

2023



PROGRAM REPORT



www.alpha1.org

Our Mission

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

The Alpha-1 Foundation is dedicated to finding a cure for Alpha-1 Antitrypsin Deficiency (Alpha-1) and is tirelessly improving patient care along the way. Our initiatives in four main areas—Research, Education/Outreach, Awareness/Fundraising, and Public Policy/Advocacy—are paving the way for important discoveries, new therapies, and increased awareness and understanding of this rare genetic condition.

We focus on Alpha-1 at its earliest stages by supporting programs that push the boundaries of existing data, detection, and patient support. Our most recent accomplishments and non-profit accreditations reflect our unwavering commitment to medical research at its finest: we have invested over \$92 million to support Alpha-1 research and programs at 129 institutions in North America, Europe, South America, the Middle East, and Australia. These innovative, impactful projects have built upon the solid infrastructure that the Foundation has created for promoting ongoing research and the development of new therapies. We have also fostered collaborations with investigators throughout the United States and Europe, working closely with the pharmaceutical industry, the National Institutes of Health (NIH), the U.S. Food and Drug Administration (FDA), and Alpha-1 patients and families to push Alpha-1 research forward.

Our work would not be possible without our dynamic community of volunteers, researchers, clinicians, partners, and donors—all of whom inspire and empower us every day in our mission to raise awareness and deliver results for those affected by Alpha-1. We are incredibly grateful to our global network of specialists, which spans multiple industries, areas of research, and academic expertise. It is thanks to these innovative and patient-centric groups that we are driving real, positive change.

We sincerely thank you for your support as we continue to improve the treatment, quality of life, and outcomes for all Alphas while working toward a cure.



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A man with dark hair, wearing a grey lab coat over a red shirt, is focused on a task in a laboratory. He is wearing blue nitrile gloves and is holding a small, clear vial or pipette tip. The background is slightly blurred, showing laboratory equipment and shelves. The overall tone of the image is professional and scientific, with a blue and purple color overlay.

RESEARCH & SCIENCE

Accelerating research toward a cure for Alpha-1

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

Alpha-1 Research Registry

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

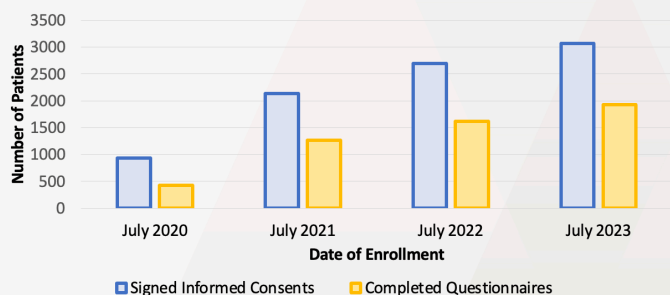
The Alpha-1 Research Registry, comprised of both patient-supported data and clinical data, has become an invaluable resource for the Alpha-1 community. Since its update in 2019, it has served as a solution to one of the most significant barriers slowing Alpha-1 research – the difficulty locating patients living with or impacted by this rare condition to participate in research studies. The Alpha-1 Research Registry plays a pivotal role in the Alpha-1 Biomarkers Consortium (A1BC) Study and is the foundation of data collection for the Therapeutic Development Network (TDN).

We recruited over **365** Alphas and family members to the Alpha-1 Research Registry in FY 2023, bringing the total number of Registry participants to **3,078**.

Abstracts Accepted for Poster or Presentation

- 2022 European Respiratory Society (ERS)
- 2022 National Organization of Rare Disorders (NORD) Breakthrough Summit
- 2023 Rare Disease Day National Institutes of Health (NIH)
- 2023 American Thoracic Society (ATS) International Conference

Alpha-1 Research Registry Growth



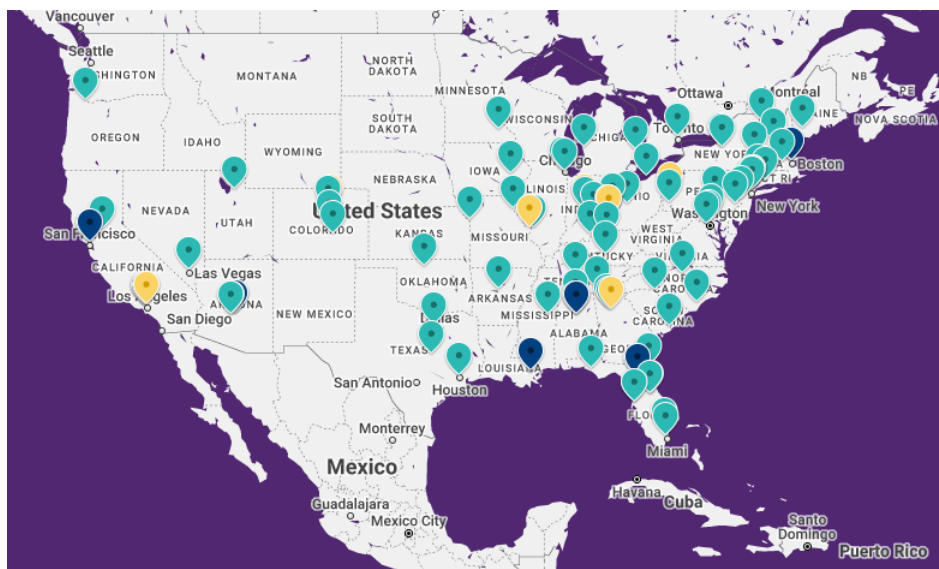
SPOTLIGHT: Looking Ahead

Alpha-1 Research Registry

There is a vital need to help match individuals with Alpha-1 to clinical studies in order to accelerate therapeutic development. We have created the key infrastructure for our efforts within the Alpha-1 Research Registry which has enabled the Foundation to perform targeted screening of potential participants to qualify them for studies. The functionality of our recruitment process has provided innovative and impactful solutions to clinical trials as we have seen early success in the response from Alpha-1 Registry participants with 75% interested in participating in our first screening project for clinical trial recruitment.

Clinical Resource Centers (CRCs)

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



**An Expansive
Network of
Alpha-1 Specialists
and Research
Institutions**

83

CRC Sites in the
U.S. and Canada

98

CRC Physicians

Designated Clinical Resource Centers (CRCs) provide comprehensive care to Alphas, including specialized care for lung disease and liver disease. CRCs play a critical role in helping the Foundation recruit patients for the Alpha-1 Research Registry. In addition, CRCs provide patients 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.

We are excited to announce our 7 newest Pediatric CRCs:

Cincinnati Children's Hospital Medical Center

Cincinnati, Ohio

Akihiro Asai, MD, PhD

UPMC Children's Hospital of Pittsburgh

Pittsburgh, Pennsylvania

Simon Horslen, MD

Cincinnati Children's Hospital Medical Center

Cincinnati, Ohio

Kathleen Campbell, MD

Children's Hospital Los Angeles

Los Angeles, California

Rohit Kohli, MD, MS

**Emory University School of Medicine,
Children's Healthcare of Atlanta**

Atlanta, Georgia

Nitika Gupta, MD

**Riley Children's Health,
Indiana University Health**

Indianapolis, Indiana

Jean Molleston, MD

Children's Hospital Los Angeles

Los Angeles, California

Emily Gillet, MD

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 clinical trial platform through which leading Alpha-1 specialists, researchers, and biopharma partners work to improve trial recruitment and design advanced patient-focused drug development programs. The TDN leverages the Alpha-1 Research Registry and our extensive network of CRCs to help industry partners with clinical trial recruitment and site selection.



SPOTLIGHT: **Looking Ahead**

CRCs & the TDN

The goal to provide the community with easy access to Alpha-1 specialists remains a strong commitment of the Foundation. Keeping the CRCs connected to the patients, the Foundation and the larger Alpha-1 community allows for a cohesive approach to engagement. By revitalizing the structure of the CRCs, ensuring a seamless integration with the TDN, we can ensure that the patient is at the forefront of clinical care.

As the TDN progresses with site engagement and clinical trial readiness, it is imperative that its team is well-equipped to keep driving the program towards success. This highly skilled leadership team consists of Dr. Stephen Rennard, Dr. Michael Campos and Alicia Sable-Hunt, RN, MBA, CCRA, ACRP-PM.

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 **\$2 million** in peer-reviewed research grants in FY 2023. These grants support innovative research spanning the spectrum of scientific discovery.

World's #1 Funder of Alpha-1 Research Support

- We've invested over \$92 million in Alpha-1 Research
- We've funded 129 institutions 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.

2022-2023 Grant Recipients

Research Grants

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.

Adel El Boueiz, MD

Brigham and Women's Hospital
Imaging and Multiomics Analyses of Emphysema Patterns in MM and MZ Smokers

Bibke Gooptu, MD, PhD

University of Leicester
Structural Studies of Misfolded and Polymeric Alpha-1 Antitrypsin in ERAD and ERLAD

James Irving, PhD

University College London
Rational Design of Better Diagnostic Reagents

Lela Lackey, PhD

Clemson University
Misregulation of Polyadenylation During Stress Contributes to A1AT Deficiency Phenotypes

Jungman Lee, PhD

University of Florida
Mechanisms of α -Defensins Mediated Bacterial Infection in AATD Individuals

Nunzia Pastore, PhD

Fondazione Telethon ETS
Revealing the Role of Lysosomes in the Pathology of the AATD-Related Liver Disease

Carmine Settembre, PhD

Fondazione Telethon ETS
Exploring the Role of FAM134B-MEDIATED ERPHAGY in Alpha-1 Antitrypsin Deficiency

Richard Sifers, PhD

Baylor College of Medicine
Identification of ESLD Prognostic Indicators

Pilot and Feasibility Grants

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.

Ariel Curiale, PhD

Brigham and Women's Hospital

AI Approaches to Define Emphysema Progression Risk in PiMZ and PiMS Heterozygous Subjects

Emer Reeves, PhD

Royal College of Surgeons in Ireland

Exploring Pro-Resolving Lipid Complexes to Realize the Full Therapeutic Impact of Alpha-1



Monica Goldklang, MD

Columbia University Irving School of Medicine

Alterations in inflammation and proteases during acute exacerbations of COPD in AATD

Ab Rees Fast Track Grants

The Ab Rees Fast Track Grants were awarded for the first time to four awardees to fund the preliminary work needed to apply for ancillary funding from the NIH or the Alpha-1 Foundation for the Alpha-1 Biomarker Consortium Study (A1BC). The A1BC study, founded by the NIH, was created to identify and characterize biomarkers in Alpha-1 that are needed to advance a cure for this disease.



Igor Barjaktarevic, MD, PhD

UCLA David Geffen School of Medicine

Exploring the role of Nasal Transcriptome Bioprofiling A1AT Deficiency



Karina Serban, MD

*National Jewish Health, Denver

Longitudinal biomarker study of disease-upper airway and plasma metabolomics



J. Michael Wells, MD

University of Alabama at Birmingham

Molecular Profiling AATD Respiratory Specimens: A Pilot

Alpha-1 Coded Testing (ACT) Study & Detection

A research study investigating perceived risks and benefits of genetic testing in Alpha-1 families, disease-specific risks, and co-morbidities for Alpha-1

The Alpha-1 Coded Testing (ACT) Study is a confidential testing program aimed at those who are at risk of Alpha-1. The ACT Study provides an opportunity for individuals and their family members to be tested for Alpha-1 in the privacy of their homes, without concern for health and life insurance issues associated with a diagnosed pre-existing condition.

The ACT Study also offers Alpha-1 families the opportunity to be part of research aimed at better understanding population demographics, testing attitudes and behaviors, and clinical outcomes among those at risk for Alpha-1. The study has already yielded valuable insights—**4.8%** of those tested were diagnosed ZZ Alphas, and **50%** of those tested were identified as carriers of Alpha-1.



The ACT Study has enrolled—and screened—almost 40,000 individuals, with 2,273 participating in FY 2023 alone.

Alpha-1 Biomarkers Consortium (A1BC)

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

160
enrolled
participants in
FY 2023

The Alpha-1 Biomarkers Consortium (A1BC) aims to identify biomarkers that could be used to study the clinical progression of disease and could inform the design of future clinical trials. Led by Principal Investigator Jeanine D'Armiento, MD, PhD, Columbia University, the A1BC is conducting a six-year study that taps the Alpha-1 Research Registry to identify unaddressed research questions in Alpha-1 and clinically-relevant outcomes. Research participants will be followed over a three-year period to investigate a series of serum and imaging biomarkers. Patient-reported data will be collected through questionnaires.

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

SPOTLIGHT: **Looking Ahead**

ACT & A1BC

Improving the lives of people affected by Alpha-1 is a commitment of the Foundation. To that end, early detection is one of the most important areas in which the Foundation has placed a recommitted focus. Working with patients, clinicians, industry partners and government agencies, we are working on a variety of detection initiatives to bring an enhanced experience to the community.

The A1BC has made major strides this last year. With the addition of 3 new sites at the University of California, the University of Chicago and the National Jewish Health; the study has reached a milestone of being halfway to completion. The research team and industry partners eagerly discuss updates at quarterly meetings, making sure the project remains on track and successful. The positive progression of the A1BC will also further the success of the Alpha-1 Research Registry as it will populate additional data to better support clinical trials.

Alpha-1 Research Biorepositories

Providing Alpha-1 researchers from around the world with difficult to obtain DNA, plasma, lung tissue and liver tissue donated from Alphas, as well as supplementary biomaterial samples, to advance research

The Alpha-1 **DNA and Tissue Bank** contains de-identified clinical information and DNA and plasma samples from 2,572 Alphas. It is accessible to researchers in academia, government, and industry whose applications are approved by the Foundation's DNA & Tissue Bank Advisory Committee.

The Alpha-1 **Biomaterials Exchange** is a resource for both industry and academia that matches an investigator in need of biomaterial with another investigator who is a supplier of biomaterial. Importantly, the Biomaterials Exchange includes biomaterials not included in the DNA and Tissue Bank, such as viruses, viral vectors, plasmids, mice, and cell lines.



Conferences and Forums

Generating awareness of Alpha-1, identifying new areas of research interest, and establishing the Foundation's credibility within the rare disease community as a resource for serious research, education, and advocacy

Gordon L. Snider (GLS) Critical Issues Workshop Series

The GLS Workshop series facilitates exchanges between experts and answers important questions to stimulate additional research. Three recent workshops focused on biomarkers and endpoints for drug development in AATD, exacerbations and pulmonary comorbidities, and macrophages.

Clinical Resource Center (CRCs) Forums

These forums serve as platforms for networking, planning, and collaboration among CRCs, researchers, and industry partners.



We reach thousands of physicians, academic researchers, and industry partners each year through our exhibitions at major national and international medical conferences, where we share insights into new technologies, research findings, and priority research areas.

The Alpha-1 Foundation exhibits at:

- 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

EDUCATION & OUTREACH

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



The more we understand about Alpha-1, the better equipped we are to treat it. As we work toward a cure, empowering the Alpha-1 community is our priority.

IMPACT: EMPOWERING PATIENTS TO MAKE INFORMED DECISIONS ABOUT THEIR CARE AND TAKE PART IN ALPHA-1 RESEARCH

Alpha-1 National Conference

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



600+

Total National
Conference
attendance
participation

65%

of attendees were
Patients/Family
members

36%

First Time Attendees
to the Alpha-1
National Conference,
an increase of 13%
over last year

As the largest annual gathering of the Alpha-1 community, the National Conference provides important opportunities for networking, education, advocacy, and industry publicity. Over the course of three days, attendees have access to scientific and educational presentations, meet-and-greet and Q&A sessions with Alpha-1 experts, and other networking opportunities with attendees.

Alpha-1 National Education Series

Bringing patient education to a regional audience

The Alpha-1 National Education Series is comprised of Alpha-1 Education Days for Alphas, their families, and caregivers. These one-day educational programs provide access to current medical information, timely topics affecting Alphas, resources from leading Alpha-1 experts, and opportunities to connect with the Alpha-1 community.

Alpha-1 Education Days are held across the U.S., either in collaboration with a CRC or in a specific underserved area. Local Support Group Leaders and Alphas are engaged in regional planning committees and design programming according to the specific interests and needs of the Alphas in the region they serve, with a focus on connecting people to local resources. The American Association of Respiratory Care (AARC) has approved continuing education hours for respiratory therapists in attendance.

The Foundation continues its use of a virtual event platform, enabling those who were unable to attend an Alpha-1 Education Day in-person to participate and connect with the Alpha-1 community. Extending our Education Series into the virtual space has also created community-building and engagement opportunities through more direct access to social media, polls, and surveys.

1,000

people participated in the FY 2023 Alpha-1 Education Days.



28%

of participants were first time-attendees

SPOTLIGHT: Looking Ahead

Alpha-1 National Conference and Education Series

A key aspect of these events is in-person engagement. With a stronger focus on patient-to-patient interaction, we plan on bringing the community a more personalized experience at each event. We will also continue to utilize a virtual event platform, enabling those who were unable to attend these events in-person to participate and connect with the Alpha-1 community as well through community-building and engagement opportunities.

In an effort to further expand our educational resources, we will implement a new webinar series focusing on pertinent single topic sessions geared to bring expert knowledge to the community throughout the year.

Those that are attending these events for the first time will be offered tailored information based on their interests and interactions. This engagement tracking will help us continue to grow our community and build leaders and new volunteers to help further our mission.

Professional Education

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

The Alpha-1 Foundation, in partnership with professional organizations, offers opportunities for continuing education credits on a range of topics related to Alpha-1, including diagnosis, testing, augmentation therapy and treatment.

We offer a free online course providing one contact hour for registered nurses and nurse practitioners. We also identify opportunities for virtual and in-person grand rounds and encourage CRC physicians to present on research and new therapies.

More than 95 medical providers have completed the Nurse Practitioner Continuing Education credits through the Professional Education program, including 21 in FY 2023.

More than 710 medical providers have completed the Respiratory Therapist Continuing Education credits through the Professional Education program since inception, including 45 in FY 2023.

Since the launch of our new Continuing Medical Education (CME) program with the Cleveland Clinic, more than 2,880 medical providers have taken the courses with 25% of them claiming continuing credits. This included 39 internal medicine and other medical sub-specialties with 15 countries represented.

Partner Organizations

- Association for Continuing Education (NACE)
- Nurse Practitioner Associates for Continuing Education (NPACE)
- National Association of Pediatric Nurse Practitioners (NAPNP)
- American Association of Nurse Practitioners (AANP)
- American Association for Respiratory Care (AARC)
- Cleveland Clinic

Medscape

In April 2023, we launched a continuing medical education (CME) program with MedScape. More than 1,990 medical providers have taken the course with 28% receiving the associated credits. The total video views were over 4,190 since inception.

SPOTLIGHT: Looking Ahead

Professional Education

We continue to provide opportunities for healthcare practitioners to gain new knowledge, so they can, in turn provide the best attention for Alphas in their care. Education remains an integral priority for advocating for an accurate diagnosis. By building on the current successes we have had with our partnership with the Cleveland Clinic, we plan to expand our medical education platform to other areas including a new partnership with MedScape to broaden our reach even further.

Attending and exhibiting at various medical conferences allows us to bring the Alpha-1 diagnosis and care message to the larger clinical audience. We are recommitted to participate in as many conferences as possible to ensure that the message of early diagnosis is immersed in the medical community.

Educational Resources

A photograph of two women in a classroom or meeting setting. The woman in the foreground is older, with short grey hair and glasses, wearing a blue patterned shirt. The woman behind her has long brown hair and glasses, wearing a blue and yellow floral shirt, and is holding a white pen to her chin. They are both looking towards the right side of the frame. The background is blurred, showing other people in the room.

We offer 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.

www.alpha1.org

A clearinghouse for information on Alpha-1

Our website provides educational resources, highlights news and upcoming events, and creates opportunities for virtual connection within the global Alpha-1 community. We maintain content targeted toward several different audiences, including newly diagnosed Alphas, family members and caregivers, healthcare providers, researchers and industry partners.

Alphas and their families visit our website to access educational content, build connections with others in the Alpha-1 community, and find a CRC specialist or local support group. Healthcare providers visiting our website can find guidelines on diagnosing and treating Alpha-1, as well as links to specialists for referrals, while researchers can find information on funding opportunities.

Most Visited Pages:

Home Page (87,152 visits)	Newly Diagnosed/ Liver Disease (15,650 visits)
Testing for Alpha-1 (24,541 visits)	Newly Diagnosed/ Lung Disease (15,650 visits)

www.alpha1.org received **507,779** visits from **238,131** unique visitors in FY 2023





Social Media Stats

Facebook followers increased **7.5%**. In FY23 there was **20,409** followers.

Instagram followers increased **5.6%**. In FY23, there were **2,635** followers.

Follow Us on Social Media!

We connect with the Alpha-1 community across multiple social media channels. In addition to promoting new Foundation initiatives, community education events, and fundraising campaigns, our presence on Facebook, Instagram, LinkedIn and Twitter has directly increased traffic to our website, where users can access the information they need.

-  **@Alpha-1 Foundation**
-  **@AlphaFriend**
-  **@alpha1foundation**
-  **@Alpha-1 Foundation**

SPOTLIGHT: Looking Ahead

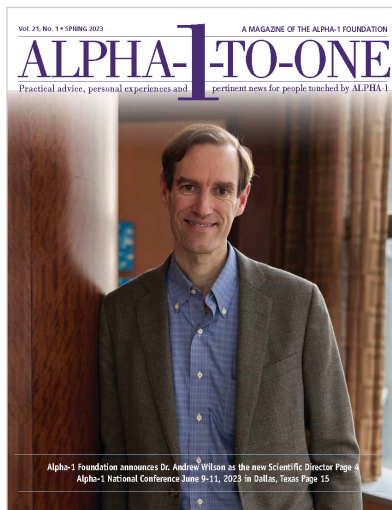
www.alpha1.org

We are excited to announce the launch of the new and improved Alpha-1 Foundation website in Q1 of FY24. Redesigning with the Alpha-1 community in mind, the revamped alpha1.org features a simplified menu to help users navigate the site and find the resources they need. It includes an integrated and searchable video library to view recent educational presentations, upgraded map features to find an Alpha-1 Specialist and Alpha-1 Support Group near you, and much more.

Our goal was to create a highly functional, information rich resource that offers a user-friendly experience for visitors, especially Alphas and their families. We also streamlined the Alpha-1 Foundation website to match more contemporary platforms and provide better overall functionality.

Alpha-1-To-One Magazine

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



Spring 2023



Fall 2022



Winter 2022

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 2023, we distributed **32,000** printed copies of each *Alpha-1-To-One* issue, including bundles to CRCs and healthcare providers; each issue is posted to a digital publishing platform on our website, where it was accessed by nearly 2,000 more visitors this year.

Each issue of *Alpha-1-To-One*, published tri-annually, includes research updates, an introduction to Alpha-1 specialists and researchers, and a recap of our outreach and advocacy efforts. Additional content is determined by an editorial board of patients, physicians and researchers, and other supporters, based on new and emerging needs of the Alpha-1 community.

Magazine subscriptions increased 6.4% this year.
There are currently over 100,000 Magazines printed and distributed annually.

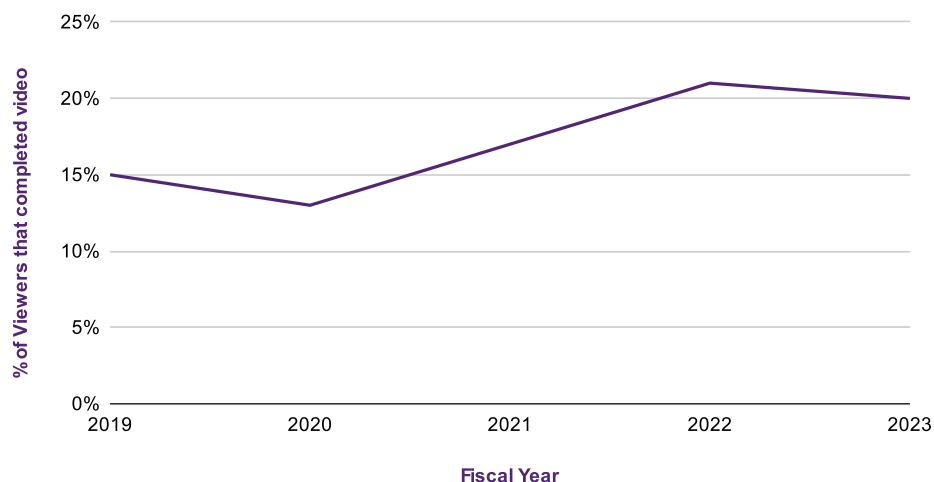
E-Education Library

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

The library, which can be accessed via our website, includes presentations from our educational events, including Alpha-1 Education Days and Alpha-1 National Conferences. It has become a valuable resource for the global Alpha-1 community and is frequently used by Alpha-1 support group leaders as a tool to offer expert presentations at meetings.

We add content to the E-Education Library throughout the year, with new videos promoted through e-mail, social media channels, and our 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 library offers useful content for everyone in the Alpha-1 community.

Video Library Engagement



E-Education Library Topics

- Advocacy
- Caregiving
- Children and Alpha-1
- Genetics
- Getting involved
- Liver
- Living with Alpha-1
- Lung
- Nutrition
- Oxygen
- Pulmonary rehab and exercise
- Research
- Transplantation

IMPACT: EMPOWERING PATIENTS TO MAKE INFORMED DECISIONS ABOUT THEIR CARE AND TAKE PART IN ALPHA-1 RESEARCH

Educational Brochures

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

We distributed more than **19,000** printed brochures in FY 2023 to numerous audiences, including Alpha-1 patients and families, healthcare professionals, and those at risk of Alpha-1.

Brochures are disseminated to: CRCs, Alpha-1 support group leaders, AlphaNet Coordinators, attendees at meetings and conferences, and inside test kits sent by the ACT Study. Brochures can also be downloaded or requested on our website by calling or emailing the Foundation.

Brochure Topics

- Healthcare Providers Guide
- How to Find an Alpha Doc
- What is Alpha-1?
- It's All in the Family - Family Testing
- The Liver and Alpha-1
- Am I an Alpha-1 Carrier?
- A Guide for the Recently Diagnosed Individual
- Clinical Trials
- Research Opportunities

IMPACT: CONNECTING AND STRENGTHENING THE ALPHA-1 COMMUNITY

Community Currents Newsletter

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

Our 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.

Community Currents subscriptions are up 5% from last year with currently 17,886 electronic subscribers.

***Alpha-1-To-One* Podcast**

An opportunity for listeners to experience portions of the *Alpha-1-to-One* Magazine in an audio format

The *Alpha-1-To-One* Podcast tells the personal stories of Alphas, families, healthcare providers, researchers, and other members of the Alpha-1 community. Grounded in the readership of the *Alpha-1-To-One* Magazine, the podcast expands the Foundation's media platform to an audio medium that has the capacity to reach a new audience, including the next generation of the Alpha-1 community.

We work closely with the Alpha-1 community to determine content for podcast episodes while building listenership and engagement.



SPOTLIGHT: Looking Ahead

Educational Resources

We are always looking for new ways to reach Alphas, increase community engagement, and inform patient care. We continue to build consistent, identifiable branding into our digital and print materials to strengthen the Foundation's identity. We plan to broaden our reach with a totally revamped version of the Alpha-1-To-One podcast. New episodes will spotlight key opinion leaders through one on one discussions with the CEO and Foundation leadership.. Topics will encompass issues and areas that affect Alphas and will provide new information and a deeper understanding of key initiatives that we are focused on.

Patient Outreach

A photograph of two women hugging, with a third person partially visible in the background. The image has a soft, purple-tinted overlay.

Our 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 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. 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.

Direct Patient Support

Patient Information Line

In FY 2023, the Patient Information Line fielded 700 calls, helping Alphas and family members locate specialists, access support, and gain information on a range of topics, including managing Alpha-1, COVID-19 risks, and understanding Alpha-1 testing.

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 can be used to pay for basic and urgent needs outside of insurance premiums or co-payments.

Oxygen Travel Assistance Program

The Alpha-1 Foundation's Oxygen Travel Assistance Program provides access to supplemental oxygen systems to support Alphas traveling for medical emergencies, transplant evaluation, and visits to CRCs. From FY 2022 to FY 2023, we loaned and facilitated the delivery of more than double the amount of supplemental oxygen systems to Alphas in need due to the return of in-person events.

CRC Access Program

Patients can apply for a one-time travel stipend of up to \$500 to visit their nearest CRC for the first visit. The CRC Access Program supports access to Alpha-1 specialists who provide can provide best-practice health management strategies and discuss augmentation therapy.

Genetic Counseling Program

Helping Alphas understand their diagnosis by providing psychological, medical, and familial support



We partner with the University of Florida to provide free genetic counseling services to Alphas, family members, healthcare providers, and other individuals considering testing or in need of support. The Genetic Counseling Program is invaluable in helping patients understand their genetic risk for developing lung and liver disease, as well as communicating that information to family members.

Trained counselors field calls for personalized genetic counseling, interpreting results from ACT Study participants, and referrals based on specific needs. Many callers are newly diagnosed Alphas looking for initial information and recommendations.

It's All in the Family — Family Testing

We feature family testing material at Alpha-1 Education Days and Clinical Resource Centers, and distribute 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.

Alpha-1 Kids Program

Information and resources for families of Alpha-1 infants, children, and teens, as well as young adults transitioning to independent care

People under 25 years old are underrepresented in Alpha-1 demographic data. We recognize that the unique needs of this population require focused support and are working closely with the Alpha-1 community to identify gaps and new areas of program expansion. The Parents of Alpha-1 Kids (PAK) virtual support group provides specialized resources for families who are looking for support during their child's Alpha-1 journey.



SPOTLIGHT: **Looking Ahead**

It's All in the Family and the Genetic Counseling Program

Supporting the families of Alpha-1 kids is vital to growing a stronger, more engaged community. With the recent expansion of the Clinical Resource Center network to include 7 new pediatric liver specialists, we help to provide access to the right care for our children. Alpha mom, Erin Carr, has been instrumental in the recruitment of new physicians around the country with experience treating Alpha-1 kids. To further this outreach, we plan on creating a Pediatric Liver Roundtable meeting to bring together the leading experts to discuss the latest advances in Alpha-1 and to learn about, discuss, and debate current topics in clinical applications. We will cover a review of the natural history of the disease, current management, family testing, highlight new developments, share findings of the Children's network, and discuss possible new therapies.

Educational Scholarships

Funding educational opportunities for those impacted by Alpha-1

Annually, we award educational scholarships to Alphas and family members seeking to further their education at an accredited university, college, community college, or technical institute. In FY 2023, we saw an increase of **76%** in applications for scholarships and awarded 10 Alpha-1 Educational Scholarships, totaling \$25,000.

James Quill Memorial Scholarships

Aaron Layman, from Ney, Ohio, is a junior at the University of Toledo majoring in Mechanical Engineering.

Carlos Yanez Navarro, from Seattle, Washington, is a recent graduate from the University of Washington who majored in International Studies.

Robert J. Haggerty Memorial Scholarship

Luke Moser, from Chazy, New York, is a freshman at Ithaca College majoring in Sports Management.

John W. Walsh, III (Jack) Memorial Scholarship

Jenna Russom, from Jacksonville, Florida, is a senior at the University of Florida majoring in Microbiology.

E. Lou Glenn Memorial Scholarship

Molly Scott, from Syracuse, New York, is a junior at Syracuse University double majoring in Nutrition Science and Biochemistry.



Terry L. Young Memorial Scholarship

Chase Noel, from Cottonwood Heights, Utah, is a senior at Utah State University majoring in Finance.

Peter Smith Scholarships

Marisa Marvel, from Orem, Utah, is a freshman at Southern Virginia University majoring in Biochemistry, with a minor in Music.

Evan Mescher, from Cincinnati, Ohio, is a freshman at Purdue University majoring in Engineering.

Austin Wagner, from Olive Hill, Kentucky, is a senior at Morehead State University, majoring in Business Administration, Accounting.

Audrey Woods, from Longmeadow, Massachusetts, is a senior at Amherst College, majoring in Political Science.

Strengthening the Alpha-1 community

We accomplish our mission with help from Alphas, those impacted by Alpha-1, our industry partners, researchers, and investigators. Our progress depends on building our 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



Support from the Alpha-1 community is a critical component of our goal to raise awareness of Alpha-1 and to find a cure. Through the Building Friends for a Cure (BFC) program, we steward relationships with volunteers to boost awareness of Alpha-1 and get the community involved in working toward a cure.

In the last year, Alphas, friends and family, researchers, industry and medical professionals came together to raise funds for research for a cure for Alpha-1. Some of this year's activities included successful social network campaigns and the utilization of online fundraising platforms. Over the last year, the Foundation has seen much success as it transitioned from virtual events back to hybrid events. Events included walkathons, golf tournaments and the two signature events: Escape to the Cape and the Celtic Connection. The Escape to the Cape brought over 100 members from the Alpha-1 community for a two -day bike ride in Cape Cod, MA. Towards the end of the year the annual Alpha-1 Virtual Walk took place during Alpha-1 Awareness Month with participation of over **850** participants on **75** teams from around the country.



Building Friend for a Cure consists of a series of community fundraising events in support of Alpha-1 research, including:

- | | |
|-------------------------|-----------------------------|
| – Riding for a Reason | – Walks, runs and marathons |
| – Escape to the Cape | – Alpha Hours |
| – Alpha-1 Virtual Walks | – Climbs and hikes |
| – Celtic Connection | – Video game streaming |

SPOTLIGHT: Looking Ahead

Building Friends for a Cure

We continue to work to build and engage the community to increase participation in the BFC program. Our goal is to continue using the virtual platform we have built using contemporary technology and strong branding to reach a wider audience and provide both virtual and in-person opportunities for Alphas worldwide to get involved in the search for a cure.

Alpha-1 Awareness

Annual campaigns to facilitate community support and improved approaches to care

Creative content created for Alpha-1 awareness campaigns appears in programming throughout the year.

#Alpha1Awareness

November is Alpha-1 Awareness Month—30 days of awareness-focused initiatives, through which the Alpha-1 community shares stories and resources while raising funds through the Alpha-1 Virtual Walk to support the search for a cure.



Special Events

Celebrating the Alpha-1 community and supporting research

Our Special Events are opportunities to expand our donor and volunteer base and raise funds for programs and research. Over 300 guests attended the following Special Events in FY 2023:

Alpha-1 Foundation Grant Awards Reception:

Alphas, physicians, researchers, and industry partners attended the reception to recognize newly awarded grantees for their work and dedication in Alpha-1 and their significant role in the search for a cure. This year's event was held in the rotunda of the National Archive Museum in Washington, DC.

Celebration of Life: This annual golf tournament and reception benefits transformative Alpha-1 research and advances our mission to find a cure. Attendees include Alphas, family members, senior leadership, and industry representatives.



Generating change to improve outcomes for Alpha-1 patients

“Access to home-based care reduces healthcare costs while giving people dignity and peace of mind as they receive quality care in their home,” said Rep. Salazar. “I am proud to lead this legislation, which would make life easier for Medicare patients struggling with this rare genetic disease.”

Advocates in Action

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

Our 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. Our public policy work is carried out in part by our grassroots community as well as by key stakeholders, staff, and our extensive network of healthcare professionals. The Foundation provides training, guidance, and resources to empower, educate, and mobilize Alphas to convey the urgency of our cause.

Advocates urge members of Congress and federal agencies to prioritize the needs of Alphas and those affected by rare and chronic conditions, with a focus 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. Alpha-1 advocates represent the Alpha-1 Foundation at forums and lobby days each year.

Reps. María Elvira Salazar (R-FL) and Chellie Pingree (D-ME) have reintroduced the John W. Walsh Alpha-1 Home Infusion Act (H.R.4438). If passed, certain Medicare beneficiaries living with alpha-1 antitrypsin deficiency would be able to receive essential augmentation infusions at home.

Policy priorities include:

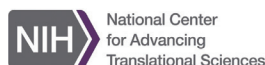
- Medical research
- Therapeutic development
- Screening and detection
- Access to care
- Federal and state funding
- Blood product safety
- Education and awareness

Institutional, Agency, and Coalition Partners

The Foundation 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 our public policy agenda for the benefit of the Alpha-1 community.

- **U.S. Food and Drug Administration (FDA):** The Foundation 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, we are providing the 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 the Foundation’s work, including data collection and research through the Alpha-1 Research Registry.

Our Partners, Memberships and Coalitions



SPOTLIGHT: Looking Ahead

Advocates in Action

We recognize the importance of the patient voice and the collective impact the patient community can have on awareness and advocacy. We, along with our team of policy consultants, meet regularly with the NIH and have ongoing conversations with the FDA to determine how best to work collectively to ensure the Alpha community has access to, and approval for, novel treatments and therapies.

We are working with the 118th Congress to find a solution to the patient access problem facing Medicare beneficiaries who are not able to obtain a home infusion due to Medicare law. The John W. Walsh Alpha-1 Home Infusion Act (H.R.4438) has been reintroduced by Representative Salazar. This process is ongoing, and we will continue to work diligently with advocates and legislators to meet the needs of the Alpha-1 community. Advocates will have meetings throughout the year both in-person and virtually with their representatives to encourage their support of the bill.

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 the Alpha-1 Global website. Alpha-1 organizations and individuals connect to share ideas, strategies, and tools on three main topics: awareness, detection, and access to care. The website also provides access to information on the latest Alpha-1 developments around the world, as well as educational seminars, and an online discussion forum.

The **Alpha-1 Advocacy Pathway** is an interactive platform that provides tools for becoming a dedicated Alpha-1 advocate on a number of levels.

Alpha-1 Global maintains **an extensive global database** of patient, physician, industry, and researcher contacts that spans six continents and is segmented into unique audiences for various communications campaigns.



SPOTLIGHT: **Looking Ahead**

Alpha-1 Global

In 2024 we are excited to continue our global leadership role. We are working with our International Patient Colleagues and the European Alliance to leverage the recognition of November Awareness Month in the U.S. and include patients, and key stakeholders around the world to raise awareness of AATD. We are planning to participate in the European Union Parliamentary Proceedings on AATD. We are strengthening our relationship with EARCO through the creation of shared initiatives and plan to expand our efforts with the strong partnership we have built with researchers and patients in Ireland.

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.



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