



2024

PROGRAM
REPORT



OUR MISSION

The Alpha-1 Foundation (A1F) is committed to finding a cure for Alpha-1 Antitrypsin Deficiency (Alpha-1) and improving the lives of people affected by Alpha-1 worldwide.

The Alpha-1 Foundation (A1F) is dedicated to finding a cure for Alpha-1 Antitrypsin Deficiency (Alpha-1) and tirelessly improves Alpha-1 patient care. This past year, A1F experienced incredible success and unmatched engagement within the Alpha-1 community - from record-setting attendance at Alpha-1 Education Days and the Alpha-1 National Conference to revamping its support group network, reaching \$100 million in Alpha-1 research funded and advancing support for the John W. Walsh Home Infusion Act. All of these efforts support the Alpha-1 patient journey and continue A1F's global leadership in education, advocacy, content, and support.

A1F is the number one source of information and resources focused on Alpha-1 and supports programs that push the boundaries of existing data, detection, and patient support. A1F's most recent accomplishments and highest-achieving accreditations in the nonprofit sector reflect its unwavering commitment to medical research in the field of Alpha-1.

The dynamic and involved community of volunteers, researchers, clinicians, partners, and donors makes the work of A1F possible. Their dedication inspires and empowers A1F every day in its mission to raise awareness of Alpha-1 and deliver results for those affected by the condition. A1F is incredibly grateful to its global network of specialists who provide Alpha-1 patient care; a network which spans multiple industries, areas of research, and academic expertise. Thanks to these innovative and patient-centric groups, A1F is driving real, positive change for the Alpha-1 community.

It is an exciting moment in time. A1F finds itself at a pivotal point in its nearly 30-year history. The synergy of science, medicine, and technology, along with leadership, has A1F primed to deliver hope for all Alpha-1 patients and their families.

The Alpha-1 Foundation sincerely appreciates your outstanding support as it continues to fund critical research, and improve treatment, quality of life, and outcomes for all Alphas, while working toward a cure for Alpha-1.

A1F Staff at the 2024 A1F National Conference



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RESEARCH & SCIENCE

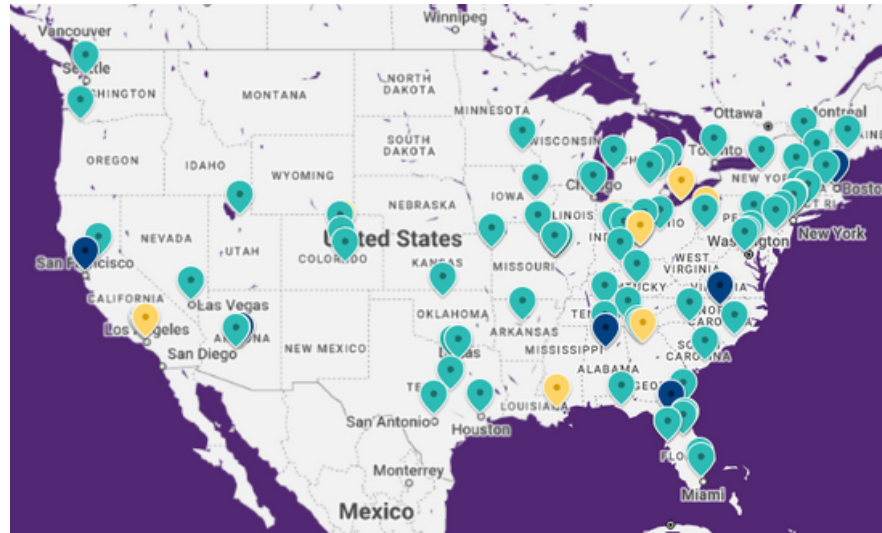
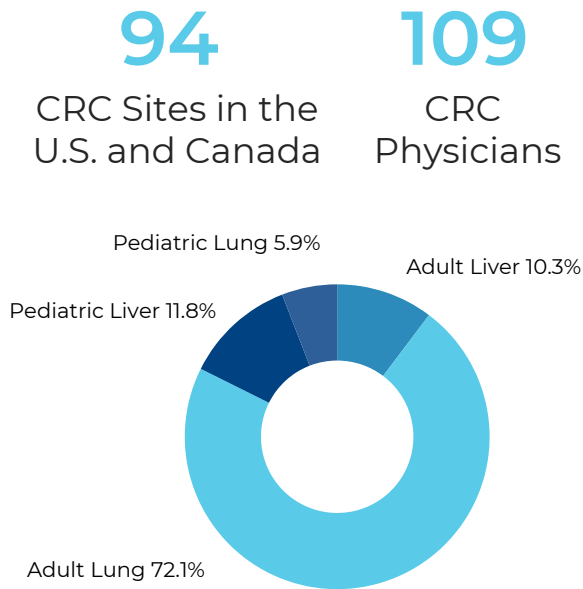
Accelerating research toward a cure for Alpha-1

The Alpha-1 Foundation takes a comprehensive, big-picture approach to research, from funding targeted, high-impact programs to advancing the Alpha-1 research field.



CLINICAL RESOURCE CENTERS (CRCs)

A growing network of research institutions and physicians specializing in Alpha-1 treatment, education, and care



Key: ● Liver (adult) ● Liver (pediatric) ● Lung (adult) ● Lung (pediatric)

Designated 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 AIF'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.

LOOKING AHEAD: Clinical Resource Centers (CRCs)

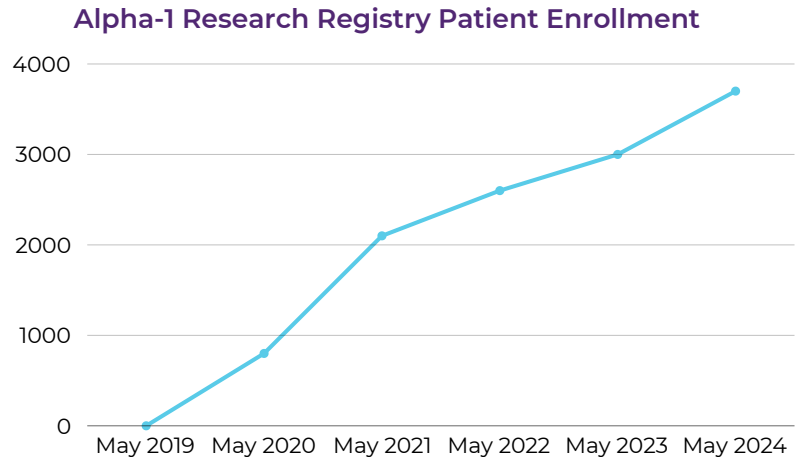
The Alpha-1 Foundation is committed to further strengthening the CRCs to better serve Alphas and the broader research community. One of AIF's key initiatives is revamping the program to enhance its effectiveness and reach. A series of one-on-one interviews with current specialists were conducted to gather feedback and suggestions on program enhancement. Ideas included increasing utilization of the CRC Access Program, which is designed to ensure that more Alphas can benefit from the expertise and support offered by CRCs.

Another critical focus is the development of multi-disciplinary teams within each CRC. AIF recognizes that the complexities of Alpha-1 require a holistic approach to care. By building teams that include specialists from various disciplines—such as pulmonologists, hepatologists, nutritionists, and mental health professionals—AIF can provide more integrated and coordinated care. These teams will work together to create personalized care plans that address the full spectrum of patient needs, from medical treatment to emotional and psychological support.

ALPHA-1 RESEARCH REGISTRY

Facilitating participation in research studies and clinical trials for new treatments and therapies

The Alpha-1 Research Registry, a comprehensive database integrating both patient-contributed and clinical data, has emerged as an essential resource for the Alpha-1 community and a powerful tool in advancing medical research. It is a platform to address one of the most formidable obstacles in rare disease research—the challenge of identifying and connecting with patients who are living with or affected by these conditions. This difficulty often results in delays or roadblocks in the recruitment of participants for research studies, which in turn slows the pace of therapeutic development.



Recognizing the urgency of this issue, AIF created an enhanced Alpha-1 Research Registry 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 by helping to match eligible individuals with appropriate clinical studies. The Alpha-1 Research Registry plays a pivotal role in the Alpha-1 Biomarkers Consortium (A1BC) Study and will serve as the patient clinical data repository for the Therapeutic Development Network (TDN).

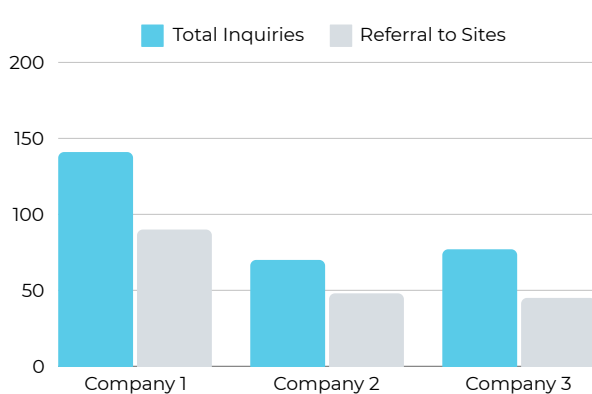
600+ Alphas & family recruited in FY 2024

3,681 Total participants in the Alpha-1 Research Registry

1,100+ Participants that are ZZ Alphas

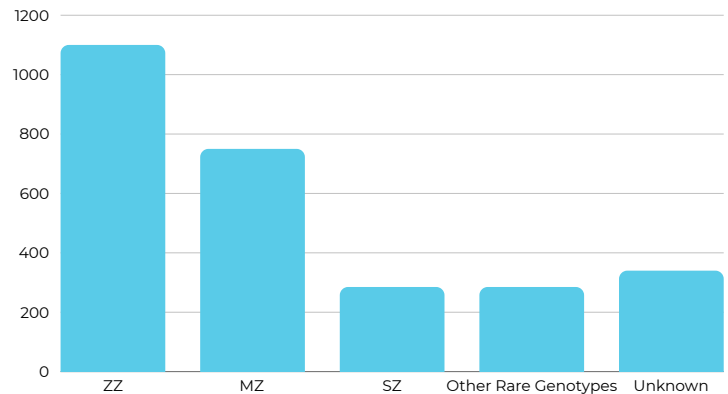
>50% Report having deficient alleles (ZZ, SZ, FZ, FF, SS, MF, Znull, Mnull, Snull, ZMheerlen, ZMmalton or other rare deficient alleles)

Total Inquiries and Referral Metrics



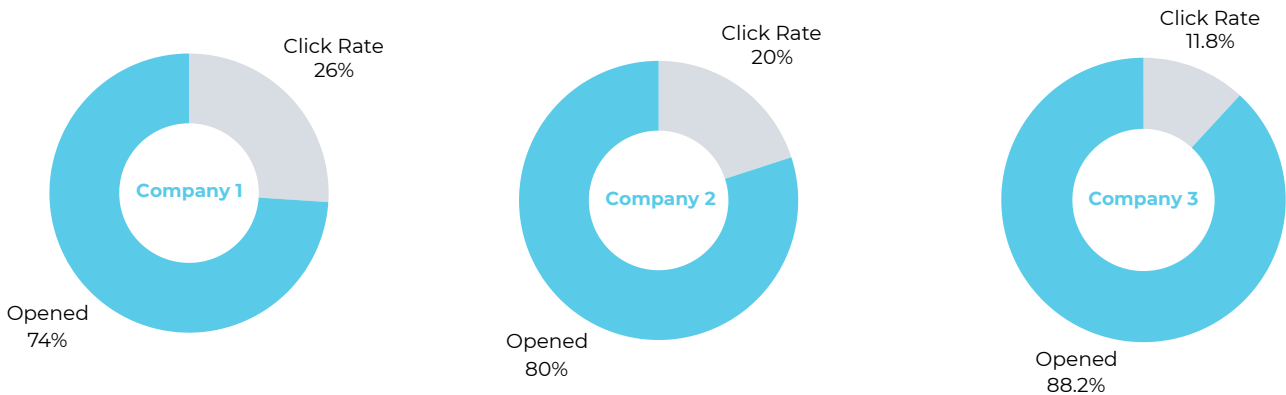
Three trials averaging 96 potential participants with 64% referred to trial sites

Genotype Distribution Among Alpha-1 Research Registry Participants



Email Success Metrics

Alpha-1 Research Registry Outreach Promoting Clinical Trial Availability



Abstracts Accepted for Poster/Presentation

- 2023 AATD Liver Meeting Naples, Italy
- 2023 and 2024 European Respiratory Society (ERS) International Congress
- 2023 and 2024 National Organization of Rare Disorders (NORD) Breakthrough Summit
- 2023 and 2024 Rare Disease Day at National Institutes of Health (NIH)
- 2023 and 2024 American Thoracic Society (ATS) International Conference
- 2023 and 2024 CHEST Annual Meeting
- 2024 AASLD The Liver Meeting



Dr. Jeanine D'Armiento presenting an Alpha-1 Research Registry poster

LOOKING AHEAD: Alpha-1 Research Registry

The infrastructure established within the Alpha-1 Research Registry is key to the success of A1F's efforts. It enables the Foundation to perform targeted screenings of potential participants, ensuring that they meet the specific criteria required for various studies.

A1F's innovative recruitment process has already yielded encouraging results. There have been early successes, with a growing number of Alphas responding positively to invitations for study participation. This response underscores the impact of A1F's approach, as more patients are being successfully enrolled in clinical trials, paving the way for the development of new therapies and treatments. Many partners have utilized this impressive resource, including Vertex, Takeda, Inhibrx/Sanofi, Cleveland Clinic, and University College London.

It is now more crucial than ever for Alpha-1 patients to enroll in the Alpha-1 Research Registry, especially liver-affected Alphas. As A1F embarks on a Natural History Liver Study of Alpha-1 and prepares for global recruitment for clinical trials around gene editing, having patients as partners is more important than ever before.

THERAPEUTIC DEVELOPMENT NETWORK (TDN)

Connecting the top specialists and industry partners to accelerate the development of novel treatments for Alpha-1

The Therapeutic Development Network (TDN) is a cornerstone of A1F's efforts to advance patient care and drug development. This clinical trial platform brings together a diverse group of leading specialists, researchers, and biopharma partners, all working in concert to improve the recruitment process for clinical trials and design innovative, patient-focused drug development programs.

The TDN leverages the power of the Alpha-1 Research Registry and the extensive network of Clinical Resource Centers (CRCs) to facilitate clinical trial recruitment and site selection for industry partners. By tapping into these resources, A1F can more effectively identify and engage with patients who are eligible to participate in clinical studies, thereby accelerating the development of new therapies.

This network also allows A1F to strategically select sites that are best equipped to conduct these trials, ensuring that each study is carried out with the highest standards of care and scientific rigor.






23 Qualifying Sites

14 Contracted

3 In finalization stages

1/3 Of TDN enrollees are new to the Alpha-1 Research Registry

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

LOOKING AHEAD: Therapeutic Development Network (TDN)

The Alpha-1 Foundation recognizes the importance of continually evolving its approach to meet the changing needs of patients and the research community. A key initiative is the revitalization of the CRCs, with a focus on ensuring seamless integration with the TDN to ensure that patient care remains at the heart of clinical efforts.

As the TDN continues to progress in site engagement and clinical trial readiness, it is imperative that the team remains well-equipped to drive the program toward continued success. A1F plans to expand partnerships with biopharma companies, academic institutions, and other key stakeholders to keep pace with the latest advancements in drug development.

The TDN is a vital component of the mission to improve patient care and accelerate the development of new therapies. By leveraging the Alpha-1 Research Registry, CRCs, and industry partnerships, the TDN is helping to transform the landscape of clinical research.

RESEARCH GRANTS AND AWARDS

Sponsoring research into lung and liver disease, as well as ethical, legal, and social issues impacting people with genetic conditions

The Alpha-1 Foundation directed more than **\$3.5 million** in peer-reviewed research grants in FY 2024. These grants support innovative research spanning the spectrum of scientific discovery.

World's #1 Funder of Alpha-1 Research

\$100+

Million invested in Alpha-1 Research

130

Institutions funded in 14 countries worldwide

Advancing the Next Alpha-1 Breakthrough

Research funded by the Alpha-1 Foundation has improved understanding of the mechanisms underlying the clinical manifestations of Alpha-1 and identified novel drug targets likely to lead to new therapeutic solutions.

2023-2024 GRANT RECIPIENTS

In-Cycle Research Grants Recipients

The research grants were awarded in-cycle for basic and translational research and provide funds to encourage the development of new information that contributes to the understanding of Alpha-1.



Valerie Gouon-Evans, PhD
Boston Medical Center
Promoting Progenitor-Driven Liver Regeneration as an AATD Associated Liver Disease Therapy

Devipriya Harinath, PhD
Columbia University
Diversity of SERPINA1 Mutations as Reflected in an Indian Population



Kristen Hudock, PhD
University of Cincinnati
A Novel Strategy to Deliver Intrapulmonary AAT & Limit Lung Destruction in Models of AATD

Jorge Lascano, MD
University of Florida
Nicotine as a Cause of Airway Inflammation and Lung Emphysema in a Novel AATD Mouse Model



Camila Lopes-Ramos, PhD
Brigham and Women's Hospital
Multi-omic Sex Differences in Alpha-1 Antitrypsin Deficiency Associated COPD

Karen McDonald, PhD
University of California, Davis
Production and Characterization of a Biobetter Recombinant AAT-Fc Therapeutic Protein



Francesca Polverino, MD, PhD
Baylor College of Medicine
B Cell Adaptive Immune Profile in A1AT Deficiency-Associated Emphysema

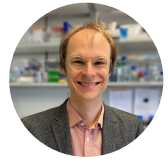
Sheikh Tamir Rashid, MD, PhD
Imperial College of Science, Technology and Medicine
Deciphering the Molecular Landscape in Alpha-1 Antitrypsin Deficiency Liver Disease





Leonard Riley, MD
 University of Kansas Medical
 Center Research Institute
*Impact of a Multicenter Best
 Practice Alert to Improve AATD
 Testing and Detection*

Florian Rosenberger, PhD
 Max Planck Institute of Biochemistry
*Alpha-1 Hepatocyte Dynamics: A
 Single-Cell and Spatial Proteomics
 Study*



Konstantinos Thalassinos, PhD
 University College London
*Cellular Effects of A1AT
 Aggregation with Single-Cell and
 Crosslinking Proteomics*

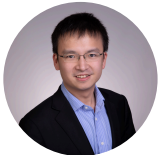
Pilot and Feasibility Grant Recipients

The objective of the Pilot and Feasibility grants are to provide funds to encourage the development and testing of new hypothesis and/or new methods in research areas relevant to Alpha-1.



Emily Moser, PhD
 University of Florida
*Alpha-1 Antitrypsin Promotes
 Vaccine Antibody Responses*

Suzanne Roche, MB, BCH, BAO
 Royal College of Surgeons in Ireland
*Genetic Discrimination – Tackling
 a Growing Issue in Alpha-1
 Antitrypsin Deficiency*



Huiliang Wang, PhD
 University of Texas at Austin
*Genetic Engineered Exosomes for
 Efficient DNA Delivery for Alpha-1
 Antitrypsin Deficiency*

Rhiannon Werder, PhD
 Murdoch Children's Research Institute
*Investigating the Cellular and
 Molecular Mechanisms of Respiratory
 Infections in AATD*



Postdoctoral Research and Fellowship Grants



Vera Khodzhaeva, PhD
 Cambridge Institute for Medical
 Research - University of Cambridge
*FGF21 and Cellular Homeostasis
 in Alpha-1 Antitrypsin Deficiency*

Mark Murphy, PhD
 Royal College of Surgeons in Ireland
*A Detailed Study of Lung
 Immune Cell Outcomes in AATD*



Hirofumi Kiyokawa, MD, PhD
 Boston University
*Regeneration of Lung Epithelial
 Stem Cell Compartments in a Novel
 Z-AAT Mouse Model*

Valentina Schiano, PhD
 Fondazione Telethon ETS
*Targeting Inflammation as
 Therapeutic Approach for AATD*



ALPHA-1 DETECTION

Finding all Alphas to improve individual outcomes and advance the AIF mission

The Alpha-1 Foundation is deeply committed to improving the lives of individuals affected by this condition, a mission that drives every aspect of its work. With this in mind, and with the support of the Board of Directors, AIF has renewed its focus on one of the most crucial aspects of healthcare: early detection. Early detection is the key to better outcomes, offering the possibility of timely interventions, more effective treatments, and ultimately, a better quality of life for those affected.

To achieve this goal, AIF 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. AIF's approach to early detection is not just about developing new tools and technologies; it's about creating an experience that enhances every aspect of the detection process. This initiative includes educating patients and healthcare providers about the importance of early detection, improving access to screening programs, and ensuring that the latest diagnostic tools are available.

One such tool is the Alpha-1 Coded Testing (ACT) Study. It is a confidential program designed to support individuals who are at risk by offering them a discreet and free way to understand their Alpha-1 status. Family testing material is featured at Alpha-1 Foundation Education Days and Clinical Resource Centers, and AIF distributes a series of communications to engage Alphas in this important detection program, including the *It's All in the Family* brochure and newsletter features on Family Testing.

The ACT study has already yielded valuable insights:

5.4%

of those tested were diagnosed as ZZ Alphas

34%

of those tested were identified as carriers of Alpha-1

The ACT study has enrolled and screened more than:

40,000

individuals since inception

1,306

participants in FY 2024



LOOKING AHEAD: Detection

Early detection is one of the most important areas in which AIF has placed a recommitted focus to transform the lives of those affected by Alpha-1. As a leader of innovation in the Alpha-1 community, AIF is committed to continuously improving and expanding efforts, integrating new technologies, and fostering stronger collaborations. Through collaborative efforts with patients, clinicians, industry partners, and government agencies, AIF is driving forward a variety of detection initiatives designed to enhance the overall experience for the community. After reviewing the initial market survey results, it was clear that AIF needs to play a larger role in detection for Alpha-1. Discussions with key opinion leaders and experts have assisted AIF in creating an exploratory plan to move forward. By prioritizing early detection, AIF is not only improving individual outcomes but also contributing to the broader goal of better health and well-being for all Alphas.

ALPHA-1 BIOMARKERS CONSORTIUM (A1BC)

Studying disease indicators to identify at-risk patients and determine early interventions

The Alpha-1 Biomarkers Consortium (A1BC) aims to identify biomarkers that could be used to study the clinical progression of disease and inform the design of future clinical trials. The A1BC is conducting a study that taps into the Alpha-1 Research Registry to identify unaddressed research questions in Alpha-1 and clinically relevant outcomes.

The A1BC has successfully completed enrollment with over 270 PiZZ Alpha participants. This 3-year study aims to identify biomarkers in blood, sputum, or CT imaging that could help better predict prognosis and progression of Alpha-1. The Foundation is excited to announce the expansion of this study to include patients with the PiMZ genotype. The PiMZ Longitudinal Cohort (PiMZ LogiC) aims to take a deeper look into lung-affected PiMZ Alphas with COPD and hopes to identify factors contributing to lung disease progression in an often-overlooked segment of Alphas. Participating centers for the MZ protocol include Columbia University Irving Medical Center, University of Alabama, National Jewish Hospital, University of California – Los Angeles, and University of Chicago.

The A1BC study is funded through a multi-center grant from the National Institutes of Health (NIH) and supplemented by funding from the Alpha-1 Foundation.

Columbia University Medical Center



Principal Investigator
Jeanine D'Armiento, MD, PhD
& Co-Investigator Monica
Goldklang, MD

Medical University of South Carolina



Co-Principal Investigator
Charlie Strange, MD

University of Alabama Medical Center



Principal Investigator
J. Michael Wells, MD, MSPH &
Co-Investigator
Surya Bhatt, MD, MSPH

Boston University



Site Investigator
Andrew Wilson, MD

Royal College of Surgeons in Ireland



Site Investigator
Prof. Gerry McElvaney, MD, DSc,
FRCPI, FRCPC
& Co-Investigator
Oliver McElvaney, MD, PhD, MRCPI

University of Chicago



Site Investigator
Kyle Hogarth, MD

University of North Carolina, Chapel Hill



Site Investigator
M. Bradley Drummond, MD, MHS

University of California, Los Angeles



Site Investigator
Igor Barjaktarevic, MD &
Co-Investigator Russell Buhr, MD

National Jewish Health



Site Investigator
Robert Sandhaus, MD, PhD

University of Utah Health Center



Site Investigator
Cheryl Pirozzi, MD

ALPHA-1 RESEARCH BIOREPOSITORIES

Providing researchers from around the world with difficult to obtain DNA, plasma, lung & liver tissue donated from Alphas, as well as supplementary biomaterial samples, to advance research

The Alpha-1 Biomaterials Exchange is a dynamic and essential resource designed to bridge the gap between industry and academia by facilitating the exchange of critical biomaterials. This platform serves as a vital connector, matching investigators in need of specific biomaterials with those who can supply them. The Alpha-1 Biomaterials Exchange goes beyond the traditional offerings of biobanks by including a diverse array of biomaterials that are often pivotal in cutting-edge research but may not be readily available through conventional channels. These materials include viruses, viral vectors, plasmids, genetically modified mice, and specialized cell lines, all of which play crucial roles in various fields of biomedical research.

The Alpha-1 DNA and Tissue Bank contains de-identified clinical information and DNA and plasma samples from 2,632 Alphas. It is accessible to researchers in academia, government, and industry whose applications are approved by A1F's DNA & Tissue Bank Advisory Committee.

The Alpha-1 Foundation is committed to the principles of open science and the sharing of resources. The A1F research program embodies this commitment by not only facilitating the sharing of biomaterials but also promoting the exchange of knowledge and data associated with these materials.



LOOKING AHEAD: Alpha-1 Research Biorepositories

The Alpha-1 Foundation aims to continue expanding its biomaterial offerings and reach, becoming an even more integral part of the global research ecosystem. Most recently, the Foundation has been working with several industry partners to assist with the validation of various assay panels through Dr. Andrew Wilson at the Center for Regenerative Medicine.

A1F envisions a future where it not only facilitates the sharing of biomaterials but also serves as a hub for collaboration, innovation, and the dissemination of open-source data and resources. By continually adding new biomaterials and enhancing the platform's capabilities, the Foundation can ensure that researchers have the tools they need to drive forward scientific discovery and improve human health.

CONFERENCES AND FORUMS

Generating awareness, identifying new areas of research interest, & establishing AIF’s credibility within the rare disease community as a resource for research, education, & advocacy

The Alpha-1 Foundation reaches thousands of physicians, academic researchers, and industry partners each year through exhibitions at major national and international medical conferences, where insights are shared into new technologies, research findings, and priority research areas.

Gordon L. Snider (GLS) Critical Issues Workshop Series

The GLS Critical Issues Workshop Series facilitates expert exchanges and answers important questions to stimulate additional research. Two recent workshops focused on pragmatic solutions to clinical trial endpoints for lung disease in Alpha-1 Antitrypsin Deficiency (AATD), alpha-1 antitrypsin (AAT) variant nomenclature, and Z variant heterozygosity. Participants discussed lessons learned from the naming and classification of other disease states, assessed the current landscape for nomenclature, and considered options for a simplified, rational approach to classifying and naming variants in the AAT gene so that research can be streamlined and advanced. The workshop also provided attendees with much to consider for developing trials that meet regulatory requirements and advance research in Alpha-1 lung disease. This included discussions on the importance of data sharing to validate endpoints and hypotheses. Both well-attended workshops included researchers, clinicians, government representatives, patients, donors, industry and biotech representatives, and academia.



AIF Staff exhibiting at ATS

Alpha-1 Foundation Exhibits

- European Respiratory Society (ERS) International Congress
- American Association for the Study of Liver Diseases (AASLD) Meeting
- American Association for Respiratory Care (AARC) Congress
- National Institutes of Health (NIH) Rare Disease Day
- American Thoracic Society (ATS) International Conference

LOOKING AHEAD: Conferences and Forums

The Alpha-1 Foundation’s mission is profoundly enriched by its commitment to hosting conferences that serve as pivotal gatherings for relevant stakeholders. These conferences stand as a cornerstone of AIF’s efforts, igniting enthusiasm within the field and solidifying its reputation as a leader within the rare disease community. Upcoming conferences include a GLS Workshop entitled “Experimental Models of Alpha-1 Antitrypsin Deficiency Lung and Liver Disease,” an AIF Investigators’ Meeting showcasing recently funded research findings and advances in the state of the cure, and a Global Research Conference in Lisbon, Portugal, to bring the worldwide Alpha-1 and research communities together to forge partnerships that will bring them even closer to finding a cure for Alpha-1.

EDUCATION & OUTREACH

Empowering the Alpha-1 community with educational resources and direct support

The more the Alpha-1 Foundation understands about Alpha-1, the better equipped it is to treat it. As it works toward a cure, empowering the Alpha-1 community remains a top priority.



AIF NATIONAL CONFERENCE

Bringing the Alpha-1 community together for education and awareness in the search for a cure

811 Total conference attendees in-person & virtually

75% were patients & family, a 10% increase over 2023

30% increase in attendance from 2023

37% were first-time attendees of the conference

As the largest annual gathering of the Alpha-1 community, the AIF 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-greet and Q&A sessions with Alpha-1 experts, and networking opportunities with other participants.

2024 attendees (both in-person and virtual) noted that their favorite session was "Emerging Therapies in Alpha-1," presented by companies with a vested interest in Alpha-1 therapies and treatments. This robust session outlined current and upcoming research on therapeutic development. Other well-attended topics included Alpha-1 101, the MZ Alpha, the Alpha-1 Research Panel, and Everyday Living as an Alpha.

A favorite session from the conference, the *Alpha Empowerment Hour*, featured four panelists who shared their personal Alpha-1 journeys as patients, parents, and advocates, highlighting the theme, "The Power is in You to Make a Difference."



IMPACT

Empowering patients to make informed decisions about their care & take part in Alpha-1 research

Patient First-Time Attendee Scholarship Program

In 2024, for the first time, the Alpha-1 Foundation provided 100 scholarships to Alpha-1 patients who had not previously attended an A1F National Conference in person due to financial constraints and who wanted to attend to learn about their condition and meet others living with Alpha-1.

“ The information we were able to receive and the in-person experience we were able to be part of were incredibly helpful to us in understanding this diagnosis. Meeting and talking with other Alphas and hearing their stories and journeys helped us to see this diagnosis in a more positive light. The presentations were fantastic from start to finish. Being able to ask a question and get an immediate answer was so helpful in trying to understand the complexities involved in being an Alpha. The conference was both informative and uplifting. Each and every person we encountered from the Alpha-1 Foundation, the presenters, doctors, industry, or Alphas themselves were so caring and genuine. We plan to attend again in 2025... and beyond!

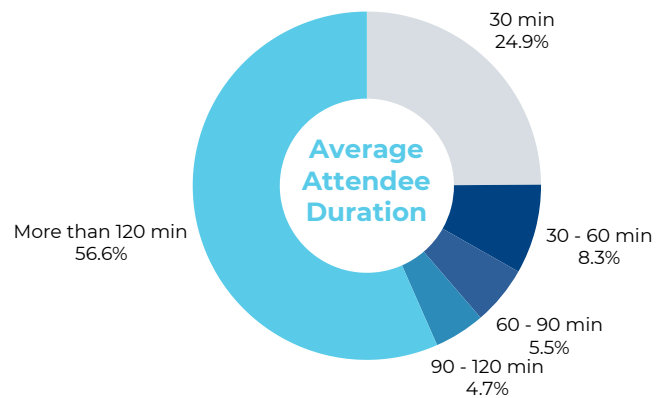
- First-time conference attendee & Alpha-1 patient

Virtual Conference Participation

The Virtual Environment was open for 30 days post-conference for on demand viewing. During that time, there were:

7,095 Content views & impressions

3,401 Webinar views



ALPHA-1 NATIONAL EDUCATION SERIES

Bringing patient education and awareness of Alpha-1 to a regional audience

1,040 total attendees in FY 2024

71% were patients & family members

31% were first-time attendees

The Alpha-1 National Education Series is comprised of Alpha-1 Foundation Education Days for Alphas, their families, and caregivers. 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 continues to use a virtual event platform, enabling those who are unable to attend an A1F Education Day in person to participate virtually, gain access to Alpha-1 information and resources, and connect with the Alpha-1 community.

Those who attend these events for the first time are offered tailored information based on their interests and interactions. This engagement tracking will help A1F continue to grow the community and build leaders and new volunteers to help further its mission.



Boston Alpha-1 Foundation Education Day in November



PROFESSIONAL EDUCATION

Filling gaps in education for healthcare providers and improving understanding of Alpha-1 within the medical community

The Alpha-1 Foundation is dedicated to advancing the professional development of healthcare practitioners through a robust array of continuing education opportunities. In collaboration with esteemed professional organizations, A1F offers continuing education credits covering a broad spectrum of crucial topics, including diagnosis, testing, augmentation therapy, and treatment. This commitment ensures that healthcare professionals are equipped with the latest knowledge and skills necessary to provide the highest standard of care to their patients.

To support the ongoing learning needs of healthcare practitioners, A1F provides a free online course designed to offer one contact hour of continuing education for registered nurses and nurse practitioners. The course content is developed to address critical areas of practice and provide valuable insights into effective Alpha-1 patient management.

In addition to online offerings, A1F actively seeks out and promotes opportunities for virtual and in-person grand rounds. These educational sessions are a platform for Clinical Resource Center physicians to present on Alpha-1 and discuss patient management and detection with their peers.



Respiratory Therapist Continuing Education

743+

medical providers have completed since inception

Continuing Medical Education (CME) program with the Cleveland Clinic

4,145 medical providers have taken the courses since last year

27% claimed continuing credits

41 internal medicine and other medical sub-specialties

19 countries represented

LOOKING AHEAD: Professional Education

The Alpha-1 Foundation’s focus remains on enhancing its educational initiatives and broadening its reach. By leveraging its partnerships, like the one with the Cleveland Clinic, and engaging with the medical community, it aims to ensure that the message of early diagnosis and effective patient care is deeply embedded within the healthcare landscape.

A1F continues to attend and exhibit at various medical conferences, allowing it to bring the Alpha-1 diagnosis and care message to the larger clinical audience. An application has been submitted to the American Thoracic Society to convene an Alpha-1 symposium at their 2025 conference. A1F is recommitted to participating in as many conferences as possible to ensure that the message of early diagnosis is immersed in the medical community.

EDUCATIONAL RESOURCES

The Alpha-1 Foundation offers a suite of educational resources designed to increase access and understanding, enhance community engagement, and promote best practices related to Alpha-1 testing, diagnosis, and care.



ALPHA-1-TO-ONE MAGAZINE

A seasonal magazine providing practical advice, patient stories, and pertinent news to the Alpha-1 community

32,500 copies disseminated per issue

3x issues published per year

The *Alpha-1-To-One* magazine is a valued resource for the Alpha-1 community; most readers identify as Alphas, family, and caregivers. Other audiences of the magazine include medical professionals, researchers, and donors.

In FY 2024, *Alpha-1-To-One* was distributed to the A1F mailing list as well as in bundles to CRCs and healthcare providers; each issue is posted to a digital publishing platform on the A1F website, where it was accessed by nearly 2,000 more visitors in FY 2024.

Each issue of *Alpha-1-To-One*, published tri-annually, includes Alpha-1 research updates, an introduction to Alpha-1 specialists and researchers, a recap of community outreach and advocacy efforts, and spotlights of A1F events throughout the year. Additional content and topics are determined by an editorial board consisting of patients, physicians, researchers, and other supporters, based on new and emerging needs in the Alpha-1 community. A must-read of each magazine is the “Ask the Alpha Doc” article, frequently asked questions answered by Alpha-1 experts.



Highlights in this year’s issues included:

- The 2023 Alpha-1 Foundation National Conference
- The launch of the new A1F website - alpha1.org
- Peg Iverson Commemorates 50 years since Alpha-1 Diagnosis

COMMUNITY CURRENTS E-NEWSLETTER

Monthly e-newsletter highlighting current A1F initiatives and opportunities for involvement

The Alpha-1 Foundation’s *Community Currents* monthly e-newsletter provides timely updates on upcoming programs and events, fundraising activities, and research efforts, such as the Alpha-1 Research Registry. This extension of monthly happenings in the Alpha-1 community partnered with a summary of activities provide easy ways for Alphas to get involved.

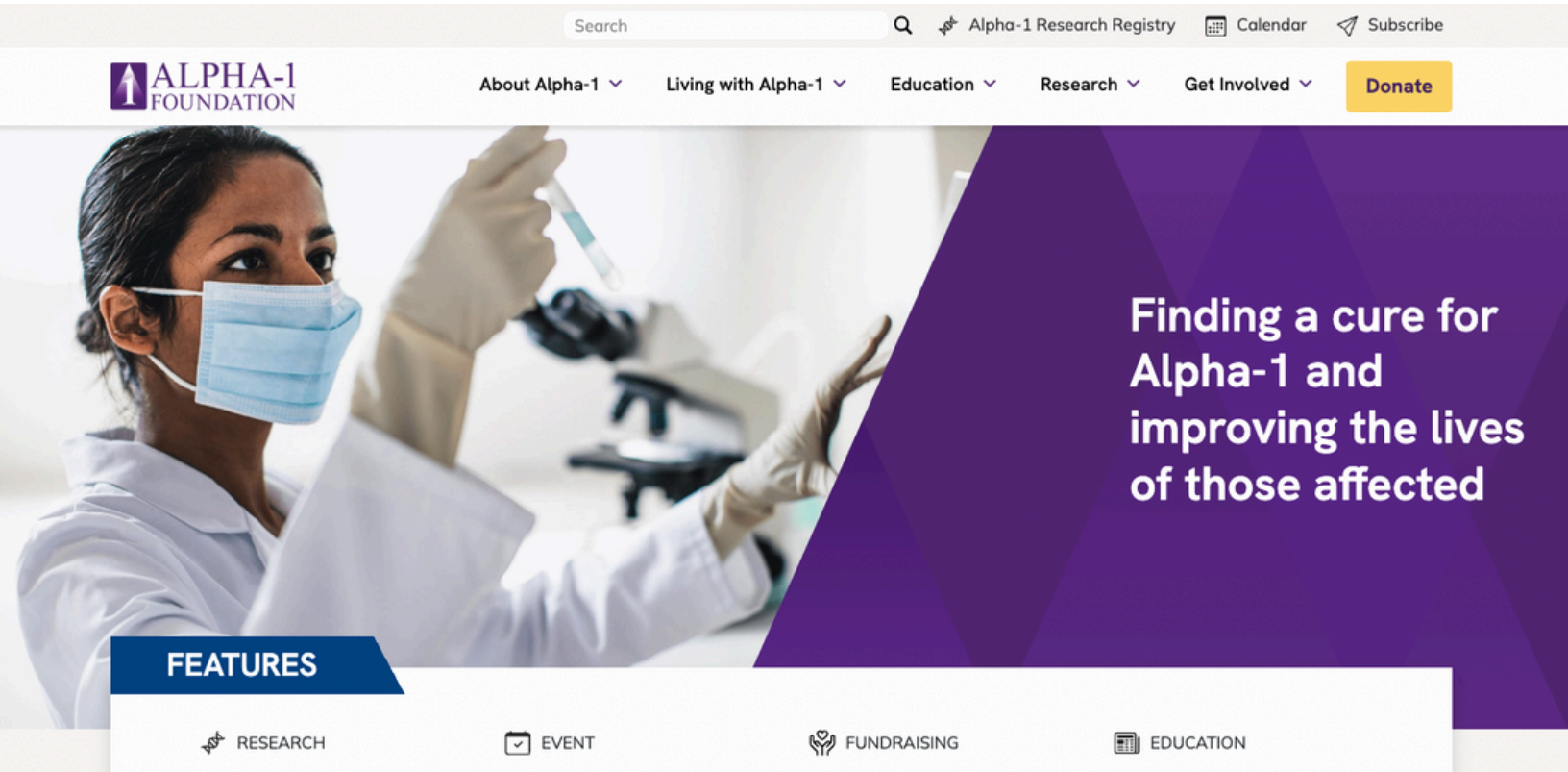
20,614 FY2024 subscribers

15% increase from 2023

ALPHA1.ORG

A clearinghouse for information on Alpha-1

The Alpha-1 Foundation website serves as a comprehensive hub for educational resources, community engagement, and up-to-date information for a diverse range of audiences connected to our mission. Whether for Alpha-1 patients, families, caregivers, healthcare providers, researchers, or industry partners, the website is crafted to provide valuable information and facilitate meaningful interactions.



Finding a cure for Alpha-1 and improving the lives of those affected

FEATURES

RESEARCH

EVENT

FUNDRAISING

EDUCATION

467K

Total website views

70,594

visits to homepage

30K

Liver Disease visits, 2X visits in 2023

Most Visited Pages

1. What is Alpha-1?
2. Liver Disease
3. Testing & Diagnosis
4. Lung Disease
5. Find an Alpha-1 Specialist

For patients and their families, our website is a crucial resource that offers a wealth of educational content tailored to their needs. Newly diagnosed individuals can access information that helps them understand their condition, explore treatment options, and manage their health journey. For those looking for an Alpha-1 specialist, the website offers links to physicians and expert Clinical Resource Centers, facilitating smooth and efficient connections between patients and the right healthcare

professionals. Healthcare providers visiting our website will find a range of resources designed to support their professional needs. For researchers our website provides valuable information on funding opportunities, research initiatives, and collaborative projects. We enhance this experience through the use of an email Welcome Series to new visitors to our site.

VIDEO LIBRARY

Alpha-1 related content—anywhere, anytime, on any device

63

New videos added in FY 2024

9,693

Total views up 74% from FY 2023

4,888

Unique viewers up 71% from FY 2023

1,624

Hours watched up 114% from FY2023

44

Countries accessed the library & watched videos

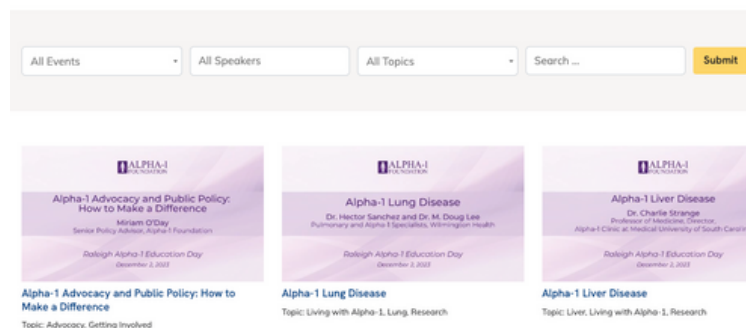
The newly revamped Video Library, accessible on the A1F website, includes presentations from A1F educational events, including Education Days and National Conferences. It is fully searchable by event, speaker, and topic as well as a type-in search box. Content is added throughout the year, with new videos promoted through e-mail, social media channels, and the A1F website. With presentations ranging from “Clinical Trials Explained” & “How a Therapy Comes to Market” to “Pulmonary Rehab Webinar: Exercises You Can Do at Home,” the video library offers useful content for everyone in the Alpha-1 community.

The Video Library has become a valuable resource for the global Alpha-1 community and is frequently used by Alpha-1 Support Groups as a tool to offer expert presentations at meetings.

Video Library Topics

- Advocacy
- Caregiving
- Children & Alpha-1
- Genetics
- Getting Involved
- Liver
- Living with Alpha-1
- Lung
- Nutrition
- Oxygen
- Pulmonary Rehab & Exercise
- Research
- Transplantation

Video Library



Looking Ahead: Video Library

The Alpha-1 Foundation monitors the educational needs of the community and is constantly adding and updating presentation topics as necessary. The E-Education presentations will continue to be used as a valuable tool for Support Group education. The library will continue to expand with the addition of all educational presentations that take place at A1F Education Days and the A1F National Conference.

Two new educational videos are currently in production. These updated whiteboard videos will contain lay level language on Alpha-1 101 and Clinical Trials. Understanding the basics of Alpha-1 is essential for any newly diagnosed Alpha. This video will provide easy to understand terminology and provide answers to many commonly asked questions. The Clinical Trials video will provide a broad overview of what clinical trials entail and how an Alpha-1 patient can take part in research.

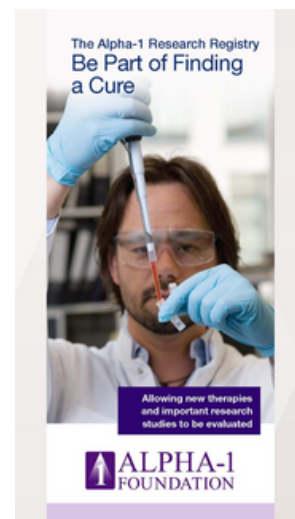
EDUCATIONAL BROCHURES

Medically validated brochures to help the Alpha-1 community stay informed

14,036 distributed in FY2024

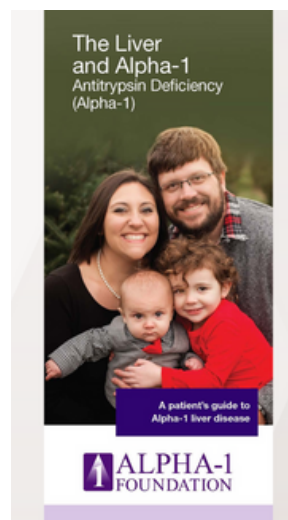
Educational brochures and materials provide validated medical information and are designed to increase knowledge and promote positive practices related to Alpha-1 testing, diagnosis and care among Alphas, their families, those at risk for Alpha-1 and healthcare professionals.

A1F is always looking for new ways to reach Alphas, increase community engagement, and inform patient care. With the recent redesign of the website, A1F is simultaneously building consistent, identifiable branding into digital and print materials to strengthen its identity. The goal is to provide innovative content packaged in a familiar form which includes a rebranding of all educational brochures.



Brochure Topics

- What is Alpha-1?
- The Liver and Alpha-1
- Am I an Alpha-1 Carrier?
- It's All in the Family: Family Testing
- A Guide for the Recently Diagnosed
- Healthcare Providers Guide
- How to Find an Alpha Doc
- Alpha-1 Research Registry: Be Part of Finding a Cure
- Genetic Counseling Program for patients
- Genetic Counseling Program for HCPs
- Research Opportunities

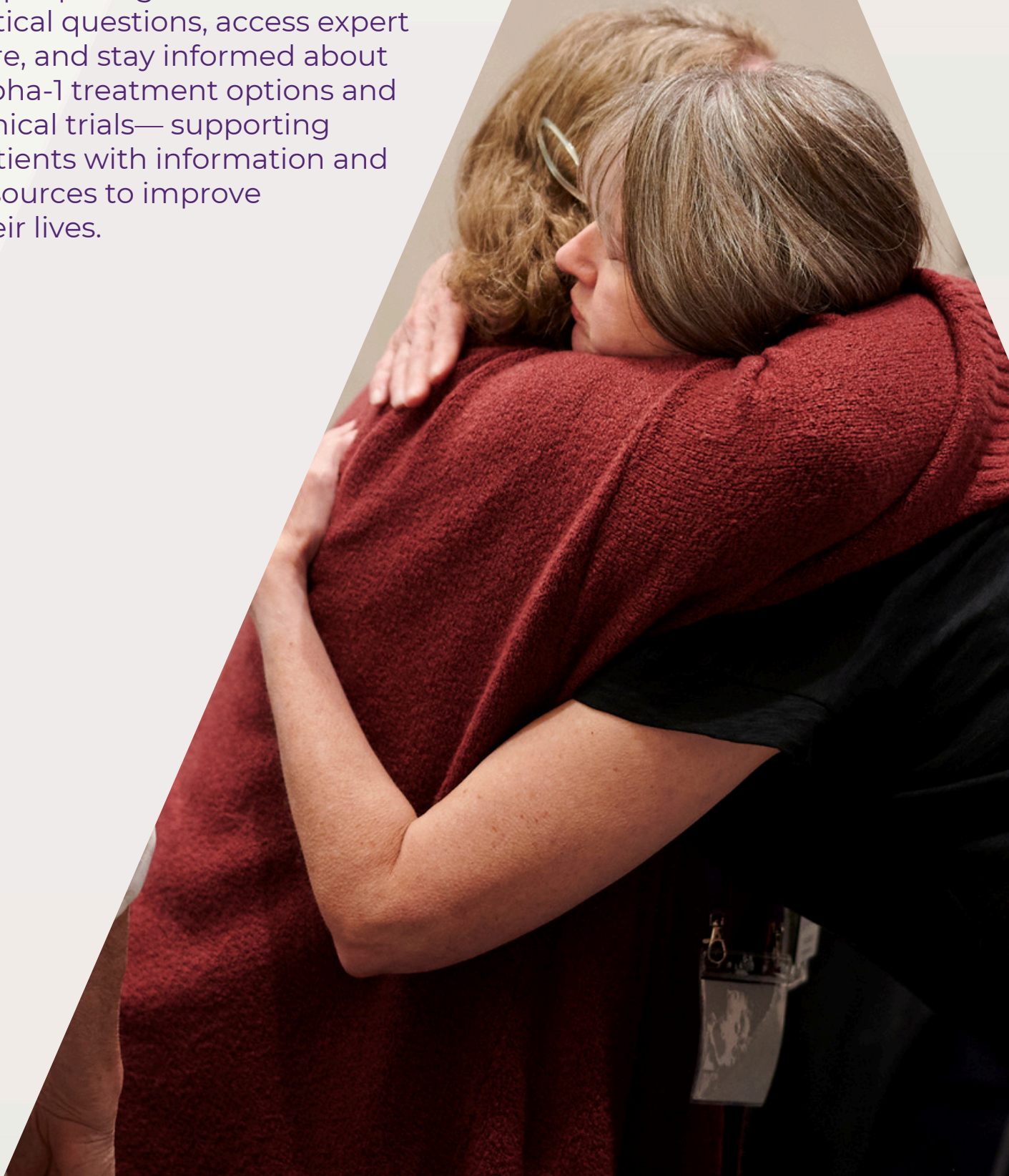


LOOKING AHEAD: Educational Brochures

The A1F Educational Brochures are going through an entire re-brand to be consistent with the new A1F website and materials. As part of this re-brand, content is being reviewed for updates and consistency. New brochures will be available as part of A1F's 30th Anniversary launch in 2025.

PATIENT OUTREACH

Alpha-1 Foundation programs help Alphas get answers to critical questions, access expert care, and stay informed about Alpha-1 treatment options and clinical trials— supporting patients with information and resources to improve their lives.



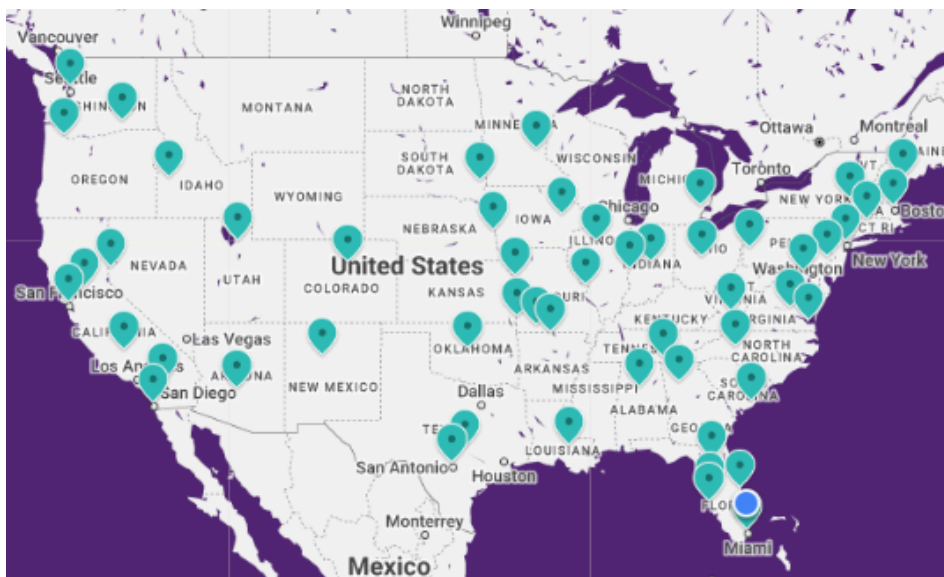
PATIENT SUPPORT NETWORK

Providing support and education to Alphas and family members and creating awareness of Alpha-1 in local communities

The Patient Support Network is comprised of Alpha-1 Support Groups and direct patient support programs including the Patient Information Line, Peer Guide Program, Emergency Relief Fund, CRC Access Program, and Oxygen Travel Assistance Program.

The Patient Support Network is designed to foster an enhanced, healthy sense of identity and connection with the larger Alpha-1 community while extending the Alpha-1 Foundation's mission to local communities.

The Alpha-1 Foundation has a nationwide network of Alpha-1 Support Groups led by Alphas and caregivers. Support Group Leaders receive education and training throughout the year, with many serving as advocacy ambassadors in regions with a local CRC. Support Group Leaders work with physicians and CRC staff to organize coordinated community education and awareness events and assist in the planning of Alpha-1 Education Days.



20% Increase in SG Meetings from FY2023 to FY2024

In FY2024, the Alpha-1 Support Group Network was revamped to establish six regions, allowing AIF to conduct regional Support Group meetings in conjunction with AIF Education Days. This new structure provides a robust program with expert speaker-based content as part of Support Group meetings and broadens in-person reach, targeting populations of patients that are either underserved or located in areas with large cohorts of patients. This approach allows local Support Group Leaders to engage with new patients and families.

Regional Support Group Meetings held in:

- Raleigh, NC
- Sacramento, CA
- Seattle, WA

AIF Support Group Leaders at the 2024 AIF National Conference in Miami, FL



DIRECT PATIENT SUPPORT

Patient Information Line

The Patient Information Line helps connect Alphas and family members to the Alpha-1 community to help them better manage and understand their diagnosis. In FY 2024, it fielded over 730 calls, helping callers locate specialists, access support, and gain information on a range of topics, including managing Alpha-1, COVID-19 risks, and understanding Alpha-1 testing.

Peer Guide Program

The Peer Guide Program connects a newly diagnosed Alpha, or someone whose life has been affected by Alpha-1 (Peer), to another Alpha (Guide) with a similar set of circumstances. AIF currently has over 50 peer guides available, ranging in experiences from newly diagnosed, transplantation, self-infusion, supplemental oxygen, parents of Alphas and caregivers.

Emergency Relief Fund

The Emergency Relief Fund is a collaboration between the Alpha-1 Foundation and the National Organization for Rare Disorders (NORD) that provides financial assistance to Alphas and their families. Support of up to \$500 is available to pay for basic and urgent needs not covered by insurance premiums or co-payments.

CRC Access Program

The CRC Access Program provides Alphas a one-time travel stipend of up to \$500 to visit their nearest Clinical Resource Center for the first visit. This program supports access to Alpha-1 specialists who provide best-practice health management strategies and discuss augmentation therapy. Program utilization increased in FY2024 with the return to in-person events and expansion of community engagement opportunities with their local CRCs.

Oxygen Travel Assistance Program

The Oxygen Travel Assistance Program provides Alphas access to supplemental oxygen to help with travel for medical emergencies, transplant evaluation, visits to CRCs, and Alpha-1 educational events. In FY 2024, the program provided 29 Alphas in need supplemental oxygen systems.

Genetic Counseling Services

AIF partners with the University of Florida to provide free genetic counseling services to Alphas, their family members, healthcare providers, and other individuals considering testing or in need of support. Genetic counseling services are important in helping patients understand their genetic risk for developing lung and liver disease, as well as communicating that information to family members. Trained experts handle calls for personalized genetic counseling, interpret results from ACT Study participants, and make referrals based on specific needs. Many callers are newly diagnosed Alphas seeking initial information and recommendations.



Patient at the AIF National Conference

LOOKING AHEAD: Patient Support Network

A full-day Support Group Leader training is scheduled in October 2024 to train Support Group Leaders on best practices, communication skills, AIF programs and services, and other timely topics.

Recruitment of new Support Group Leaders is ongoing, with five new leaders currently in training. AIF is consistently looking to recruit new leaders for underserved regions. There is a process in place to vet and train individuals who have been recommended as new Support Group Leaders.

ALPHA-1 KIDS PROGRAM

Resources for families of Alpha-1 infants, children, teens, and young adults transitioning to independent care

Recognizing the unique needs of Alpha-1 Kids and their families, A1F is intensifying its efforts to provide targeted support and pinpoint existing gaps and opportunities for program expansion. A1F's commitment is underscored by the establishment of specialized resources, like the Parents of Alpha-1 Kids (PAK) Virtual Support Group, led by Alpha-1 moms, providing comprehensive assistance to families navigating their child's healthcare journey.

A1F provides scholarships for parents to attend the A1F National Conference, a dedicated track with sessions relating to the care of Alpha-1 children, young adults, and their families, and an Alpha-1 Kids room with activities so parents can attend the sessions. The conference provides families with the ability to network with other affected families, access to leading physicians, and the opportunity to learn the latest research and resources from renowned pediatric liver doctors.

The addition of pediatric liver specialists to the CRC network has enhanced access to specialized care for Alpha-1 children facing liver health challenges. Additionally, A1F is revitalizing its engagement with the North American Society for Pediatric Gastroenterology, Hepatology & Nutrition (NASPGHAN) by leveraging the expertise of leading professionals, sharing the latest Alpha-1 research and developments, and fostering a collaborative community.

A1F is forming dedicated focus groups for parents of Alpha-1 children and young adults. These groups serve as platforms for collaborative discussions aimed at improving patient care and support. Notably, A1F recently facilitated a transformative opportunity for parents to attend a BFC Training, equipping them with essential fundraising skills during their visit to Celtic Connection, a signature fundraiser for A1F.

A1F is committed to expanding the Alpha-1 Kids program and ensuring that every child with Alpha-1 receives the best possible care and support. It strives to empower Alpha-1 families and advance medical knowledge in pediatric liver health, fostering a brighter future for young Alphas.



Alpha-1 Patient family at A1F National Conference



Alpha-1 Kids Panel at A1F National Conference



Parents of Alpha-1 children at the BFC Training



EDUCATIONAL SCHOLARSHIPS

Funding educational opportunities for those impacted by Alpha-1

AIF awards educational scholarships annually to Alphas and immediate family members seeking to further their education at an accredited university, college, community college, or technical institute. For the second year, applications increased exponentially with 50% more submissions in FY 2024. 10 Alpha-1 Educational Scholarships were awarded, totaling \$25,000.



James Quill Memorial Scholarships



Elizabeth Hipperling, from Sound Beach, NY, is a sophomore at the University Of New Haven majoring in Forensic Science.

Grace Johnson, from St. John's, FL, is a freshman at Florida State University majoring in Marketing.



John W. Walsh, III (Jack) Memorial Scholarship



Kelly Grant, from Klamath Falls, OR, is a sophomore at Portland Community College majoring in Physics.

E. Lou Glenn Memorial Scholarship

Eliana Ainsa, from Bluffdale, UT, is a freshman at the Brigham Young University-Idaho majoring in Exercise Kinesiology.



Robert J. Haggerty Memorial Scholarship



Stella Guthman, from Omaha, NE, is a junior at the University of Nebraska-Omaha majoring in Graphic Design.

Terry L. Young Memorial Scholarship

Tara Mulholland, from Gaithersburg, MD, is a sophomore at Pennsylvania State University majoring in Supply Chain Management.



Peter Smith Scholarships



Joseph Reidy, from Waldwick, NJ, is a junior at Rochester Institute of Technology majoring in Cybersecurity.

Hannah Cathrae from Boise, ID, is a sophomore at Idaho State University majoring in Nursing.



Gabrielle Salzman, from Vance, AL, is a freshman at the University of Alabama majoring in Forensic Science and Laboratory Sciences.

Tiffany Nutter, from Richmond Hill, GA, is a junior at Georgia Southern University majoring in Psychiatric Mental Health Nurse Practitioner.



ALPHA-1 STRATEGIC PLANNING

Prioritizing the evolving needs of the Alpha-1 community for maximum impact

For the Alpha-1 Foundation, developing a comprehensive 3-5 year strategic plan is essential to achieve sustainable impact and long-term success. A well-structured strategic plan provides clear direction, ensures alignment with A1F’s mission, and helps prioritize resources effectively as the Foundation addresses the evolving needs of the Alpha-1 community.

First, A1F interviewed and engaged with key opinion leaders (KOLs)—such as healthcare professionals, researchers, patients, and advocacy leaders. They provided valuable insights into emerging trends, innovative therapies, and potential partnerships. These interviews allow A1F to align its strategies with broader healthcare advancements and ensure that the programs offered are relevant, evidence-based, and forward-looking.

Then, A1F delved into a patient segmentation study. This study helped the Foundation identify and categorize different patient groups based on their specific needs, conditions, and experiences. This study ensures that A1F tailors its services, outreach efforts, and programs to meet the distinct needs of each patient segment. By understanding variations in demographics, treatment access, and health outcomes, A1F can develop targeted interventions that enhance programs and services and patient care.

Finally, A1F just recently conducted a community market survey as a critical tool for gathering insights and understanding the broader Alpha-1 community’s perceptions, needs, and challenges. This survey will identify any gaps in services, highlight areas of unmet need, and gather community input on future initiatives. It will also help assess community awareness of A1F’s mission and its impact, informing strategic decisions around outreach and public engagement.

A well-developed strategic plan will offer several benefits to A1F. It establishes clear organizational goals, allowing A1F leadership to align resources, efforts, and staff to focus on mission-driven activities. By incorporating data from patient segmentation, KOL interviews, and the community survey, the plan is grounded in real-world insights, ensuring that strategies are responsive to actual needs. The plan will also help A1F to forecast future challenges, secure funding, and allocate resources efficiently to support long-term growth. Regular input from patients, community members, and healthcare leaders creates stronger relationships and ensures that all stakeholders of the Alpha-1 community feel valued and heard in the decision-making process.



Patients at the A1F National Conference



Patients at the A1F National Conference



Patients at the A1F National Conference

“

It's an exciting time in the history of Alpha-1 treatment and care. As the leading Alpha-1 organization, it's imperative to understand the needs and wants of our collective community so we can develop, adjust and enhance our business strategy to achieve our goals. To that end, we embarked on the largest Alpha-1 Community Market Survey in the history of the condition and the resulting data will provide us the direction and insight we need to remain the leader and deliver on our mission.

- Scott Santarella, CEO, Alpha-1 Foundation

”

AWARENESS & FUNDRAISING

Strengthening the Alpha-1 community

The Alpha-1 Foundation accomplishes its mission with help from Alphas, those impacted by Alpha-1, industry partners, researchers, and investigators.

AIF's progress depends on building the community and encouraging every person to take part in finding a cure for Alpha-1.



BUILDING FRIENDS FOR A CURE (BFC)

Community fundraising programs designed to increase awareness and promote research

Over the past year, the Alpha-1 Foundation has witnessed an incredible outpouring of support from Alphas, their families, friends, researchers, industry professionals, and the medical community. The involvement of such a diverse group highlights the widespread commitment to the Foundation’s cause and underscores the importance of community in achieving its goals.

Notable successes have marked the Foundation’s fundraising activities over the past year, thanks to the enthusiastic participation of the Alpha-1 community. The Foundation has utilized various platforms and strategies to engage the community and drive contributions. Successful social network campaigns have played a pivotal role in spreading awareness and encouraging donations, leveraging the power of digital communication to reach a broad audience. Online fundraising platforms have also proven effective, providing a convenient way for individuals to contribute and support the cause.

In addition to digital efforts, A1F has organized and participated in a variety of in-person events that have significantly contributed to its fundraising success. Events such as the Celtic Connection and Alpha-1 Riding for a Reason have not only raised substantial funds but also fostered a sense of community and shared purpose. Most notably, the A1F Virtual Walk took place during Alpha-1 Awareness Month, with over 885 participants on 75 teams from around the country.

BFC is a series of community fundraising events supporting Alpha-1 research:

- Alpha-1 Riding for a Reason
- Escape to the Cape (E2C)
- Alpha-1 Foundation Virtual Walks
- Celtic Connection
- Extreme Sports (hiking, mountain climbing)
- Local Fun Events (Cones for the Cure)
- Youth Events (Baseball Tournaments)



Escape to the Cape in Cape Cod, MA



Baseball Tournament in Cooperstown, NY



Celtic Connection in Boston, MA



Cones for a Cure in Omaha, NE

LOOKING AHEAD: Building Friends for a Cure (BFC)

A1F’s focus remains on expanding and deepening its engagement with the Alpha-1 community. Plans are in place to hold in-person training sessions with the community, along with online beginner sessions. The Foundation is committed to building on the successes it has achieved and exploring new ways to involve more people in its efforts. By leveraging contemporary technology and strong branding, A1F aims to reach an even wider audience and provide diverse opportunities for involvement.

ALPHA-1 AWARENESS

Annual campaigns to facilitate community support and improved approaches to care

November is Alpha-1 Awareness Month, and during this time, the Alpha-1 Foundation (A1F) launches the #Alpha1Awareness campaign to increase awareness of Alpha-1 Antitrypsin Deficiency (Alpha-1) while also raising funds to find a cure and improve the lives of those affected by this rare condition.

Last year's theme was: "Make Your Move for Alpha-1." Small decisions can make a big impact. Whether the move was posting about Alpha-1 on social media, creating a team and fundraising for the Alpha-1 Foundation Virtual Walk, or signing up for the Alpha-1 Research Registry to help move research forward—it made an impact on the Alpha-1 community.



A1F Grant Awards Reception at ATS in San Diego, CA

SPECIAL EVENTS

Celebrating the Alpha-1 community and supporting research

Special Events are opportunities to expand the A1F donor and volunteer base and raise funds for programs and research. Over 300 guests attended the following events in FY 2024:

Alpha-1 Foundation Grant Awards Receptions

Alphas, physicians, researchers, and industry partners attend these receptions to recognize newly awarded grantees for their work and dedication in Alpha-1 and their significant role in the search for a cure. One event is held during the American Thoracic Society's (ATS) annual conference for awardees of lung-focused grants, while another event is held during the American Association for the Study of Liver Diseases' (AASLD) annual conference for awardees of liver-focused grants.

Celebration of Life

This annual golf tournament and reception benefit transformative Alpha-1 research and advance the mission to find a cure. Attendees include Alphas, family members, senior leadership, and industry representatives.



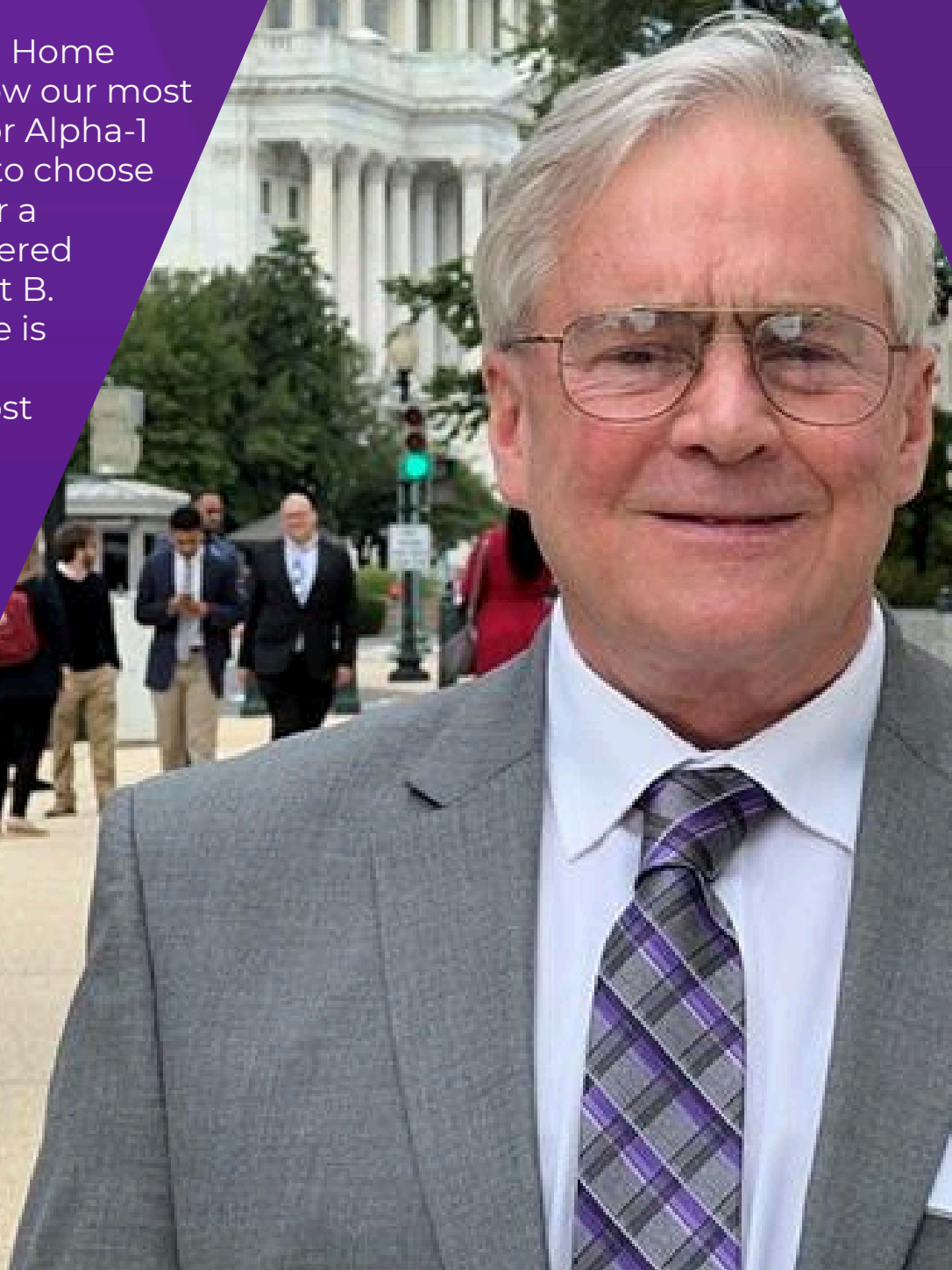
Bettina B. Irvine Invitational Classic in Greenwich, CT

PUBLIC POLICY & ADVOCACY

Generating change to improve outcomes for Alpha-1 patients

“The John W. Walsh Home Infusion Act will allow our most compromised senior Alpha-1 patients the ability to choose home infusions over a hospital setting covered under Medicare part B. The standard of care is home infusions not only because it is cost effective, but it has better outcomes for the patients.”

- Alpha-1 Advocate
Dan Grimm



ALPHA-1 ADVOCACY

Advocating for the Alpha-1 community by monitoring and influencing legislative and regulatory issues

The A1F advocacy program targets policymakers, regulatory agencies, the public, and the media with campaigns to promote research and raise awareness of the challenges faced by Alpha-1 patients and care providers. Public policy work is carried out in part by the grassroots patient community as well as by key stakeholders, staff, and the extensive network of healthcare professionals. Alpha-1 advocates represent A1F at forums and lobby days each year. A1F provides training, guidance, and resources to empower, educate, and mobilize Alphas to convey the urgency of the cause.

Advocates urge members of Congress and federal agencies to prioritize the needs of Alphas and those affected by rare and chronic conditions, focusing on eliminating barriers to healthcare coverage, advocating for telehealth and home infusion, ensuring access to essential medications and therapies, raising awareness of patient needs, and improving patient detection and outcomes.

Reps. María Elvira Salazar (R-FL) and Chellie Pingree (D-ME), original co-sponsors, continue to support the John W. Walsh Alpha-1 Home Infusion Act (H.R.4438). If passed in this Congressional term, certain Medicare beneficiaries living with Alpha-1 Antitrypsin Deficiency would be able to receive essential augmentation infusions at home.

Policy priorities include:

- Medical research
- Therapeutic development
- Screening and detection
- Access to care
- Federal and state funding
- Blood product safety
- Education and awareness



Advocacy Training Workshop in Raleigh, NC

Institutional, Agency, and Coalition Partners

A1F works strategically with federal agencies, memberships, and coalitions—focusing on the issues that matter most to Alphas—to inform legislators of the burdens of the patient community and to advance its public policy agenda for the benefit of the Alpha-1 community.

- **U.S. Food and Drug Administration (FDA):** A1F stewards a relationship with the FDA at the highest level and regularly collaborates with the Agency regarding best practices for clinical trials and guidance specific to Alpha-1 therapies.
- **Critical Path Institute (C-Path):** In partnership with C-Path, A1F provides input and data to navigate regulatory pathways for clinical trial design and medical product development.
- **National Institutes of Health (NIH):** Recommendations by the NIH inform much of A1F's work, including data collection and research through the Alpha-1 Research Registry.

OUR PARTNERS, MEMBERSHIPS AND COALITIONS



LOOKING AHEAD: Alpha-1 Advocacy

Currently with 58 cosponsors, The John W. Walsh Home Infusion Act (H.R. 4438) continues to engage policymakers and raise awareness of Alpha-1. Alpha advocates are working diligently with legislators to meet the needs of the Alpha-1 community. Advocates will continue to have meetings throughout the year until the new term, both in-person and virtually, with the representatives to encourage their support of the bill.

A1F is collaborating with Plasma Protein Therapeutics Association (PPTA) for the 32nd annual PPTA Plasma Protein Forum titled *“Taking Steps Towards a Better Tomorrow”* on November 19-20, 2024 in Washington, D.C.. This annual event brings industry, policymakers, health care providers, scientists, and patients together to learn more about and discuss key developments and issues. A1F also partners annually with the Immune Deficiency Foundation (IDF) on the Plasma Awareness Campaign that takes place in October.

A1F provides constant educational updates to the community on changes to Medicare Part D as a result of the Inflation Reduction Act, Medicare oxygen payment reform, co-Pay accumulators and access to home based pulmonary rehab. Always advocating for the patient, A1F signs on with organizations to support decisions being made on behalf of patients across the nation each and every day. The annual Open Enrollment Webinar will take place in October 2024 and is geared specifically to Alphas. A1F partners with NORD to provide the most up to date information that applies to rare disease patients and their choices during the 2025 open enrollment.

A1F has acquired a new grassroots advocacy platform, *MUSTER* an online resource to empower advocates and supporters to have their voice heard and ensure their message resonates loud and clear. The software streamlines engagement between supporters and policymakers with tools to drive engagement and deliver public policy results. It includes embeddable action forms, one click advocacy actions, exportable in-depth analytics and reporting and petition campaigns.

ALPHA-1 GLOBAL

A communications platform for the global Alpha-1 community

Alpha-1 resources and educational materials are available in a variety of languages through Alpha-1 Global. Alpha-1 organizations and individuals connect to share ideas, strategies, and tools on three main topics: awareness, detection, and access to care. The Foundation also provides access to information on the latest Alpha-1 developments around the world as well as educational seminars.

The Alpha-1 Foundation worked with our international patient colleagues and the European Alliance to leverage the recognition of November Awareness Month in the U.S. and included patients and key stakeholders around the world to raise awareness of Alpha-1. The Foundation also participated in the European Union Parliamentary Proceedings on Alpha-1.



LOOKING AHEAD: Alpha-1 Global

Close relationships across international patient, clinician and scientific communities are aimed to increase awareness, encourage earlier diagnosis and family testing, promote disease management, and enhance participation in research. The Alpha-1 Foundation continues to develop avenues in which the global community can collaborate in the most effective manner.

The Alpha-1 Foundation is strengthening its relationship with EARCO through the creation of shared initiatives and plans to expand its efforts with the strong partnership it has built with researchers and patients. Strategic meetings will also be held in collaboration with the European Association for the Study of the Liver (EASL). This initiative will address specific challenges and advancements related to Alpha-1 liver disease. The Foundation’s efforts extend to South America, where it plans to enhance its presence in the region and provide valuable resources to individuals and families affected by Alpha-1.

AIF will host the 2025 Global Patient Congress and International Research Conference in Lisbon, Portugal, to bring the entire Alpha-1 community together (patients, caregivers, physicians, researchers, and corporate partners) to promote engagement and collaboration across the board. This gathering of the Alpha-1 community provides educational resources, social interaction and networking opportunities, and access to key partners. Attendees will have access to educational programs presented by international experts and leaders in the Alpha-1 field.





The Alpha-1 Foundation (A1F) 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.



@Alpha1Foundation



@Alpha1Foundation



@AlphaFriend



@Alpha-1 Foundation

ALPHA1.ORG

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