disease Risk and Treatment Implications

2024

- Pragmatic Solutions to Clinical Trial Endpoints for Lung Disease in AATD, and AAT Variant Nomenclature
- Experimental Models of Alpha-1 Antitrypsin Deficiency Lung and Liver Disease

2025

- October - 28th GLS Critical Issues Workshop



The Gordon L. Snider Scholar Award was created after Gordon L. Snider, MD passed away in 2013 at age 91. The first recipient of the award was Karina Serban, MD (National Jewish Health and now at the University of Florida) in 2015. In 2018, two recipients were awarded, Monica Goldklang, MD (Columbia University Irving Medical Center) and Pasquale Piccolo, PhD (Telethon Institute of Genetics and Medicine).

AIF has successfully hosted 27 GLS Critical Issues Workshops since 1999, bringing together the top minds in the research and medical fields to discuss single-issue topics that foster exchange between experts, answer important questions, and/or prompt additional investigation.

The AIF mission is profoundly enriched by its commitment to hosting these pivotal gatherings for the research and scientific community. These workshops are a cornerstone of AIF's efforts to ignite enthusiasm within the field and solidify its reputation as a leader within the rare disease community.

“Dr. Snider moved to Boston in 1968 to become the head of the pulmonary section at Boston University (BU), as well as the Chief of Pulmonary at the VA. Ultimately, he developed the research program in lung cell and molecular biology that I’m a product of and made BU what it is today in terms of lung disease research, and I think Gordon was ahead of his time. The title of his last publication called ‘Only Cell and Molecular Biology Can Lead to an Understanding of the Pathogenesis of Lung Disease,’ truly captured the spirit of our last workshop titled ‘Experimental Models of Alpha-1 Antitrypsin Deficiency Lung and Liver Disease.’ This is an incredible time. We have made amazing technical advances that we are starting to see applied to Alpha-1, and we are incredibly excited about the potential application of those advances on our patients,” said Andrew Wilson, MD, AIF Scientific Director. 📌



RARE DISEASE DAY®



Rare Disease Day

FEBRUARY 28, 2025

THERE ARE 300 MILLION PEOPLE
WORLDWIDE LIVING WITH A RARE DISEASE.

#RARE
DISEASE
DAY

EVERYONE HAS A UNIQUE ALPHA-1 STORY.
SHARE YOURS WITH US!

VISIT [ALPHA1.ORG/RARE-DISEASE-DAY](https://alpha1.org/rare-disease-day)



Discover the ZEMAIRA difference...

...available in 1-, 4-, and 5-gram vials



The first and only Alpha-1 therapy to offer 1-, 4-, and 5-gram vials to help streamline preparation and offer greater simplicity for people with Alpha₁ antitrypsin deficiency (AATD).

IMPORTANT SAFETY INFORMATION

ZEMAIRA[®], Alpha₁-Proteinase Inhibitor (Human), is indicated to raise the plasma level of alpha₁-proteinase inhibitor (A₁-PI) in patients with A₁-PI deficiency and related emphysema. The effect of this raised level on the frequency of pulmonary exacerbations and the progression of emphysema have not been established in clinical trials.

ZEMAIRA may not be suitable for everyone; for example, people with known hypersensitivity to components used to make ZEMAIRA, those with a history of anaphylaxis or severe systemic response to A₁-PI products, and those with certain IgA deficiencies. If you think any of these may apply to you, ask your doctor.

Early signs of hypersensitivity reactions to ZEMAIRA include hives, rash, tightness of the chest, unusual breathing difficulty, wheezing, and feeling faint. Immediately discontinue use and consult with physician if such symptoms occur.

In clinical studies, the following adverse reactions were reported in at least 5% of subjects receiving ZEMAIRA: headache, sinusitis, upper respiratory infection, bronchitis, fatigue, increased cough, fever, injection-site bleeding, nasal symptoms, sore throat, and swelled blood vessels.

Because ZEMAIRA is made from human blood, the risk of transmitting infectious agents, including viruses and, theoretically, the Creutzfeldt-Jakob disease (CJD) agent and its variant (vCJD), cannot be completely eliminated.

Please see brief summary of prescribing information on following page and full prescribing information for ZEMAIRA available at ZEMAIRA.com.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

You can also report side effects to CSL Behring's Pharmacovigilance Department at 1-866-915-6958.

ZEMAIRA®, Alpha₁-Proteinase Inhibitor (Human)
lyophilized powder for reconstitution for intravenous use
Initial U.S. Approval: 2003

BRIEF SUMMARY OF PRESCRIBING INFORMATION

These highlights do not include all the information needed to use ZEMAIRA safely and effectively. See full prescribing information for ZEMAIRA.

-----INDICATIONS AND USAGE-----

- ZEMAIRA is an alpha₁-proteinase inhibitor (A₁-PI) indicated for chronic augmentation and maintenance therapy in adults with A₁-PI deficiency and clinical evidence of emphysema (1).
- The effect of augmentation therapy with ZEMAIRA or any A₁-PI product on pulmonary exacerbations and on the progression of emphysema in A₁-PI deficiency has not been demonstrated in randomized, controlled clinical studies (1).
- ZEMAIRA is not indicated as therapy for lung disease patients in whom severe A₁-PI deficiency has not been established (1).

-----DOSAGE AND ADMINISTRATION-----

For intravenous use after reconstitution only (2).

- The recommended weekly dose of ZEMAIRA is 60 mg/kg body weight. Dose ranging studies using efficacy endpoints have not been performed with ZEMAIRA or any A₁-PI product (2).
- Administer through a suitable 5 micron infusion filter (not supplied) at room temperature within 3 hours after reconstitution (2.2).
- Do not mix with other medicinal products. Administer through a separate dedicated infusion line (2.2).
- Administer at a rate of approximately 0.08 mL/kg/min as determined by the response and comfort of the patient (2.2).
- Monitor closely the infusion rate and the patient's clinical state, including vital signs, throughout the infusion. Slow or stop the infusion if adverse reactions occur. If symptoms subside promptly, the infusion may be resumed at a lower rate that is comfortable for the patient (2.2).

-----DOSAGE FORMS AND STRENGTHS-----

ZEMAIRA is supplied in a single-dose vial containing approximately 1000 mg, 4000 mg, or 5000 mg of functionally active A₁-PI as a white to off-white lyophilized powder for reconstitution with 20 mL, 76 mL, or 95 mL of Sterile Water for Injection, USP. The amount of functional A₁-PI is printed on the vial label and carton (3).

-----CONTRAINDICATIONS-----

- History of anaphylaxis or severe systemic reactions to ZEMAIRA or A₁-PI protein (4).
- Immunoglobulin A (IgA)-deficient patients with antibodies against IgA, due to the risk of severe hypersensitivity (4).

-----WARNINGS AND PRECAUTIONS-----

- Observe any signs of hypersensitivity such as tachycardia, hypotension, confusion, syncope, oxygen consumption decrease, and pharyngeal edema when administering ZEMAIRA to patients with known hypersensitivity to an A₁-PI product (5.1).
- Patients with selective or severe IgA deficiency can develop antibodies to IgA and, therefore, have a greater risk of developing potentially severe hypersensitivity and anaphylactic reactions. If anaphylactic or severe anaphylactoid reactions occur, discontinue the infusion immediately (5.2).
- Because ZEMAIRA is made from human blood, it may carry a risk of transmitting infectious agents, e.g., viruses, the variant Creutzfeldt-Jakob disease (vCJD) agent and, theoretically, the Creutzfeldt-Jakob disease (CJD) agent (5.3).

-----ADVERSE REACTIONS-----

- Serious adverse reactions reported following administration of ZEMAIRA in pre-licensure clinical trials included one event each in separate subjects of bronchitis and dyspnea, and one event each in a single subject of chest pain, cerebral ischemia and convulsion.
- The most common adverse reactions occurring in at least 5% of subjects receiving ZEMAIRA in all pre-licensure clinical trials were headache, sinusitis, upper respiratory infection, bronchitis, asthenia, cough increased, fever, injection site hemorrhage, rhinitis, sore throat, and vasodilation (6).

To report SUSPECTED ADVERSE REACTIONS, contact CSL Behring Pharmacovigilance at 1-866-915-6958 or FDA at 1-800-FDA-1088 or www.fda.gov/med-watch.

Based on September 2022 revision

Impacting Alphas Worldwide: Alpha-1 Global Program



AATD Therapeutics In Clinical Trials

Phase, Estimated Completion Date*, Therapy Type, as of 5-Mar-2023



AATD Therapeutics In Clinical Trials

Phase, Estimated Completion Date*, Therapy Type, as of 5-Mar-2023



Alpha-1 Global 8th Patient Congress





In 2013, after the Alpha-1 Global Patient Congress was held in Barcelona, the Alpha-1 Foundation (A1F) officially launched the Alpha-1 Global program, designed for Alphas around the world to communicate, collaborate, and exchange news, information, and ideas to improve awareness, detection, and access to care worldwide. Global patient leaders, physicians and scientists came together to discuss the latest developments in patient advocacy, clinical care, and Alpha-1 Antitrypsin Deficiency (Alpha-1) research.

With a rich history of international collaboration, A1F is committed to improving the lives of people affected by Alpha-1 and to increase awareness, detection, and access to care for Alphas around the world.

Global Priorities

Access to Treatment: Plasma is crucial for Alphas—for the development of treatments and ultimately for improving their lives. The European Union (EU) Substance of Human Origin (SoHO) Regulation focuses on plasma collection and distribution within the EU aiming to ensure its self-sufficiency. A1F, together with Alpha-1 patient organizations around the world, engages with national policymakers, contributes patient perspectives on the way plasma is collected throughout the EU and ultimately ensures that there is sufficient plasma supply for all patients relying on plasma-derived medicinal products (PDMPs) in Europe. A1F has developed an extensive advocacy toolkit available for global patients to collaborate in the most effective manner for awareness, detection, and access to care.

Patient Education: Alpha-1 patients play a pivotal role in effecting change. A1F provides support for patient advocates and underscores the importance of amplifying patient voices in policy debates and discussions. Alpha-1 resources and educational materials are available in a variety of languages for distribution and also online. The Alpha-1 National Education Series is available virtually and is open to all

global Alphas to provide current medical information, topics affecting Alphas, resources from leading Alpha-1 experts, and opportunities to connect with the community.

International Community: Just as A1F has created a cohesive and highly motivated community for people with Alpha-1, it has also cultivated an international network of scientists committed to applying their specialties to explaining all aspects of the condition and research towards a cure.

A1F International Research Conferences and Alpha-1 Global Patient Congresses

- **2003: Barcelona, Spain**
- **2004: Dublin, Ireland**
- **2007: Rome, Italy**
- **2013: Barcelona, Spain**
- **2015: Barga, Italy**
- **2017: Lisbon, Portugal**
- **2019: Dubrovnik, Croatia**
- **2021: Virtual**
- **2023: Dublin, Ireland**
- **2025: Lisbon, Portugal**

Held every other year, the A1F International Research Conference and Global Patient Congress provides incredible opportunities for representatives from the Alpha-1 patient, caregiver, medical, industry, and government communities around the world to come together and connect on the most current and relevant information about Alpha-1.

Each Alpha-1 International Research Conference brings the global Alpha-1 research community together to forge partnerships that will bring us closer to a cure for Alpha-1. A multidisciplinary group of scientists and experts present and discuss novel treatment paradigms in Alpha-1 to accelerate research toward a cure.

The Alpha-1 Global Patient Congress provides a platform for education, discussion, and networking to further the mission of Alpha-1 organizations around the world. The program includes research and therapeutic updates, access to care discussions, medical collaboration, patient engagement in research, advocacy advancement through public policy and clinical practice, mental and physical wellness, and the importance of awareness, testing, and detection.





Goals of the Alpha-1 Patient Congresses:

- Present the latest education concerning Alpha-1 research, therapies and patient care
- Develop pathways for expanded access to augmentation therapy
- Broaden individual awareness and participation and inspire local action
- Identify possible next steps for organizations and individuals who are looking for new solutions
- Exchange best practice and success stories in advocacy and collaborative efforts

- Provide networking opportunities between patients and experts internationally

Alpha-1 Europe Alliance

A1F is working collaboratively with the Alpha-1 Europe Alliance to represent the Alpha-1 patient community voice. The Alpha-1 Europe Alliance is a collaborative effort by various national patient organizations across Europe to advocate for access to timely diagnosis, individualized care, and holistic therapies, while raising awareness, promoting

The Alpha-1 Foundation

7th Global Research Conference & 10th Patient Congress

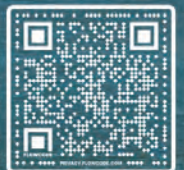


April 4 - 5, 2025
Lisbon, Portugal


For more information,
please contact:
Adriana De Arce at
adearce@alpha1.org

For industry inquiries,
please contact:
Angela McBride at
amcbride@alpha1.org

LEARN MORE



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research, and empowering Alpha-1 national patient organizations. Annual EU Alpha-1 Awareness Day is observed on April 25th each year and is dedicated to raising awareness about the genetic condition across Europe, encouraging testing and early diagnosis. Held for the past seven years, EU Alpha-1 Awareness Day allows the European Alpha-1 community to come together to share their Alpha-1 stories, experiences, and hardships. The goal of this day is to provide information on this genetic condition to achieve increased rates of detection and diagnosis and ultimately achieve greater access to treatment for those affected by Alpha-1. During November, members of the European Parliament joined A1F in raising awareness for Alpha-1 with a series of videos shared on A1F's social media platforms. Together, we've highlighted the urgent need for early diagnosis, better support, and improved care for Alpha-1 patients. 





In 2025, the Alpha-1 Foundation (A1F) proudly commemorates its 30th anniversary. This milestone celebrates and honors the legacy of the many who have made an impact on the Alpha-1 community, especially the patients and families A1F serves.

Over the course of three decades, and with the insight of the Alpha-1 community, A1F has designed and launched innovative programs and services to help patients along their journey; this includes a solid infrastructure to promote research and pioneering science, and the establishment of collaborative partnerships to advance the development of novel therapies. The essence of A1F's work has resulted in improving the quality of life for those diagnosed with Alpha-1 Antitrypsin Deficiency (Alpha-1).

A1F's achievements can be attributed to the organization's ability to bring together a vast network of stakeholders with a patient-focused approach. The dedication of the entire community to A1F's mission to find a cure for Alpha-1 will continue without falter for as long as it takes. Please join the Alpha-1 Foundation in recognizing the last 30-years of Insight, Innovation, Investment, and Impact as the journey continues toward a cure for Alpha-1.

The A1F mission is supported by you, the Alpha-1 patient and your families, who make it possible for A1F to provide the resources and services to the Alpha-1 community and accelerate research forward. Thank you for your investment of funds, time, and talent.



Together, We are Making a Difference:
Building Friends for a Cure (BFC)



The Alpha-1 Foundation (A1F) 30th anniversary seal builds on and complements the strong A1F brand. The core square shape of the seal symbolizes the cornerstone that A1F is for the entire Alpha-1 community.

Incorporating the sharp triangular shape of the highly recognized A1F logo, the square represents a strong framework for A1F's four interconnected pillars:

- ▲ Insight
- ▲ Innovation
- ▲ Investment
- ▲ Impact

The soft glow at the center emanates outward toward the future, shining on you, the Alpha-1 patient and essential members of the Alpha-1 community helping A1F achieve its mission to find a cure for Alpha-1 Antitrypsin Deficiency (Alpha-1) and improve the lives of people affected by Alpha-1 worldwide.



The Alpha-1 Foundation: Moving Forward for 30 Years and Counting

1995

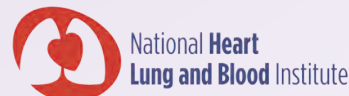


The Alpha-1 Foundation is established to provide leadership and resources with the mission to find a cure for Alpha-1.



AlphaNet founded to provide health management for Alphas.

1996



The Alpha-1 Foundation Clinical Resource Centers (CRCs) are established at NHLBI study sites.

1997



The Alpha-1 Foundation Research Registry is established to facilitate enrollment in research studies.



1st international scientific meeting to promote Alpha-1 research across disciplines.



Health Foundation of South Florida gives first \$1 million donation to Alpha-1 Foundation.

1998

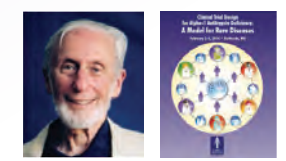


Alpha-1 Detection Laboratory opens at the University of Florida.



Alpha-1 DNA & Tissue Bank established to provide a reliable source for Alpha-1 researchers.

1999



Gordon L. Snider (GLS) Critical Issues Workshop series begins.



Prolastin Direct program launches for distribution by AlphaNet.

2000

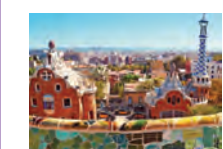


The Fernandez Family provides a \$5M donation for liver-focused research grants.

2003



The Alpha-1 Foundation organized and funded ATS-ERS guidelines for treatment of Alpha-1 is published.



1st International Alpha-1 Patient Congress held in Barcelona.



FDA approves additional Alpha-1 augmentation therapies, Aralast and Zemaira.

2005



Building Friends for a Cure (BFC) community fundraising program begins.

2006



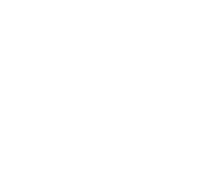
Genetic Information Nondiscrimination Act (GINA) passes in the US, preventing discrimination by employers and health insurers based on genetic information.

2010



Cadwgan Family Foundation makes \$1M donation to Alpha-1 research programs.

2014



The Alpha-1 Association merges into the Alpha-1 Foundation.



Alpha-1 Global initiative launches to support the global Alpha-1 community.

2015



The Alpha-1 Association merges into the Alpha-1 Foundation.



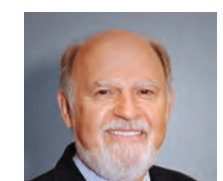
Alpha-1 Global initiative launches to support the global Alpha-1 community.

2016



The John W. Walsh Translational Research Fund is created and named after John W. Walsh, co-founder of the Alpha-1 Foundation and AlphaNet.

2020



The John W. Walsh Translational Research Fund is created and named after John W. Walsh, co-founder of the Alpha-1 Foundation and AlphaNet.

2022

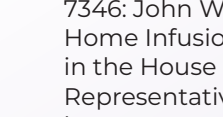


Introduction of H.R. 7346: John W. Walsh Home Infusion Act in the House of Representatives to improve access to augmentation therapy infusions at home for individuals with Alpha-1.

2023



Professional education CME program established with Cleveland Clinic to aid in the knowledge, diagnostic and treatment competence needs to improve as clinicians and healthcare providers need to learn about Alpha-1.

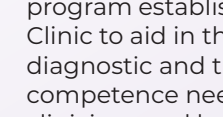


The Alpha-1 Research Registry celebrates the 3,000th participant enrolled.

2024



The Alpha-1 Research Registry celebrates the 3,000th participant enrolled.



The Alpha-1 Research Registry celebrates the 3,000th participant enrolled.

2025



Alpha-1 Foundation celebrates its 30th Anniversary.



The Alpha-1 Project (TAP) is established to accelerate therapeutic development for Alpha-1 lung and liver diseases.



FDA approves Glassia, first liquid augmentation therapy for Alpha-1.



NIH Awards Multicenter Rare Disease Grant to Alpha-1 Antitrypsin Disease Cohort: Longitudinal Biomarker Study of Disease.

The Alpha-1 Biomarkers Consortium (AIBC) is created.



Amid COVID-19 pandemic, AIF provides the Alpha-1 community online access to events, education and resources, developing a revolutionary Virtual Environment (VE) Platform bringing live presentations, community networking, and resources to Alpha-1 patients and families in the safety of their homes.



AIF announces the inaugural recipients of the 2023 Ab Rees Fast Track Grants, funded through AlphaNet donation in honor of Ab Rees' legacy—four \$50,000 grants to fund the preliminary work needed to apply for ancillary funding from the NIH or the Alpha-1 Foundation for the AIBC study.



The Therapeutic Development Network (TDN) enrolls its 1st patient.



Publication of the first tool to predict childhood liver disease risk from the Childhood Liver Disease Research Network (ChiLDReN), heavily funded by AIF.



Over \$100 million invested in research at over 130 institutions funded.

Alpha-1 Foundation's investment in research around the globe hits the \$100 million mark!

Since 2005, the Building Friends for a Cure (BFC) program has fostered Alpha-1 community involvement, encouraging people to come together to make a difference for Alpha-1 Antitrypsin Deficiency (Alpha-1). It is a community built on hope, action, and the belief that we are stronger when we stand together. From the beginning, the BFC program has captured the best of what this community represents — individuals and families affected by Alpha-1 supporting one another while raising critical funds for Alpha-1 research, programs and awareness.

The foundation for the BFC program was laid years earlier with the success of Team Alpha-1, a program that significantly increased Alpha-1 awareness nationwide. Recognizing its success, AIF built on its framework by integrating fundraising and officially launched Building Friends for a Cure. This innovative approach has positioned AIF as a leader in the not-for-profit sector for peer-to-peer fundraising, creating a model that inspires both engagement and results.

BFC community fundraisers not only raise funds for Alpha-1 research and programs but also educate and empower participants with valuable Alpha-1 knowledge. The program's influence extends beyond the patient community. By involving physicians, researchers, and industry partners, the BFC program offers a unique opportunity for stakeholders to gain firsthand insight into life with Alpha-1. Their active involvement fosters a deeper understanding and a renewed commitment to making a difference. Time and again, participants walk away educated, engaged, and empowered to further support the Alpha-1 cause.

The unwavering support of the entire community has been the true driving force behind

this program's success. When challenged to fund a cure for Alpha-1, they stepped up with extraordinary passion and determination. For the past 20 years, their efforts, spirit, and determination have demonstrated the power of collective action and resilience.

One inspiring example is **Jonathan Maidment**. Everyone has a unique way of fundraising, and for Jonathan, it's through hiking. He has achieved the extraordinary feat of hiking the Triple Crown of Hiking—the Appalachian Trail (AT), Pacific Crest Trail (PCT), and Continental Divide Trail (CDT)—covering nearly 8,000 miles across 22 states and over one million feet of elevation gain. He also completed the Arizona Trail, a rugged 800-mile journey spanning deserts, mountains, and diverse landscapes. Through his adventures, Jonathan has raised over \$250,000 and carries the memory of his grandmother and the Alpha Angels, embodying the "Keep the Faith" mantra by John W. Walsh. His journey is a testament to both his physical endurance and compassionate spirit, inspiring countless others along the way.

There are several events that define the BFC program and have become cornerstones of AIF, offering Alphas, their families, friends, and their local networks moments to celebrate progress, and continue the search for a cure for Alpha-1.

One of AIF's annual events is **Escape to the Cape** held in Cape Cod, MA in proud partnership with the American Lung Association (ALA). **Joe Reidy**, Alpha-1 patient, past AIF Board of Director and

long-time BFC fundraiser shared, "Fred, Johnny, and I had been to about 25 or more Escape to the Cape events over the years. In the beginning, Fred and I rode many legs of the bike trek together. As time went on, that started to become more difficult for us, but we enjoyed helping in any way we could. Escape to the Cape is a great event for the Alpha-1 Community, and so much fun. I hope it continues for many, many years."



Another meaningful, and heartfelt annual BFC event is **The Celtic Connection**, held in Boston, MA. For 16 years, the Celtic Connection has gathered Alpha-1 supporters and friends for an Irish celebration centered around St. Patrick's Day, showcasing the strength and unity of the Alpha-1 community. "Together, with family and friends, we can make a difference—raising funds, increasing awareness, and sharing time with loved ones for a cause that brings hope. Every step we take brings us closer to a future where a cure is no longer a dream, but a reality," said **Bob Healy**, Alpha-1 patient and long-standing Chair of The Celtic Connection Planning Committee.



In 2020, amid the COVID-19 pandemic, AIF and the community pivoted to virtual fundraising, launching the **Riding for a Reason** initiative. An incredibly successful first year led to what has since become an annual companion fundraiser to Escape to the Cape held every September. By expanding the reach of the original bike trek, it allows people in the Alpha-1 community who are unable to join and ride in person to participate virtually as they ride in their own cities.

When circumstances made it difficult for the Alpha-1 community to meet in person, the **AIF Virtual Walks** ensured that the BFC goal continued uninterrupted. These walks even prior to the pandemic have allowed countless people

in the community the ability to participate in Alpha-1 awareness and fundraising, no matter where they are located. "This is my 4th year participating in the AIF Virtual Walk during Alpha-1 Awareness Month. It is so important to me that I share my knowledge about Alpha-1 and raise as much money as I can for the cause. Together, our community can and will make a difference. I am a mother to my 5-year-old daughter, Malia. I won't stop fighting until we have a cure for her and for all our Alpha-1 brothers and sisters", said **Kara Hodges**, Alpha-1 parent and BFC fundraiser.

The BFC program's impact is measured by the funds raised for Alpha-1 research and programs and in the deep and lasting connections it has built within the Alpha-1 community. **Anita Sellers**, BFC Fundraiser and Alpha-1 caregiver said, "Building Friends for a Cure will always hold a special place in my heart. BFC helps raise necessary dollars to fund research and the scientific breakthroughs helped provide many extra years with Keith that I'm forever

grateful for. Each year I am humbled by the generosity of family and friends helping to raise awareness and funds for research. My prayer is that one day a cure is found and the dollars raised through BFC will have helped. For me, it is all about the cure." Anita has raised over \$60,000 and inspired hundreds of people in memory of her late husband Keith.

For some, participating in the BFC program has been a life-changing experience. **Peg Iverson**, Alpha-1 patient, long-time volunteer, and now Vice Chair of the AIF Board of Directors, started a BFC fundraiser in 2008 called "Iowa Get the Scoop on Alpha-1 Walk" to help fund a cure for Alpha-1 and found herself deeply connected to the Alpha-1 community.

Peg shared, "Throughout the many years of my Alpha-1



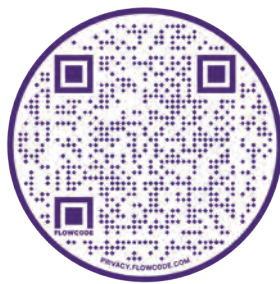
journey, the BFC program has provided me many opportunities to connect with other Alphas, not only locally in Iowa, but across the country. Every Alpha is empowered when they are actively involved in helping fund the cure for Alpha-1. I have tremendous gratitude to the Alpha-1 Foundation staff for teaching and helping us all connect in this very impactful and fun way!" Stories like Peg's highlight the personal and creative side of the BFC program and its ability to bring people together in meaningful ways.

As AIF celebrates 20 years of the BFC program, we're reminded of how far we've come—and how much there is still left to do. Whether you have been with us since the beginning or are just now learning about the BFC program, there's always a way to get involved. Join AIF at virtual and in person events, help fundraise for Alpha-1 and share your story. Together, we are making a difference, and together we will keep pushing forward towards a cure for Alpha-1.

Angela McBride, Senior Director of Corporate Relations and Strategic Alliances at AIF shared, "The BFC program has been an incredible initiative to oversee and a remarkable journey of growth and impact. Since its inception in 2005, this program

has become a cornerstone of Alpha-1 awareness and fundraising, touching lives across the country. As we celebrate two decades of BFC, we reflect on the program's achievements and look forward to continuing this vital work. Together, we are building not just friendships but hope and a brighter future for the Alpha-1 community."

To learn more about Building Friends for a Cure (BFC) and how you can be part of this incredible community, scan the QR code below or visit alpha1.org/building-friends-for-a-cure. Every effort, big or small, brings the Alpha-1 community closer to the day when a cure for Alpha-1 will be found. The BFC program isn't just about raising funds, it's about hope, connection, and the belief that by working together, we can change lives. 🙋



Patients at the Center of Research: The Alpha-1 Research Registry

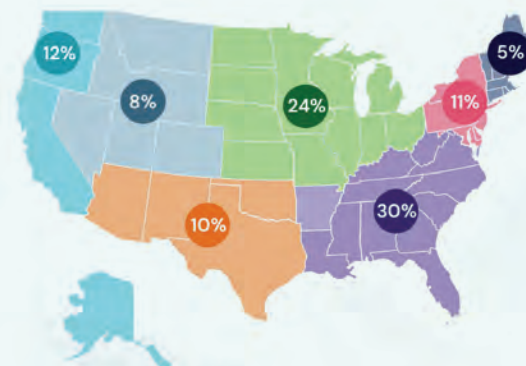


Did you know?

Over 38% of Alpha-1 Research Registry participants are affected by liver disease



Where do Alpha-1 Research Registry Participants live?



Patients from all 50 states and the District of Columbia are represented in the Alpha-1 Research Registry!

The Alpha-1 Foundation (A1F) has been supporting a patient registry since the mid 1990s. In 2019, A1F re-invigorated the Alpha-1 Research Registry by transferring it in-house to further enhance its capabilities. The Alpha-1 Research Registry is a confidential, comprehensive database of individuals with Alpha-1 Antitrypsin Deficiency (Alpha-1) and Alpha-1 carriers, designed to advance research, promote the development of improved treatments, and ultimately contribute to finding a cure for Alpha-1.

A1F created the Alpha-1 Research Registry to advance innovative Alpha-1 research, including investigations into improved

treatments and a cure for the condition. Located at A1F in Coral Gables, Florida, the Alpha-1 Research Registry employs procedures that ensure the highest level of confidentiality for participants.

Developed with the insights of Alpha-1 key opinion leaders, the Alpha-1 Research Registry operates under the direction of the A1F Board of Directors and is guided by an Advisory Committee composed of medical and scientific experts and patients.

One of the largest obstacles in Alpha-1 research is finding enough volunteers to participate in studies. Participation in research is voluntary, and Alpha-1

Research Registry participants are always given the option to accept or decline research invitations, allowing individuals to have control over their involvement. Participation in the Alpha-1 Research Registry enables researchers and patients to make a meaningful impact on studies that result in valuable data to get closer to a cure.

This comprehensive database, integrating both patient-contributed and clinical data, has shown powerful insight into the journeys of Alpha-1 patients and the impact Alpha-1 has had on their lives. The Alpha-1 Research Registry has emerged as an essential resource for the Alpha-1 community and a powerful tool

in advancing medical research as a central hub for gathering critical data and facilitating the connection between patients and researchers. It not only serves as a repository of valuable information but also as a dynamic platform that actively supports the research community, matching eligible individuals with appropriate clinical studies, thus further enhancing opportunities for treatment development.

The abundance of ongoing clinical trials investigating a diverse array of potential treatments for Alpha-1 is a credit to the efficacy of AIF's pipeline, bringing discoveries from benchtop to bedside. These efforts ensure a constant flow of new therapeutic options for Alpha-1 patients.

The Alpha-1 Research Registry plays a pivotal role in the Alpha-1 Biomarkers Consortium (AIBC) Study and serves as the patient clinical data repository for the Therapeutic Development Network (TDN). These initiatives aim to improve understanding of Alpha-1 and accelerate the discovery and development of new treatments. The Alpha-1 Research Registry is supporting new therapies and important research studies that will bring the Alpha-1 community closer to a cure.

Where Are We Now?

The Alpha-1 Registry currently has over 3,800 people with Alpha-1 enrolled. Patients tested for Alpha-1 largely due to lung symptoms (42%), family history (32%), liver symptoms (10%), and other reasons (8%); incidental findings on direct-to-consumer testing also accounted for a significant number of diagnoses (8%).

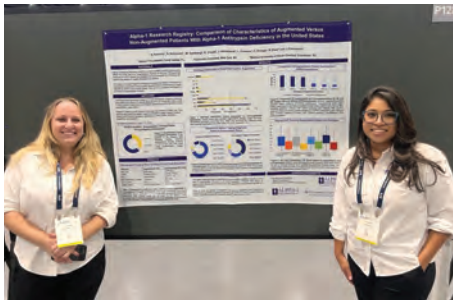
The Alpha-1 Research Registry team has continued to present at multiple international conferences, providing valuable insights into the lives of Alpha-1 patients for practitioners and researchers worldwide. The team has presented over 15 posters and presentations based on insights



garnered through the Alpha-1 Research Registry.

Most recently, the Alpha-1 Research Registry team presented for the first time on “Liver Profile and Diagnostic Disparities Among AATD in the US” at the American Association for the Study of Liver Diseases (AASLD) Annual Liver Meeting in San Diego, CA.

The Alpha-1 Research Registry highlights the diverse clinical manifestations associated with Alpha-1, helping researchers learn more about Alpha-1 and increasing awareness among



healthcare providers and the public. This ultimately improves the diagnosis rate of Alpha-1, which is essential for ensuring appropriate management and treatment.

Poster Presentations by the Alpha-1 Research Registry

2022

- American Thoracic Society (ATS)
- NORD Breakthrough Summit
- European Respiratory Society (ERS)

2023

- Rare Disease Day (RDD)
- American Thoracic Society (ATS)
- NORD Breakthrough Summit
- European Respiratory Society (ERS)
- The American College of Chest Physicians (CHEST)
- AATD Liver Meeting

2024

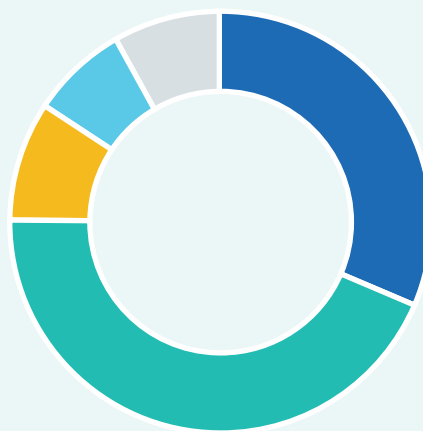
- Rare Disease Day (RDD)
- American Thoracic Society (ATS)
- AIF National Conference
- European Respiratory Society (ERS)
- The American College of Chest Physicians (CHEST)
- The Liver Meeting - American Association for the Study of Liver Disease (AASLD)

The Alpha-1 Research Registry recently reached an important milestone by successfully meeting a major enrollment goal for a clinical trial focused on patients with Alpha-1 emphysema. The results from this study have the potential to provide critical data that could increase the time between augmentation therapy treatments, significantly improving the quality of life for patients living with Alpha-1.

To date, the Alpha-1 Research Registry has assisted in recruiting participants for six research studies, ranging from simple surveys to more involved therapeutic clinical trials. These studies are helping to expand our understanding of Alpha-1 and drive the development of new treatments.

Participation in the Alpha-1 Research Registry is entirely voluntary, and individuals always have the option to accept or decline research invitations based on their personal preferences. If you're unsure whether you are part of the Alpha-1 Research Registry, you can easily check by using our Alpha-1 Research Registry Lookup Tool. [↑](#)

Reasons Patients Are Tested for Alpha-1



- Family Testing
- Lung Symptoms
- Liver Symptoms
- Direct-to-Consumer
- Other Reasons

Join the Alpha-1 Research Registry



We are recruiting for Alpha-1 related studies and need your participation!

We encourage you and your family members to join the Alpha-1 Research Registry to help advance Alpha-1 research, diagnosis, and treatment.

Anyone diagnosed with Alpha-1 can join the Registry in three steps:

1. Complete a Registration Form
2. Sign the Informed Consent
3. Complete the Questionnaire



To enroll or for more information:

alpha1.org/join-the-alpha-1-research-registry



Patient Recruitment Goals

- 270 Patients
- Recruited from patients who are in the Registry
- Followed over three years
- Can participate in trials during those three years
- Extensive clinical characterization of the patients

Investment in Alpha-1: Funding the AIF Mission

The Alpha-1 Foundation (AIF) finds itself at a pivotal, and potentially transformative, point in its 30-year history. The synergy of science, medicine, and technology, combined with AIF leadership over the last three decades has the foundation primed to deliver impact that will provide hope for all Alpha-1 Antitrypsin Deficiency (Alpha-1) patients and their families. As AIF celebrates its 30th anniversary, it invites the Alpha-1 community to join in reflecting on how far we have come and hope you will accompany AIF in committing to the next chapter of the journey.

On average, it takes nearly ten years and \$2.6 billion for a drug or therapy to reach market from its initial discovery, with clinical trials alone taking close to seven years. These numbers may increase substantially when applied to rare diseases. AIF and key Alpha-1 community stakeholders continue to forge ahead to ensure we fulfill co-founder John W. Walsh's vision: to find a cure for Alpha-1.

AIF's ability to make an impact depends on its donors. The tenacity and generosity of the entire Alpha-1 community drives our success. Thanks to the community's never-ending support, AIF is the largest funder of Alpha-1 research worldwide, proudly donating over \$100 million over the past three decades. AIF would not be where it is today without the generous support of AIF's donors and their dedication and belief in the mission. This investment has played a crucial role in advancing scientific discovery and advancements that now has the Alpha-1 community on the brink of breakthroughs that once seemed out of reach.

When an organization accepts donations to support their programs, it is essential that the non-profit serves as a trusted steward of donor dollars. At AIF, we have always embraced the importance of donors knowing that their contribution will be invested wisely and utilized to serve the mission. For AIF, transparency is a key factor that governs all operations. Recognized for good operating practices, accountability, and responsible governance, AIF has received the National Health Council's Full Compliance Certification and the Charity Seal from the Better Business Bureau's Wise Giving Alliance. These are the highest accreditations attainable in the not-for-profit and voluntary health sectors. AIF has also received an Exceptional 4-Star

rating by Charity Navigator, the highest rating given by that organization. GuideStar has also awarded the Platinum Seal of Transparency, their highest level of recognition as well.



The Alpha-1 Legacy Society

Planned giving allows donors to make significant contributions through estate planning – this may include bequests, charitable trusts, or beneficiary designations. The Alpha-1 Legacy Society brings together these donors who want to make an impact in the future of Alpha-1. The charitable goals of members are linked together by their desire to create a better world for those with Alpha-1 for generations to come. Members of this society are recognized for their commitment to the AIF mission and their willingness to secure its future through a planned gift. The John W. Walsh Founder's Circle is a tribute to the AIF co-founder who dedicated his life's work to finding a cure for Alpha-1. Members of the John W. Walsh Founder's Circle have named AIF as a primary beneficiary of planned gifts in the amount of \$50,000 or more.

Major Gifts

Major gifts to AIF are substantial contributions made by individual donors and families over the amount of \$5,000. These donations often fund large-scale initiatives, such as major Alpha-1 research projects and programs. Major donors frequently collaborate with AIF to direct their gift toward an area of particular interest, ensuring that their contribution creates a meaningful, targeted impact.

Annual Giving

While not every donation is a major gift, it is important to know that every dollar and every act of generosity fuels the shared vision of a healthier, better world for every Alpha. AIF is able to maintain and enhance essential programming that drives its mission because of these smaller, recurring donations that are the backbone of its work. AIF

salutes the individuals, corporations and foundations that share our commitment to finding a cure for Alpha-1 and to improving the lives of people affected by Alpha-1 worldwide. Impact of support resonates through every breakthrough we achieve and every life of every Alpha we touch.

Workplace Giving

There are many ways to get involved in workplace giving. Workplace giving is an easy, efficient way to make a charitable contribution to support AIF through matching gifts, payroll deduction, or volunteering.

Community Giving

The Building Friends for a Cure (BFC) program brings the Alpha-1 community together to help promote awareness, raise funds, and advocate for a better future for people living with Alpha-1. This volunteer-led program plays a huge role in raising revenue and allows participants to engage their network of friends and family to support Alpha-1 research and programming. To read more about the BFC program, turn to page 38.

Commemorative Giving

A commemorative gift to AIF is a meaningful way to pay tribute to a loved one who has passed away or to honor a special person in your life while funding a better future for people living with Alpha-1. These gifts may also be set up to recognize an event like a birthday, wedding, or anniversary. While honoring those we care about, we are also providing funding to move the mission forward.

Looking back with gratitude and ahead with determination, your donation supports critical research to discover new therapies and improve treatment for Alphas everywhere. Each gift fuels innovation and provides critical services and empowers AIF to tackle Alpha-1's biggest challenges. Giving to AIF is more than a financial transaction – it's an investment in a cause that has the power to transform lives, communities and the future of Alpha-1. Every gift advances research, care, advocacy, and education for people living with Alpha-1 related lung and liver disease. Together, we are fighting for a cure for Alpha-1. 📌



Using your Voice to Make an Impact: Alpha-1 Advocacy and Public Policy Through the Years



The Alpha-1 Foundation (A1F) has been at the forefront of advocacy and public policy for nearly three decades, working tirelessly to improve the lives of individuals affected by Alpha-1 Antitrypsin Deficiency (Alpha-1). Through strategic planning, grassroots advocacy, and collaborations with federal agencies, A1F has built a legacy of legislative and regulatory achievements that continue to generate positive outcomes for the Alpha-1 community.

Origins of Advocacy: A Journey of Determination

A1F's advocacy journey began with the groundbreaking American Lung Association (ALA) "Big Ride" that two Alphas, Shirley Dennis and Mary Pierce, biked from Washington State to Washington, D.C. Their mission culminated in a meeting with John W. Walsh (A1F co-founder), Miriam O'Day (public policy expert), and members of Congress, resulting in the inclusion of Alpha-1

language in an appropriations bill. This pivotal moment marked the start of a dedicated focus on addressing the needs of Alpha-1 patients at the national level.

Early efforts also laid the groundwork for Alpha-1 to be officially recognized as a rare disease, setting a Medicare payment precedent for infusion therapy. This advocacy milestone ensured that Alpha-1 patients could access necessary treatments under Medicare, creating a model for equitable healthcare access.

Current Achievements in Public Policy

AIF's efforts have consistently influenced legislative priorities. A cornerstone of its advocacy has been H.R. 4438: The John W. Walsh Alpha-1 Home Infusion Act, which aims to make home infusion therapy a permanent Medicare Part B benefit. While the 118th Congress did not finalize this legislation, it remains a priority for the 119th Congress, supported by over 60 co-sponsors and a robust grassroots advocacy network.

The introduction of the SOAR Act (the Supporting Oxygen Access Reform Act) to address the critical shortage of liquid oxygen and reform the Medicare payment system was another significant legislative effort inspired by Alpha-1 patients. This legislation aims to address the critical challenges faced by patients who require medical oxygen, including those with Alpha-1. The Act focuses on improving access to liquid oxygen and addressing systemic barriers in oxygen delivery, particularly for individuals who rely on home-based care. It emphasizes equitable and reliable access to oxygen as an essential component of managing chronic respiratory conditions.

AIF has been a voice for pediatric patients who have unmet needs.

The Alpha-1 Pediatric Liver Disease Registry represents a landmark achievement in the advocacy efforts of AIF, highlighting the importance of early detection and research into the pediatric manifestations of Alpha-1. The registry was originally established by the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) under the National Institutes of Health (NIH) following AIF's first successful Lobby Day and legislative collaboration.

This pivotal moment in AIF's history was accomplished through a strategic partnership with the National Organization for Rare Disorders (NORD) and bipartisan support in Congress. The legislative victory emphasized the importance of understanding Alpha-1's liver-related complications in children, a critical aspect of the condition.

The result was the establishment of a national resource to:

- 1. Collect Data:** Gather comprehensive clinical and genetic information about Alpha-1-related liver disease in children.
- 2. Guide Research:** Provide a robust foundation for scientific studies, enabling a better understanding of the progression and management of pediatric liver disease caused by Alpha-1.
- 3. Promote Therapeutic Development:** Support the development of targeted therapies and interventions for children impacted by this rare disease.

The Alpha-1 Pediatric Liver Disease Registry remains a vital tool for advancing knowledge, supporting



families, and influencing policy decisions related to Alpha-1. It underscores AIF's commitment to fostering collaborative advocacy, promoting early detection, and driving research that improves outcomes for individuals affected by Alpha-1 from infancy through adulthood.

Advancing Research and Regulatory Engagement

The Alpha-1 Foundation has built strong partnerships with key federal agencies, including the NIH and Federal Drug Administration (FDA). These collaborations have led to significant advancements in research and regulatory processes:

- The NIH's Learn More, Breathe Better campaign, launched with the AIF's support, brought national attention to the genetic underpinnings of Chronic Obstructive Pulmonary Disease (COPD)
- The creation of an in-house Alpha-1 Research Registry enabled critical data collection, driving therapeutic development
- FDA initiatives, such as Alpha-1-focused meetings on biomarkers and endpoints, have streamlined clinical trial processes and paved the way for novel therapies
- AIF's engagement with the Critical Path Institute further advances regulatory science by working towards establishing endpoints and biomarkers critical for Alpha-1 therapeutic development

Community Empowerment and Awareness

AIF has excelled in empowering patients and caregivers to become effective advocates. Initiatives such as Grassroots Advocacy Training and an Annual Advocacy Day provide tools for direct engagement with policymakers. Media campaigns and public outreach efforts have amplified awareness of Alpha-1, its impact, and the importance of equitable care.

Strategic Plan for the 119th Congress: "Generating Change to Improve Outcomes"

As AIF prepares for the challenges ahead, it has outlined a bold strategic plan for advocacy and public policy:

Mission Goals:

1. Access to Care: Address Medicare reform to ensure home infusion therapy access.
2. Medical Research: Advocate for increased federal funding for Alpha-1 therapeutic development.
3. Education and Awareness: Expand public and professional understanding of Alpha-1.

4. Screening and Detection: Promote nationwide screening initiatives.
5. Regulatory Advocacy: Strengthen FDA and NIH partnerships to streamline treatment approvals. Reauthorization of the Rare Pediatric Review Voucher Program.

Key Legislative Priorities:

- Passing the John W. Walsh Alpha-1 Home Infusion Act and the Supporting Oxygen Access Reform Act
- Expanding telehealth coverage for Alpha-1 patients so they can access virtual telehealth pulmonary rehabilitation
- Advocating for increased NIH funding.

Regulatory Objectives:

- Strengthening FDA collaborations to advance Alpha-1 therapies
- Enhancing the Alpha-1 Research Registry to support therapeutic innovation

Legacy and Future Impact

AIF's advocacy achievements are a testament to the vision of its founders, in particular John W. Walsh, and the dedication of its community. Through initiatives such as the national COPD Action Plan, partnerships with NIH and FDA, and grassroots advocacy, AIF has consistently bridged gaps between rare disease advocacy and broader public health priorities.

As the 119th Congress approaches, AIF remains steadfast in its mission to generate change and improve outcomes for Alpha-1 patients. By leveraging strategic partnerships, legislative advocacy, and community empowerment, it continues to pave the way for a brighter future for those affected by Alpha-1. 📌





The #1 prescribed alpha-1 therapy for more than 30 years¹

- **15-minute** infusion time
- **Proven** to effectively raise the alpha₁ antitrypsin protein levels in patients with severe alpha₁-antitrypsin deficiency, also known as alpha-1
- The **PROLASTIN DIRECT**[®] program gives you the confidence that ongoing personalized support is there for you when needed

Reference: 1. Data on file, Executive Summary, Grifols.



Visit our website, www.prolastin.com/LIQUID, for more information and patient-friendly resources.

Important Safety Information

PROLASTIN[®]-C LIQUID is an alpha₁-proteinase inhibitor (human) (alpha₁-PI) indicated for chronic augmentation and maintenance therapy in adults with clinical evidence of emphysema due to severe hereditary deficiency of alpha₁-PI (alpha₁-antitrypsin deficiency).

Limitations of Use

- The effect of augmentation therapy with any alpha₁-PI, including PROLASTIN-C LIQUID, on pulmonary exacerbations and on the progression of emphysema in alpha₁-PI deficiency has not been conclusively demonstrated in randomized, controlled clinical trials
- Clinical data demonstrating the long-term effects of chronic augmentation or maintenance therapy with PROLASTIN-C LIQUID are not available
- PROLASTIN-C LIQUID is not indicated as therapy for lung disease in patients in whom severe alpha₁-PI deficiency has not been established

PROLASTIN-C LIQUID is contraindicated in immunoglobulin A (IgA)-deficient patients with antibodies against IgA or patients with a history of anaphylaxis or other severe systemic reaction to alpha₁-PI products.

Hypersensitivity reactions, including anaphylaxis, may occur. Monitor vital signs and observe the patient carefully throughout the infusion. If hypersensitivity symptoms occur, promptly stop PROLASTIN-C LIQUID infusion and begin appropriate therapy.

Because PROLASTIN-C LIQUID is made from human plasma, it may carry a risk of transmitting infectious agents, eg, viruses, the variant Creutzfeldt-Jakob disease (vCJD) agent, and, theoretically, the Creutzfeldt-Jakob disease (CJD) agent. This also applies to unknown or emerging viruses and other pathogens.

The most common adverse reactions during PROLASTIN-C LIQUID clinical trials in >5% of subjects were diarrhea and fatigue, each of which occurred in 2 subjects (6%).

Please see brief summary of the full Prescribing Information for PROLASTIN-C LIQUID on adjacent page.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088.

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August 2024

US-PRL-2400148

PROLASTIN[®]-C LIQUID

Alpha₁-Proteinase Inhibitor (Human)

HIGHLIGHTS OF PRESCRIBING INFORMATION

These highlights do not include all the information needed to use PROLASTIN-C LIQUID safely and effectively. See full prescribing information for PROLASTIN-C LIQUID.

PROLASTIN-C LIQUID
(Alpha₁-Proteinase Inhibitor [Human])
Solution for Intravenous Injection

Initial U.S. Approval: 1987

INDICATIONS AND USAGE

PROLASTIN[®]-C LIQUID is an Alpha₁-Proteinase Inhibitor (Human) (Alpha₁-PI) indicated for chronic augmentation and maintenance therapy in adults with clinical evidence of emphysema due to severe hereditary deficiency of Alpha₁-PI (alpha₁-antitrypsin deficiency).

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- Clinical data demonstrating the long-term effects of chronic augmentation or maintenance therapy with PROLASTIN-C LIQUID are not available.
- PROLASTIN-C LIQUID is not indicated as therapy for lung disease in patients in whom severe Alpha₁-PI deficiency has not been established.

DOSAGE AND ADMINISTRATION

For intravenous use only.

- Dose: 60 mg/kg body weight intravenously once per week.
- Dose ranging studies using efficacy endpoints have not been performed with any Alpha₁-PI product, including PROLASTIN-C LIQUID.
- Administration: 0.08 mL/kg/min as determined by patient response and comfort.

DOSAGE FORMS AND STRENGTHS

For injection: approximately 500 mg (10 mL), 1,000 mg (20 mL) and 4,000 mg (80 mL) of a solution for injection in single-dose vials.

CONTRAINDICATIONS

- Immunoglobulin A (IgA) deficient patients with antibodies against IgA.
- History of anaphylaxis or other severe systemic reaction to Alpha₁-PI.

WARNINGS AND PRECAUTIONS

- Severe hypersensitivity and anaphylactic reactions may occur in IgA deficient patients with antibodies against IgA. Discontinue administration of the product and initiate appropriate emergency treatment if hypersensitivity reactions occur.
- Because PROLASTIN-C LIQUID is made from human plasma, it may carry a risk of transmitting infectious agents, e.g., viruses, the variant Creutzfeldt-Jakob disease (vCJD) agent, and, theoretically, the Creutzfeldt-Jakob disease (CJD) agent.

ADVERSE REACTIONS

The most common adverse reactions during PROLASTIN-C LIQUID clinical trials in > 5% of subjects were diarrhea and fatigue, each of which occurred in 2 subjects (6%).

To report SUSPECTED ADVERSE REACTIONS, contact Grifols Therapeutics LLC at 1-800-520-2807 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

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Investing in Cutting-Edge Research to Find a Cure: Venture Philanthropy



The Alpha-1 Project (TAP), created by the Alpha-1 Foundation (A1F) Board of Directors, served as a platform for engaging with biotech and pharmaceutical companies with, or those considering the development of, therapies and treatments for Alpha-1 Antitrypsin Deficiency (Alpha-1).

In 2009, Dr. Gordon and Ruth Cadwgan generously donated

\$1 million to A1F for this venture philanthropy project on behalf of his family. Gordon, a member of the A1F Board of Directors at the time and former Chair, believed this impactful gift would help energize the community, and patients, academia, pharmaceutical and biotech companies, along with public health organizations, would continue the relentless pursuit of

cures and therapies for lung and liver diseases caused by Alpha-1.

Like venture philanthropy models created by similar cure-focused non-profits, TAP committed to accelerating progress through partnership investment, an essential need in rare conditions as limited incidence rates often require enticement to the field.

In addition, the translation of discoveries into therapies can often be stifled by the substantial financial hurdles associated with seeking regulatory approvals. AIF's venture philanthropy program enabled direct investment into promising treatments providing healthcare stakeholders opportunities to partner with biotech and pharmaceutical companies to develop new therapies.

Finding meaningful endpoints, how to test them and how to prove a potential therapy works are also tough obstacles to overcome in therapy development. The goal is to accelerate the discovery, development and commercialization of cures, treatments, and drug therapies for those with Alpha-1.

When TAP was established in 2009:

- Researchers had already unraveled most of the biochemical pathways from the deficient alpha-1 antitrypsin (AAT) gene that lead to disease, so there is an abundance of science to share with biotech companies
- A DNA and Tissue Repository of samples from Alphas and non-Alphas existed for scientists to use in their research—as well as the Alpha-1 Research Registry
- Biotech firms were—and are—in the business of producing biologically targeted drugs. Targeted drugs are especially suited to treating a single-gene defect such as Alpha-1

TAP invested directly in carefully selected and peer-reviewed new therapies that had potential to become a cure for Alpha-1 while also supporting therapies that improve the quality of life for Alphas until a cure is found. TAP aimed to bridge the gap between development, clinical trials, and the launch of new therapies or cures for Alpha-1.



Beyond being affected by Alpha-1, Gordon understands the importance of scientific integrity and peer review, and that makes him especially effective in his leadership role.

—John W. Walsh,
AIF co-founder



Accelerating the discovery, development and commercialization of cures, treatments and drug therapies has always been at the core of AIF's mission and remains a top priority. With recent gene therapy and gene editing technologies advancing in utility and additional pharmaceutical companies recognizing the Alpha-1 treatment and therapies as a viable marketplace. Today, it continues in concept under the direction of the AIF Board of Directors Executive Committee, including Ken Irvine, with guidance and insight through scientific advisors and stakeholders.

On the Horizon:

- Gene Therapy, Gene Editing and Stem Cell Therapies
- Small Molecule chaperones and modulation
- Antiproteases and Anti-inflammatories
- Devices for detection, monitoring, and treatment of Alpha-1

Since 2009, investment into basic science has led to the creation of an extensive library of publications. Supporting such research has also fostered collaborations and the generation of experts committed to discovering improved treatments. The abundance of research has produced actionable pathways and

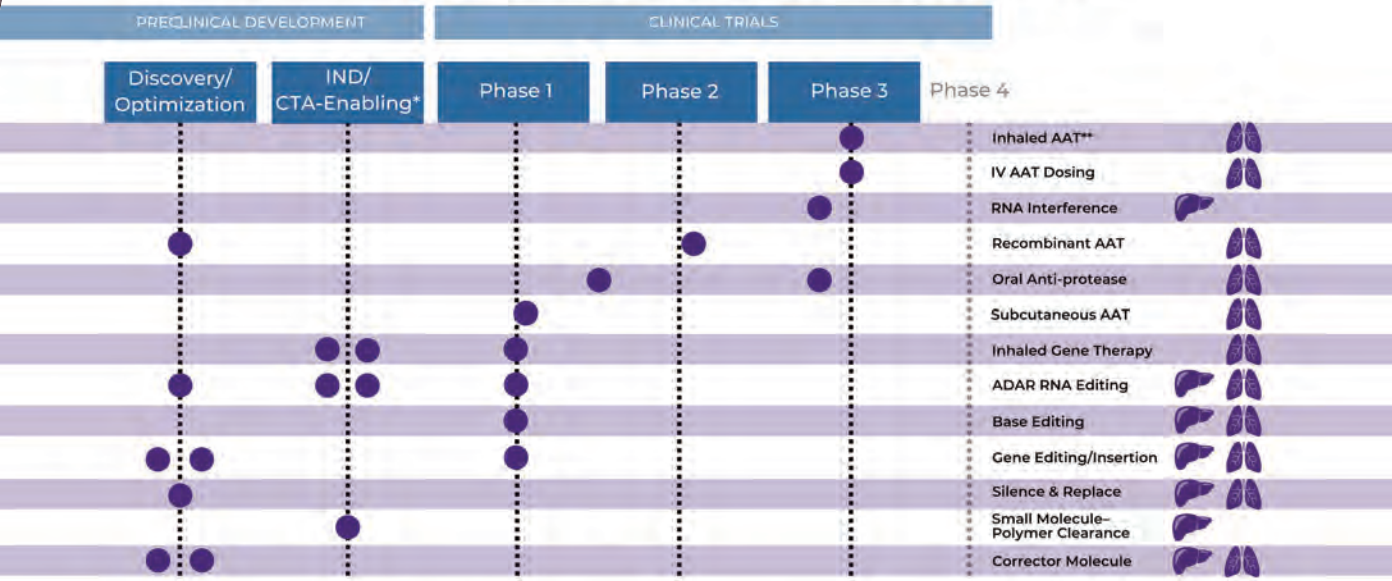
opportunities for intervention.

AIF provides access to our resources including the Alpha-1 Research Registry, DNA & Tissue Bank, key opinion leaders, and research tools helping industry accelerate drug development. AIF is committed to advancing the next Alpha-1 breakthrough by directly funding translational research in the drug development process. The research funded by AIF to date has improved the understanding of the mechanisms underlying the clinical manifestations of Alpha-1 and identified novel drug targets likely to lead to new therapeutic solutions. 📌





Potential Alpha-1 Therapeutics in Development



*Investigational new drug (IND) and clinical trial application (CTA)
 **alpha-1 antitrypsin (AAT)

Information updated as of October 1, 2024
 Chart is not to time scale
 As recorded in ClinicalTrials.gov or Clinicaltrialsregistry.eu



30 Years of Improving the Lives of Alphas and Their Families



For three decades, the Alpha-1 Foundation (A1F) mission has been centered around improving the lives of people affected by Alpha-1 Antitrypsin Deficiency (Alpha-1). Along with the support of everyone in the Alpha-1 community, A1F has built a community rooted in hope, connection, and meaningful impact.



The community is truly worldwide – people who come together and can share, encourage one another, and no longer feel alone. The Alpha-1 Foundation was the starting point, and without it, we wouldn't be where we are today.

—Patty Tew, Alpha-1 patient and
A1F committee member



Each A1F program is designed with you, the Alpha-1 community, in mind to address the unique needs you face every day. Whether it's through Alpha-1 Support Groups that meet in person and virtually to bring together Alphas and their families, or the Alpha-1 Peer Guide Program that connects you with others who truly understand your unique journey and have had similar experiences, or through educational initiatives that empower you with knowledge about your condition and care options, A1F is here to walk this path with you.

Patient Education

A1F Education Days for Alphas, their families, and caregivers are held six times per year in different regions of the United States. These one-day educational programs provide access to current medical information, topics affecting Alphas, resources from leading Alpha-1 experts, and opportunities to connect with the Alpha-1 community. A1F Education Days serve over 1,000 patients and their families each year.

The largest annual gathering of the Alpha-1 community, the A1F National Conference, provides important opportunities for networking, education, advocacy, and industry publicity. Over the course of three days, attendees have access to over 25 scientific and educational presentations, meet-and-greets and Q&A sessions with Alpha-1 experts, and networking opportunities with other participants. The first Alpha-1 National Conference took place in 1992 in Minneapolis, MN. Participation has grown exponentially through the years with the largest conference taking place in Chicago, IL in 2017 with almost 700 attendees. In 2024,



for the first time, A1F provided 100 scholarships to Alpha-1 patients who had not previously attended a conference in person due to financial constraints and wanted to attend to learn about their condition and meet others living with Alpha-1.



It's so important, to meet others that have been through what you are going through and see that there is life after this diagnosis.

—Jon Hagstrom, Alpha-1 patient, and Chair of the A1F Board of Directors



Amid the COVID-19 pandemic in 2020, A1F pivoted to virtual events, building out a fully customized Virtual Environment (VE) with access to live presentations and Alpha-1 resources. A1F continues to use the virtual event platform, enabling those who are unable to attend A1F education events in person to participate virtually, gain access to Alpha-1 information and resources, and connect with the Alpha-1 community. A1F ensures that both in-person and virtual participants have access to the educational presentations, which are live-streamed and later made available for on-demand viewing on the A1F website. This hybrid approach exemplifies

A1F's commitment to inclusivity and accessibility, ensuring everyone can benefit from knowledge shared during educational events.

Educational resources, available on alpha1.org, such as the Patient Education Programs, A1F Resource Library, and A1F Video Library provide tools to make informed decisions about your care.

The A1F Video Library has grown to over 400 videos presented by Alpha-1 experts and community members on topics including Advocacy, Caregiving, Children & Alpha-1, Genetics, Getting Involved, Living with Alpha-1 Lung & Liver, Nutrition, Oxygen, Pulmonary Rehab & Exercise, Research, and Transplantation.

Alpha-1 Testing, Care, and Specialists

The Alpha-1 Coded Testing (ACT) study provides free, confidential genetic testing for people at risk for Alpha-1 and family members. To learn more about detection efforts, turn to page 74 to read "Commitment to Finding All Alphas: Insight Into Awareness and Detection"

Free Genetic Counseling Services are available to Alphas, their family members, healthcare providers, and other individuals considering testing or in need of support to help navigate testing for Alpha-1 and what those test results mean for you and your family. Genetic counseling services help patients

understand their genetic risk for developing lung and liver disease, as well as communicating that information to family members.

Alpha-1 Specialists at A1F Clinical Resource Centers (CRCs) play a pivotal role in delivering comprehensive care to Alphas, offering specialized treatment for lung and liver diseases. These CRCs serve as the cornerstone of the patient care network, ensuring that individuals receive the highest quality of care tailored to their specific needs. Beyond direct patient care, CRCs are instrumental in advancing A1F's mission by facilitating patient recruitment for the Alpha-1 Research Registry. This collaboration is crucial for expanding the Alpha-1 Research Registry, which in turn accelerates research and the development of new therapies.



Patient Support

The Patient Information Line, managed by A1F and answered by a longstanding A1F staff member, is the first introduction many people have to the Alpha-1 community and provides real answers to their questions about Alpha-1. It is an invaluable resource for Alphas and their family members when first diagnosed, and throughout their journey with Alpha-1. The Patient Information Line receives over 700 calls each year on topics surrounding Alpha-1 testing, finding an Alpha-1 specialist, general Alpha-1 education, resources and more.

The Alpha-1 Support Group Network brings people together locally around the country to raise awareness of Alpha-1, provide education and resources, support families, and tackle the issues that matter most to Alphas. Alpha-1 Support Group Leaders provide education and support for Alpha-1 families in their local communities.

The Peer Guide Program connects newly diagnosed Alphas, family members, parents of Alpha-1 children, transplant patients and others with specific experiences with knowledgeable peers who have had similar journeys, offering encouragement, practical advice, and a listening ear.

"It's been rewarding to be able to share my experiences and experiences of others I've known, to put a perspective on it that if you are going to have a condition, this is not a bad one to have in the sense that a lot is known and it can be very manageable," said Steve Shurtz, Alpha-1 patient and A1F Support Group Leader.

Programs like the Oxygen Travel Assistance Program help ease financial burdens of those requiring supplemental oxygen to travel for medical appointments and education events, while the CRC Access Program provides



resources for Alpha-1 patients to visit an Alpha-1 specialist for the first time.

Alpha-1 Advocacy

Through countless Alpha-1 advocacy efforts, A1F has fought for better policies, funding, and access to therapies and research. To learn more, turn to page 48 to read “Using your Voice to Make an Impact: Alpha-1 Advocacy and Public Policy Through the Years”

Alpha-1 Research

A1F’s commitment to Alpha-1 research is unwavering. It has invested over \$100 million in research and programs across 130+ institutions globally. Resources like the Alpha-1 Research Registry connect Alphas who want to participate in research with groundbreaking clinical research studies. To learn more, turn to page 66 to read “A1F: Leading Alpha-1 Research Worldwide Toward a Cure”

The real impact of these efforts? It’s reflected in the stories you share with A1F every day:

- Alpha-1 patients gaining access to life-saving therapies
- Families finding support and strength in the Alpha-1 community
- Individuals thriving despite their often scary and overwhelming diagnosis

These testimonials show the power of what can be achieved together.

“You are not alone. You have access to the Alpha-1 Foundation, you have access to peer support, peer guides, and mentors. All the help that you could wish for is there, and we are one big family,” says Peg Iverson, Alpha-1 patient and Vice Chair of the A1F Board of Directors.

As A1F celebrates 30 years of progress, we’re filled with excitement about the future. From new initiatives to expanded programs and services, the journey ahead is bright. A1F remains committed to continue to build a stronger, more supportive Alpha-1 community and create brighter futures for all who are affected by Alpha-1. 📌



JOIN US IN CELEBRATING OUR
30TH ANNIVERSARY AT THE

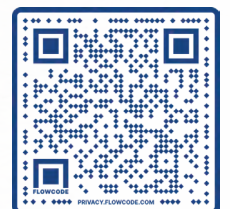
2025

ALPHA-1 FOUNDATION NATIONAL CONFERENCE

MIAMI, FL

JUNE 6-8, 2025

#A1FNC25



Advancements in Alpha-1 Liver



The Alpha-1 Foundation (A1F) continues to invest boldly in liver research, funding the most promising science to accelerate treatments for the underlying causes of Alpha-1 Antitrypsin Deficiency (Alpha-1), and to find a cure for every person diagnosed with this condition. For the first time since this condition was discovered, there are now numerous potential therapeutics in the pipeline and one or more of these novel treatments will prove beneficial in upcoming clinical trials.

A1F is working on many fronts to facilitate the success of these trials to realize their potential benefit for Alphas. These new therapeutics, which have been under development for years, are the result of insights and breakthroughs made by researchers and studies funded by A1F.

Alpha-1 liver disease is the accumulation of misfolded alpha-1 antitrypsin (AAT) and polymers inside the liver cells. Symptoms occur at a stage late in the course of disease progression, so until the liver disease is relatively advanced, a person usually won't be able to assess how well their liver is functioning without a test at the doctor's office. It is critical to focus on research and therapies for the Alpha-1 liver affected community.



The Alpha-1 Foundation has set the precedent that the liver is important. After years of really starting to understand what lung disease is and how it impacts patients, a lot of the new therapies are aimed at treating liver disease. One way to answer the right question which is who should be treated and how do we treat them, is going to be answered by one of the studies that the Foundation has supported—it's a large natural history study that enrolled individuals with cirrhosis around the country who have Alpha-1, who are impacted. Without the Alpha-1 Foundation that study would not have been completed. We have seen a lot of data come out from that study that has helped guide and direct what the next steps are for both patient care and for research.

—Virginia Clark, MD, Physician Director
for the A1F Board of Directors





A variety of other approaches to therapy or prevention of Alpha-1 liver and lung disease have been proposed and, in some cases, are being investigated. This includes the use of generic and specific chemical chaperones, mechanism-based pharmacological strategies, cell transplantation therapies, and gene transfer techniques that can repair the mutant gene.

Gene Therapy

The Z mutation is a single point mutation, so it would be correcting this single gene by getting into the nucleus of 150 trillion cells that make up a normal liver. Or possibly, correcting a small portion of the liver cells and the corrected cells gradually take over the liver as they divide more effectively than the previous liver cells.

- Correction of DNA: Attempting to induce correctly coding RNA
- Improve folding of the misfolded AAT protein: If folding can be adequately corrected with a pill, then the Alpha-1 protein could get out of the liver cell and into the bloodstream. This could lead to a

liver that heals from having less misfolded protein and polymers and normalized blood levels

- Target the pathways that degrade misfolded proteins: Expediting these pathways could reduce the toxicity of misfolded proteins to the cell

Advancements in Alpha-1 Liver Studies

5-year Adult Liver Study

Funded by AIF, the five-year study seeks to uncover the national history (the course of disease if left untreated) of Alpha-1 liver disease in adults. Jeffrey Teckman, MD, professor of pediatrics and biochemistry and molecular biology at Saint Louis University, was the principal investigator in the study.

Childhood Liver Disease Research Network (ChiLDReN)

A first-in-class tool to predict future risk of portal hypertension, liver transplant and death in children with Alpha-, based on prospective data from the Childhood Liver Disease Research Network (ChiLDReN).



For many years we have known that most kids with ZZ Alpha-1 do fine, but a few get very sick and are even at risk of needing a lifesaving liver transplant. Previously, we did not have a way to predict which kids might be at high risk of severe disease. Now, for the first time, thanks to the Alpha-1 Foundation's support of the nationwide Childhood Liver Disease Research Network, we have developed the first tool to predict kids at high risk of severe disease. The work continues and a second generation, even more powerful tool, is now under development. Thanks to the Foundation, the patients and families who participated in the study and the National Institutes of Health (NIH) for enabling this first if its kind discovery.

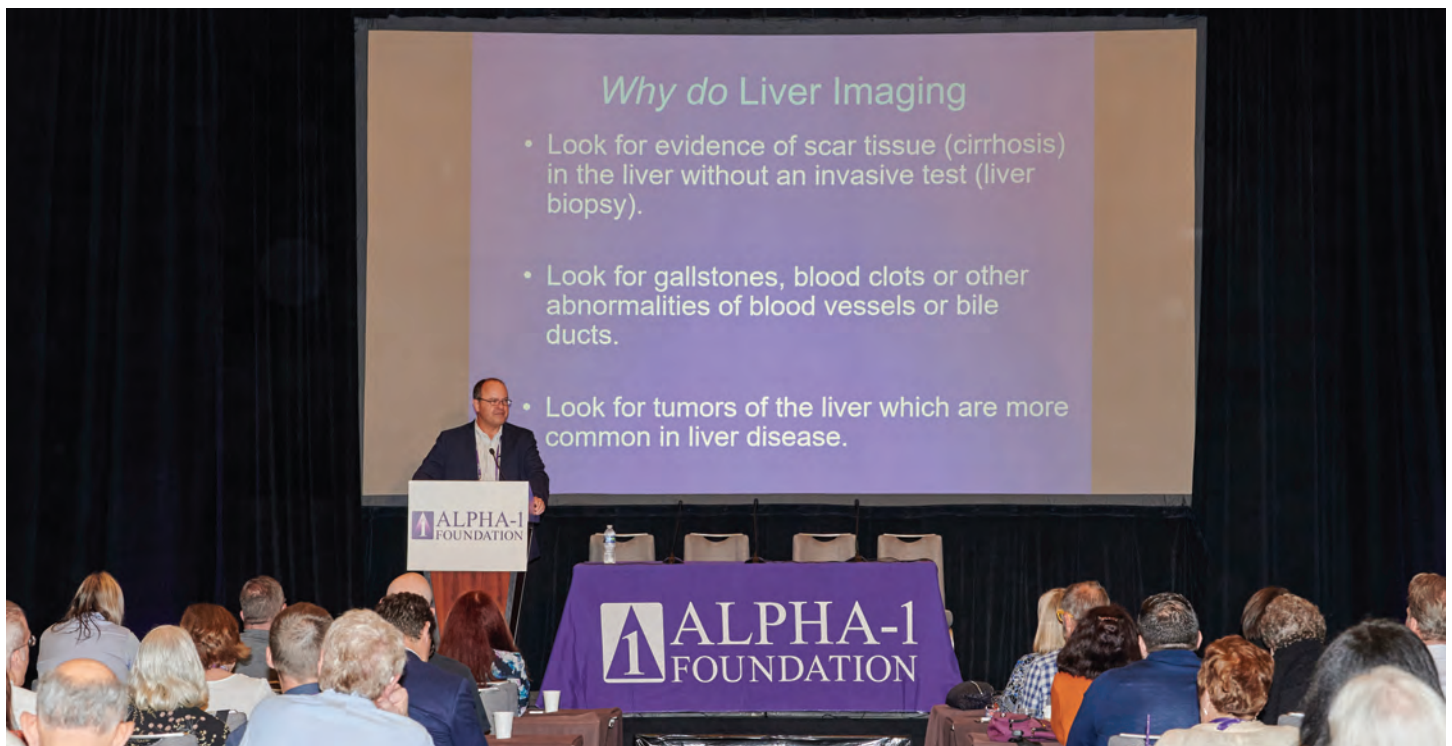
—Jeffrey Teckman, MD



ChiLDReN, a National Institutes of Health (NIH)-supported consortium of pediatric tertiary care centers across North America, focused on studying pediatric liver diseases, including Alpha-1. Researchers have identified biomarkers in high-risk infants with Alpha-1, a condition that can be life-threatening in some infants but very mild in others. For the first time, predicting which children will need lifesaving intervention is now possible. This data will enable the identification of high-risk infants soon after diagnosis, allowing for timely intervention.

Seventeen consortium centers gathered data throughout the study period, focusing on pediatric liver diseases. The aim was to describe the natural history and genetic and environmental modifiers of various metabolic-cholestatic liver diseases, including Alpha-1. Researchers measured ZZ AAT serum polymer levels from prospectively collected samples in the Longitudinal Observational Study of Genetic Causes of Intrahepatic Cholestasis (LOGIC) and compared these outcomes in the longitudinal database.





Why do Liver Imaging

- Look for evidence of scar tissue (cirrhosis) in the liver without an invasive test (liver biopsy).
- Look for gallstones, blood clots or other abnormalities of blood vessels or bile ducts.
- Look for tumors of the liver which are more common in liver disease.

Scientists have made significant progress in developing new therapies for Alpha-1 in recent years, though none are yet approved. While many adult clinical trials enroll participants, none study infants or children. The newly identified biomarkers in infants will stimulate and accelerate future pediatric clinical trials associated with high-risk infants. “Enrollment of children into trials can then be focused on this high-risk group, sparing many participants from needless trials and speeding the effective treatments to proof of efficacy and approval,” said Teckman.

The LOGIC Study

The LOGIC study is a natural history study that began in 2007 that includes patients with Alpha-1 and three other rare liver diseases. The study is aimed at acquiring information and data that will provide a better understanding of rare conditions. Participants are asked to allow study personnel to obtain information from medical records and an interview and to collect blood, urine, and tissue samples when clinically indicated, to understand the causes of these diseases and to improve the diagnosis and treatment of children with these diseases.


Fazirsiran’s Potential in Alpha-1 Liver Disease

New data from the open-label extension of Fazirsiran has recently been presented at The Liver Meeting 2024 from the American Association for the Study of Liver Diseases (AASLD) by Virginia Clark, MD, AIF Board Member, Professor of Medicine and Program Director of the Gastroenterology fellowship in the division of gastroenterology, hepatology, and nutrition at the University of Florida.

With transplantation as the only choice for patients who develop more severe complications from Alpha-1 liver disease, clinical trials are critical to advance research to bring new therapies to market. It is important to understand the presence of fibrosis and cirrhosis to determine the next steps in disease management.

“These new therapeutic agents may have the potential to reduce the burden of Alpha-1 within the liver, and that may change the trajectory of what their disease does over time,” Dr. Clark explained.

It is so important that patients get correctly diagnosed and detected early in their disease progression to help modify their risk factors including alcohol intake, healthy weight and other metabolic factors. One-year findings showed treatment with Fazirsiran resulted in sustained, dose-dependent reductions in Z-AAT concentration and improved historical findings.

Thank you to all Alphas who participate in research studies that bring the Alpha-1 community closer to finding a cure. Patients are crucial for clinical trials as their participation helps researchers evaluate the safety and efficacy of new treatments leading to advancements in medical science and potentially breakthroughs in disease management for Alpha-1. By joining the Alpha-1 Research Registry, you will learn first-hand about clinical trials you may qualify for and other clinical research opportunities. It is crucial that the Alpha-1 community continues to join the Alpha-1 Research Registry and participate in clinical research trials. 

ARE YOU ON A JOURNEY WITH ALPHA-1 LIVER DISEASE?



The Redwood Study is looking for adults 18 to 75 years of age who have a confirmed or suspected diagnosis of Alpha-1 Liver Disease with PiZZ genotype. There is currently no approved treatment available for Alpha-1 Liver Disease. The investigational study drug aims to reduce the production of the abnormal Z-AAT protein and its buildup in the liver. To learn more and see if you may qualify, talk to your doctor, visit a1f.org/redwood-study-prescreening, or scan the QR code today.

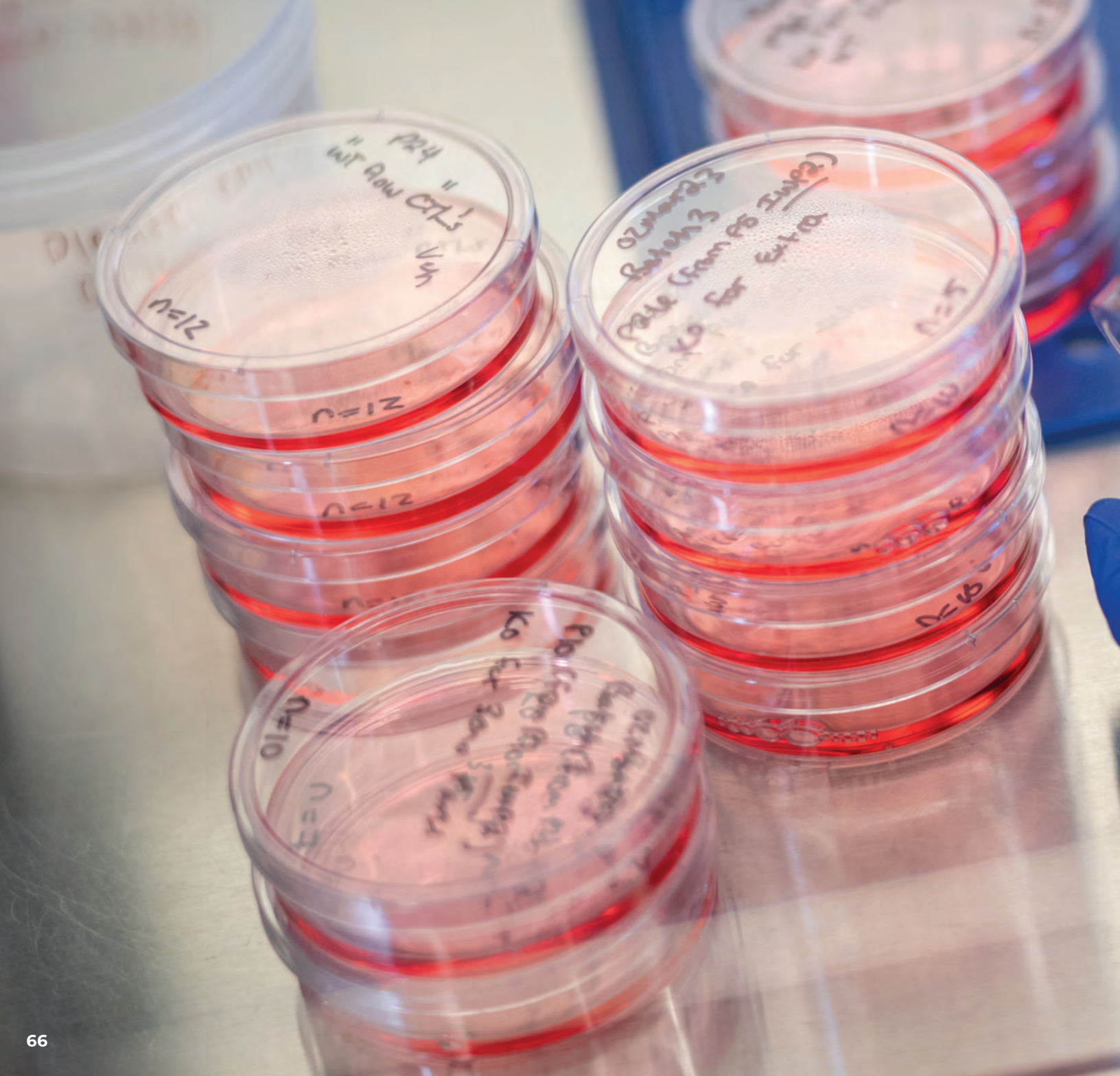


www.alpha1.org • 1-877-2-CURE-A1 (228-7321)



**The
Redwood
Study**

A1F: Leading Alpha-1 Research Worldwide Toward a Cure



The Alpha-1 Foundation (A1F) is the world's #1 funder of Alpha-1 research, investing more than \$100 million since the Grants Award Program's inception in 1999. A1F funding has supported research and programs at over 130 institutions in North America, Europe, the Middle East, and Australia, with a wide range of studies addressing Alpha-1 lung and liver conditions, along with legal and social issues that are especially important in a genetic condition such as Alpha-1.



The Alpha-1 Foundation funds research all over the world and is the leading funder of Alpha-1 research. It brings researchers together, which makes people feel part of a family of researchers with a single goal – the cure of Alpha-1 Antitrypsin Deficiency (Alpha-1).

—Prof. Gerry McElvaney,
Alpha-1 researcher and
past A1F Board Member



A1F has designed and launched innovative programs and developed a solid infrastructure to promote research and the development of new therapies. It has fostered collaborations with investigators, working closely with the National Institutes of Health (NIH), the U.S. Food and Drug Administration (FDA), the pharmaceutical industry and Alpha-1 patients to expedite the development of improved therapies.

The A1F Grants Award Program supports scientific research to advance Alpha-1 treatment and care with a focus on expediting the discovery of a cure. “The Alpha-1 Foundation models

its grant review process on the rigorous approach applied by the NIH to ensure we fund the highest quality science based on thorough assessment by our expert review panel,” says Andrew Wilson, MD, A1F Scientific Director.

The A1F research agenda is periodically reviewed by the Medical and Scientific Advisory Committee (MASAC) Members to ensure it continues to serve the changing needs of the community and advance the mission to find a cure for Alpha-1.

As part of A1F's commitment to improving the lives of individuals diagnosed , A1F established the Alpha-1 Research Registry, a confidential database made up of individuals diagnosed with Alpha-1. The Alpha-1 Research Registry was established to facilitate research initiatives, promote the development of improved treatments, and ultimately find a cure for Alpha-1. Individuals enrolled can participate directly in clinical trials of new therapeutic approaches in addition to other research opportunities. One outstanding example of the Alpha-1 Research Registry being utilized is in the Alpha-1 Biomarker Consortium (A1BC) where over 270 patients were successfully enrolled.

“This grant was only possible due to the asset of the Alpha-1 Research Registry built by the Foundation. The researchers and the Foundation together are in an ideal situation to ensure the success of this grant. We have formed an outstanding network of dedicated researchers committed to partnering with A1F and have established a strong medical and scientific core. We look forward to advancing the science of Alpha-1 and answering important questions for both the scientific and patient community,” explained Jeanine D'Armiento,

MD, PhD, Principal Investigator, AIF Medical Liaison, and immediate past Chair of the AIF Board of Directors.

To accelerate the development of new therapies for Alpha-1, regulatory flexibility and the adoption of innovative trial designs, surrogate endpoints, and accelerated pathways are critical. The FDA's collaboration with AIF and other stakeholders in recognizing the urgent need for more comprehensive therapies will help foster an environment conducive to innovation. By employing these regulatory tools, the FDA can ensure that new treatments reach patients more rapidly, fulfilling an unmet medical need and offering hope to those suffering from this debilitating condition.

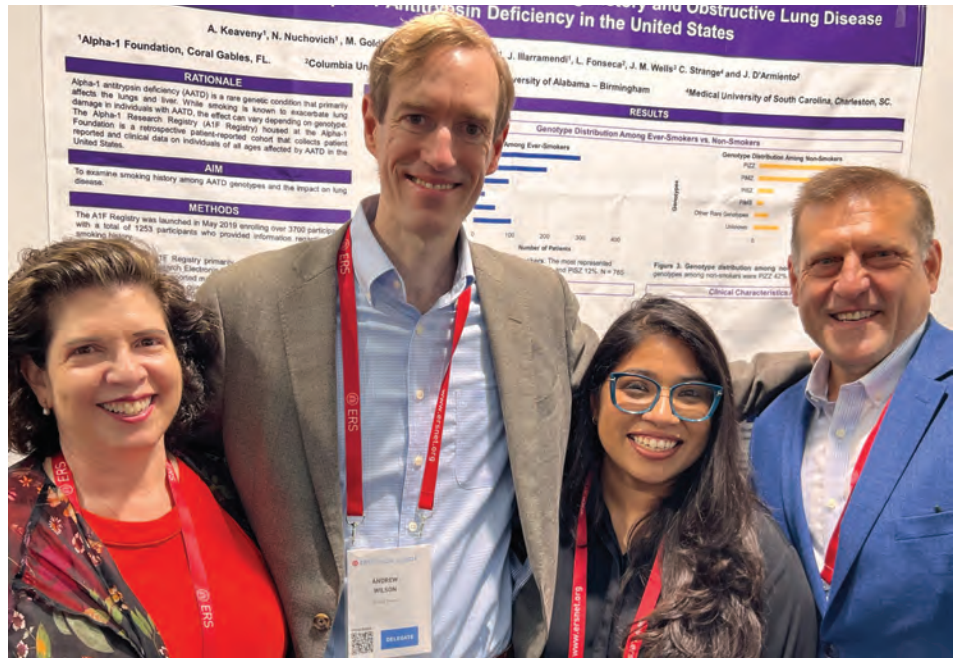


The Alpha-1 Foundation supports understanding Alpha-1 so that we can identify these potential pathways and aspects of Alpha-1 that could be targets for therapeutics. AIF has invited all this interest through its support of basic research, and through the direct support of a lot of these therapeutic interventions.

—Brooks Kuhn, MD, MAS, Director of the University of California Davis AIF Clinical Resource Center



AIF has invested heavily in the infrastructure needed to advance therapeutic development, including the creation of a comprehensive patient and medical registry, funding of translational research grants, establishment of AIF Clinical Resource Centers (CRCs), and development of the Therapeutic Development Network (TDN). These efforts are designed



to support the discovery and testing of new treatments and to facilitate patient recruitment for clinical trials.

In FY2024, AIF invested more than \$3.5 million in peer-reviewed research grants supporting innovative research spanning the spectrum of scientific discovery.

The AIF Grants Award Program funds a wide range of meritorious research efforts designed to improve the health of individuals with Alpha-1 with the goal of finding a cure. Promoting research to find better treatments and ultimately a cure for Alpha-1 is a core mission of AIF. Investigator-initiated grants have been the driving force of AIF's peer-reviewed research programs, in which researchers themselves drive scientific innovation.

AIF Grants are awarded in the following categories:

- **Ethical, Legal and Social Issues Relating (ELSI) to Alpha-1:** The ELSI grant provides support for the development of new information that contributes to the understanding of bioethical, legal, economic, and/or social issues associated

with Alpha-1

- **John W. Walsh Career Development:** The JWW Career Development grant provides support to physician-scientists as they establish a career in biomedical research and intend to continue their commitment to a cure through investment in the careers of junior physicians engaged in Alpha-1 research
- **Clinical Pilot:** The Clinical Pilot grant provides support for projects involving human subjects that will develop and test new hypotheses and/or new methods (or those being applied to the problems of Alpha-1 for the first time), and to support promising new investigators as they establish themselves in research areas relevant to Alpha-1
- **Pilot and Feasibility:** The Pilot and Feasibility grant provides support to encourage the development and testing of new hypotheses and/or new methods in Alpha-1 relevant research
- **Postdoctoral Research Fellowship:** The Postdoctoral Research Fellowship grant provides the support for postdoctoral research fellows who are starting their research careers and are working in

the laboratories of established researchers or conducting research under their mentorship.

- **Research:** The Research grant provides support to encourage the development of new information that contributes to the understanding of the basic biology of alpha-1 antitrypsin expression, and the pathogenesis/management of Alpha-1
- **Scientific Meeting Sponsorship:** The Scientific Meeting Sponsorship grant provides support for scientific

meetings, workshops, or conferences relevant to Alpha-1 and AIF's mission

- **Travel Grants:** The Travel grants provide support to attend national and international scientific conferences or meetings to present Alpha-1 related abstracts or support training at an established Alpha-1 Laboratory
- **Out-of-Cycle Grants Program:** The Out-of-Cycle Grants Program provides opportunities for investigators with large clinical projects that need support

- **Matching Grants Program:** AIF partners on a matching grant with the American Thoracic Society (ATS) titled ATS/AIF Young Investigator Grant in Alpha-1 that is targeted to early-career investigators interested in Alpha-1 research. Matching grants are also provided by American College of Chest Physicians (CHEST) and American Association for the Study of Liver Diseases (AASLD) ↑



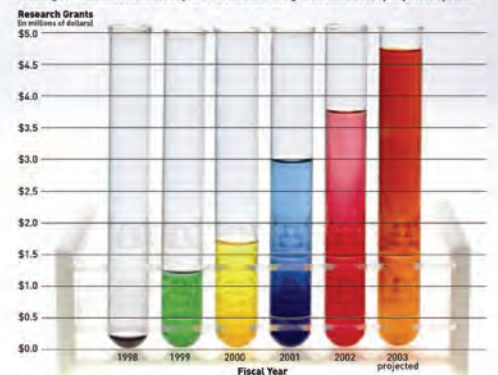
Basic science is going to continue to generate novel therapies so that the things we are only imagining right now will be the future for Alpha-1 individuals.

—Mark L. Brantly, MD,
AIF Board Member



Climbing Toward a Cure

Funding for research from the Alpha-1 foundation has grown dramatically in just six years.



Patient Centric Clinical Trials: A1F Therapeutic Development Network (TDN)



The Therapeutic Development Network (TDN) is an initiative of the Alpha-1 Foundation (A1F) to accelerate progress in drug development, improve care and lead to a cure. This advanced clinical trial platform brings together Alphas, top specialists, researchers, clinical trial sites (healthcare centers that execute clinical trials) and biopharmaceutical partners to improve clinical trial design, recruitment and enhance patient-focused drug development programs.

At its core, the TDN serves a dual mission. First, it aims to quickly identify and match Alphas interested in clinical trials with suitable opportunities. Second, it partners with the pharmaceutical industry and academic researchers to develop trials that reflect the experiences of the Alpha-1 community and execute these studies efficiently at centers with highly qualified staff. This precise, patient-centered approach streamlines recruitment while ensuring that clinical trials are conducted at the highest standards of care.

What does joining the TDN mean?

By joining the TDN, you're taking an active role in shaping future clinical trials and exploring

opportunities to take part in research that may be right for you. With just a small amount of medical information, you will contribute to the thoughtful design of new clinical trials.

When new clinical trials are available, those who may be a good fit will be contacted directly by an enrolling TDN center. Participation in any clinical trial is entirely optional and requires your consent, ensuring your involvement is always up to you.

Joining the TDN is a meaningful way to influence valuable research while staying informed about opportunities tailored to your profile.

How Can Alphas Join the TDN?

Alphas who wish to join the TDN can do so via their care center, if it is part of the Network, or through our virtual center. Participation begins with signing the TDN informed consent form, allowing the collection of targeted medical information and enabling future contact for matched clinical trials. Every three months, TDN participants are contacted for updates on their health and to ensure their medical records remain current.

Participation in the Alpha-1 Research Registry is

also required, supporting AIF's mission to build a comprehensive database detailing the natural history of Alpha-1. Additionally, participants can permit their medical data collected by the TDN to be shared with the Alpha-1 Research Registry, further strengthening this vital resource. The TDN demands only a minimal time commitment from participants, and enrollment may not require an in-person visit. As a participant in the TDN, you'll receive a modest stipend to recognize your participation.

What is the difference between the Alpha-1 Research Registry and the TDN?

The Alpha-1 Research Registry is a secure, confidential database designed to advance research for individuals with Alpha-1 and Alpha-1 carriers. Powered by patient participation, the Alpha-1 Research Registry aims to develop a deeper understanding of the natural history of Alpha-1 by gathering a wealth of patient-reported data throughout a participant's life.


Joining the Alpha-1 Research Registry is simple

and convenient. Participants can enroll online and complete health-related questions at their own pace. Additionally, the Alpha-1 Research Registry team gathers a small number of medical records to complement the self-reported information. This robust database serves as a critical resource, advancing Alpha-1 research and fostering discoveries that could improve lives. To keep participants informed, the Alpha-1 Research Registry team regularly shares updates and educational materials, as well as details about relevant clinical trials.

The TDN includes a sub-set of Alpha-1 Research Registry participants who meet specific inclusion criteria. It collects a carefully selected set of medical information entered by TDN staff. This targeted database supports the development of clinical trials by allowing TDN staff to query its data when working with industry or academic partners. TDN members also use the database to identify individuals who may qualify for third-party clinical trials, connecting Alphas with research opportunities that align with their profiles.

For more information about the TDN and to join, contact our team at TDN@alpha1.org.



 Contract Executed (14)  Negotiating Terms (3)  Pending Initial Comments (3)



Together, the Alpha-1 Research Registry and the TDN play a vital role in driving meaningful research, empowering participants, and moving closer to new treatments and therapeutic solutions for Alpha-1.

Where to Join the TDN

The TDN leverages the Alpha-1 Foundation's Clinical Resource Center (CRC) program, a network of physicians and research centers across North America, built to manage the complexities of caring for Alphas. By collaborating with experts in Alpha-1 care and research, the TDN ensures clinical trials are executed with excellence.

The first TDN center was launched in the summer of 2023 at the Hannibal Clinic in Missouri, under Dr. Humam W. Farah's leadership. Since then, the TDN has grown swiftly to include 20 contracted centers, with additional centers qualifying to join. Beyond physical locations, the TDN will soon introduce a virtual enrollment center managed by its internal team to directly engage patients, expanding the TDN's reach and accessibility.


Supporting Industry & Academia

The TDN team brings over a century of combined expertise in clinical trials and research operations, making it the most trusted resource in the field of Alpha-1. Importantly, the TDN team and the physicians and researchers within the network are readily available to assist industry and academic

partners in optimizing clinical trial design and execution. The TDN offers a comprehensive range of services tailored to support industry partners of all sizes and academic researchers who are focused on advancing treatments for Alpha-1.

Looking Ahead

AIF plans to expand its collaborations with biopharma companies, academic institutions, and other key stakeholders to stay at the forefront of drug development advancements. By continuing to innovate in clinical trial design, recruitment methods, and patient-focused research, AIF remains resolute in its mission to improve care for the Alpha-1 community.

Patient involvement remains at the heart of these efforts. Participation in the Alpha-1 Research Registry and the TDN directly supports the discovery of new therapies and progress towards a cure for Alpha-1. Together, these initiatives are paving the way for breakthroughs in the fight against Alpha-1. 



For more information about the TDN and to join, contact our team at TDN@alpha1.org.

Question	Registry	TDN
Who can participate?	<ul style="list-style-type: none"> • Anyone of any age with Alpha-1 • Alpha-1 carriers 	<ul style="list-style-type: none"> • Anyone with Alpha-1 with severe deficiency, with or without lung or liver disease • Alpha-1 carriers who are relatives of Alphas enrolled in the TDN • Alpha-1 carriers with lung or liver disease
What type of data is collected?	<ul style="list-style-type: none"> • Self-reported information regarding various aspects of your clinical history • A few medical records 	<ul style="list-style-type: none"> • Medical record information specific to known/ planned clinical trial inclusion criteria
Who enters the data into the database?	<ul style="list-style-type: none"> • Alphas answer questions regularly for as long as they participate in the Registry and at their convenience • Registry team enters medical records when provided by the participant 	<ul style="list-style-type: none"> • The TDN staff reviews the participant's medical records and enters data into the TDN database
How much data is collected?	<ul style="list-style-type: none"> • Approximately 1,200 data points collected for each patient. • Small number of medical records 	<ul style="list-style-type: none"> • Approximately 150 data points upon enrollment • Approximately 75 data points every 3 months thereafter
Who has access to the data?	<ul style="list-style-type: none"> • Registry staff • Third-party researchers are granted access to de-identified data to support epidemiologic studies upon approval post application process 	<ul style="list-style-type: none"> • TDN staff • TDN center staff—only to the information assigned to their center to identify potentially eligible clinical trial participants
How is the data used?	<ul style="list-style-type: none"> • Registry team uses the information to identify knowledge gaps and educate Alphas on key clinical issues • Epidemiologists use the data to understand Alpha-1 	<ul style="list-style-type: none"> • TDN team uses de-identified information to help researchers design clinical trials • TDN center staff use identifiable information to identify Alphas that may qualify for a clinical trial



Clinical Trials Education

Interested in better understanding how a drug comes to fruition?

Through the Clinical Trials Education Program, you can learn more about different phases and types of clinical trials, how inclusion/exclusion criteria can affect participation in a study, what is informed consent and why it is important, and the next steps to get involved!

For more information, visit:

www.alpha1.org/clinical-trials-101



Commitment to Finding All Alphas: Insight Into Awareness and Detection

The most pressing challenge facing the Alpha-1 Antitrypsin Deficiency (Alpha-1) world is the vast number of undiagnosed, and often misdiagnosed, patients with Alpha-1. As a result, patients with this undiagnosed (or misdiagnosed) condition are deprived of current Alpha-1 specific treatments and therapies that slow down the progression of disease. These Alpha-1 patients who are not detected may miss out on access to potential new treatments and therapies that are currently under development for Alpha-1. Detection of Alpha-1 is vital for patients and their at-risk family members to receive the appropriate treatment and therapies that improve and ultimately save lives.

Detection efforts have, and will always remain, an essential part of the Alpha-1 Foundation's (A1F) mission. A1F recognizes that early detection of Alpha-1 is the key to better outcomes, offering the possibility of timely interventions, effective treatments and ultimately, a better quality of life for those affected by Alpha-1.



The History of Alpha-1 Detection Programs & Initiatives

Family Testing

A1F strongly encourages people with Alpha-1 symptoms and their family members to be tested. This includes the parents, siblings, and children of a person with Alpha-1, as well as extended family members, such as aunts, uncles, and cousins. The highly accessed *It's All in the Family* A1F brochure provides a comprehensive outline for family members to learn more about Alpha-1 and steps to educate themselves on their risk and testing.

Alpha-1 Coded Testing (ACT) Study

ACT is a confidential testing program designed to support individuals who are at risk by offering them a discreet and free way to learn their Alpha-1 status. 40,000 individuals have been enrolled and screened since the inception of the ACT Study in 2001. The high detection rate of Alphas from this study can be explained by a higher degree of Alpha-1 family testing. Targeted detection concentrates its efforts on a population that carry an increased risk for a deficient Alpha-1 gene.



State of Florida Detection Program

The State of Florida Department of Health and Human Services and A1F worked together for 16 years on a Detection and Education Program to educate and identify individuals with Alpha-1.

Beginning in 2000, A1F implemented a wide variety of activities to educate healthcare providers and at-risk individuals on the importance of an appropriate diagnosis of Alpha-1. A1F partnered with statewide institutions like the Florida Medical Association, Florida Academy of Physician Assistants, Florida Society for Respiratory Care, American Lung Association in Florida and other local organizations

and institutions to schedule Grand Rounds, Continuing Medical Education (CME) programs, Continuing Education Units (CEU) programs and medical related conferences. These activities were used to educate different healthcare providers including physicians, nurses, respiratory therapists, physician assistants, and healthcare students.

AIF staff attended Better Breathers groups organized by the American Lung Association of Florida and organized special education events for the public to address the lung and liver component of Alpha-1 disease. Alpha-1 became the subject of discussion at almost every lung conference in Florida targeting healthcare professionals and led to more gastroenterologists, internal medicine doctors, and general physicians requesting information and materials from AIF about Alpha-1.

COPD Foundation

In 2002, John W. Walsh, co-founder of AIF and the COPD Foundation, began the strategic alignment of Alpha-1 with the Chronic Obstructive Pulmonary Disease (COPD) community and gathered a dedicated team of professionals and fellow patients for operation "Trojan Horse", to ensure that all at-risk COPD patients were tested for Alpha-1. The COPD Foundation was established in 2004 to help a bigger and largely unrecognized community of those with COPD.

Continuing Medical Education (CME)

AIF takes pride in providing continuing medical education to healthcare providers to increase awareness and detection of Alpha-1. Several AIF education events throughout the year are approved by the American Association for Respiratory Care (AARC) for Continuing Respiratory Care Education (CRCE). In 2022, AIF partnered with the Cleveland Clinic to develop a free series of on-demand webcasts to improve the knowledge and competence in diagnostics and treatment of Alpha-1. AIF takes pride in The Alfred and Norma Stoller Continuing Medical Education (CME) Series, an accredited program designed to help clinicians and healthcare providers learn about Alpha-1.

Scientific Meetings & Conferences

Each year, AIF exhibits at many scientific meetings and conferences to reach a wide audience of healthcare providers, researchers, and clinicians including the American Thoracic Society (ATS) International Conference, the American College of Chest Physicians (CHEST) Annual Meeting, the American Association for the Study of Liver Diseases (AASLD) Meeting, the American Association for Respiratory Care



(AARC) Congress, the National Institutes of Health (NIH) Rare Disease Day, the European Respiratory Society (ERS) International Conference, and the North American Society for Pediatric Gastroenterology, Hepatology and Nutrition Annual Meeting.

In 2003, the “Standards for the Diagnosis and Treatment of Individuals of Alpha-1” was released by ATS and ERS and continues to be disseminated at conferences and meetings.

[Moving Forward: Detection in 2025 and Beyond](#)

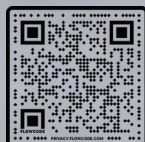
A1F has made early detection its primary strategic priority and is committing resources to initiatives designed to enhance the overall experience of the Alpha-1 community. After reviewing the initial 2024 Alpha-1 Community Market Survey results, it was clear that A1F needs to play a larger role in detection for Alpha-1. Discussions with key opinion leaders and experts have assisted A1F in creating an exploratory plan to move forward. By prioritizing early detection, A1F will improve individuals' outcomes and contribute to the broader goal of better health and well-being for all Alpha-1 patients.

To achieve this goal, A1F has embarked on a multi-faceted initiative that brings together patients, clinicians, industry partners and government agencies in a collaborative effort to enhance the detection process. A1F's approach to early detection is not just about developing new tools and technologies, it is about creating an experience that enhances every aspect of the detection process, including education about early Alpha-1 detection, improving access to screening programs, like newborn screening, and ensuring that the latest diagnostic tools are available. The diagnostic algorithm is likely to change as novel techniques and next generation sequencing becomes broadly available.

At the October 2024 A1F Board Meeting, the Board of Directors unanimously agreed that A1F would move forward and take a leadership role in centralizing detection efforts for the entire Alpha-1 community. These efforts will include partnering with all key stakeholders to develop a unified approach to align and embed guidelines, protocols and tools that set Alpha-1 screening as a routine standard of care for lung and liver conditions, thereby reducing the time it takes for a patient to receive a proper diagnosis for Alpha-1. 📌

Call for 2025 Alpha-1 Educational Scholarship Applications!

- The application will soon be available on the website. The deadline to submit the completed application with all attachments is **April 1, 2025**.
- Scholarships are available to Alphas and their immediate family members.
- Scholarships can be used for study at an approved institution for post-high school education and career change/returning adult students.
- To learn more about the award process and download an application, visit alpha1.org/alpha-1-educational-scholarships



THE POWER TO CONNECT

Thank you, Alpha-1 community!

260.6K+

People
reached on
Social Media

800+

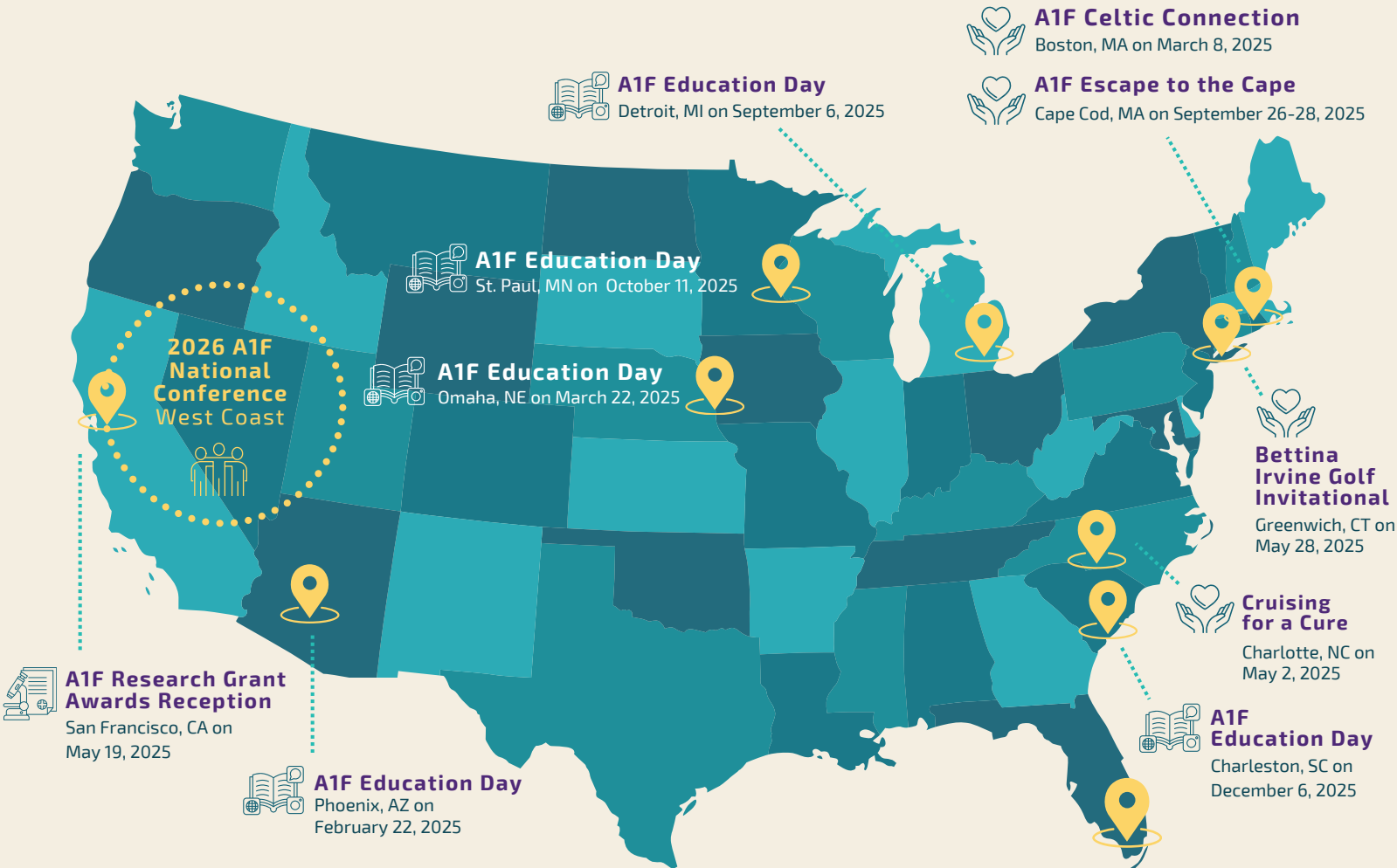
Participants
in the
A1F Virtual Walk

\$86K+


Raised for
Alpha-1 Research
& Programs



2025 Major Meetings & Events




Virtual




The Virtual Alpha-1 Foundation Education Day & State of the Union will take place **January 22, 2025**.

Alpha-1 Global



The Alpha-1 Global 7th Research Conference & 10th Patient Congress will take place **April 4-5, 2025**, in Portugal.

Netherlands



The 2025 Clinical Trials Roundtable at EASL Congress will be held in Amsterdam on **May 7 - 10, 2025**.

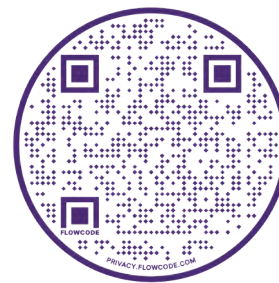
-  **Alpha-1 Foundation National Conference**
Miami, FL on June 6 - 8, 2025
-  **Alpha-1 Leadership Meetings**
Miami, FL in October 2025
-  **28th Gordon L. Snider (GLS) Critical Issues Workshop**
Miami, FL in October 2025



Calendar of Events 2025

Are you receiving our emails?

Do you receive the monthly e-newsletter “Community Currents”? This is a good time to check that you are on our e-mail list. Update your contact information by visiting alpha1.org.



AIF Support Group Meetings

February 1, 2025

Reno- Tahoe Support Group
Reno, NV

February 2, 2025

Mass Alphas
Boston, MA

For additional support group meetings, please check the calendar of events on alpha1.org/calendar

AIF Education Events

February 22, 2025

Phoenix AIF Education Day
Phoenix, AZ

March 22, 2025

Omaha AIF Education Day
Omaha, NE

April 4-6, 2025

AIF 7th Global Research Conference
& 10th Patient Congress
Lisbon, Portugal

June 6-8, 2025

2025 AIF National Conference
Miami, FL

September 6, 2025

Detroit AIF Education Day
Detroit, MI

October 11, 2025

St. Paul AIF Education Day
St. Paul, MN

December 6, 2025

Charleston AIF Education Day
Charleston, SC

AIF Upcoming Events

March 8, 2025

Celtic Connection
Boston, MA

May 2, 2025

Cruising for a Cure
Lake Norman, NC

May 29, 2025

Bettina B. Irvine Invitational Classic
Greenwich, CT

September 26-28, 2025

Escape to the Cape 2025
Cape Cod, MA

October 25, 2025

Alpha-1 Gulf Coast Ride 2025
Clearwater, FL

November

Alpha-1 Awareness Month & AIF
Virtual Walks
USA



Alpha-1 Foundation



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Be sure to update your e-mail address and contact information by visiting:

 alpha1.org/subscribe

