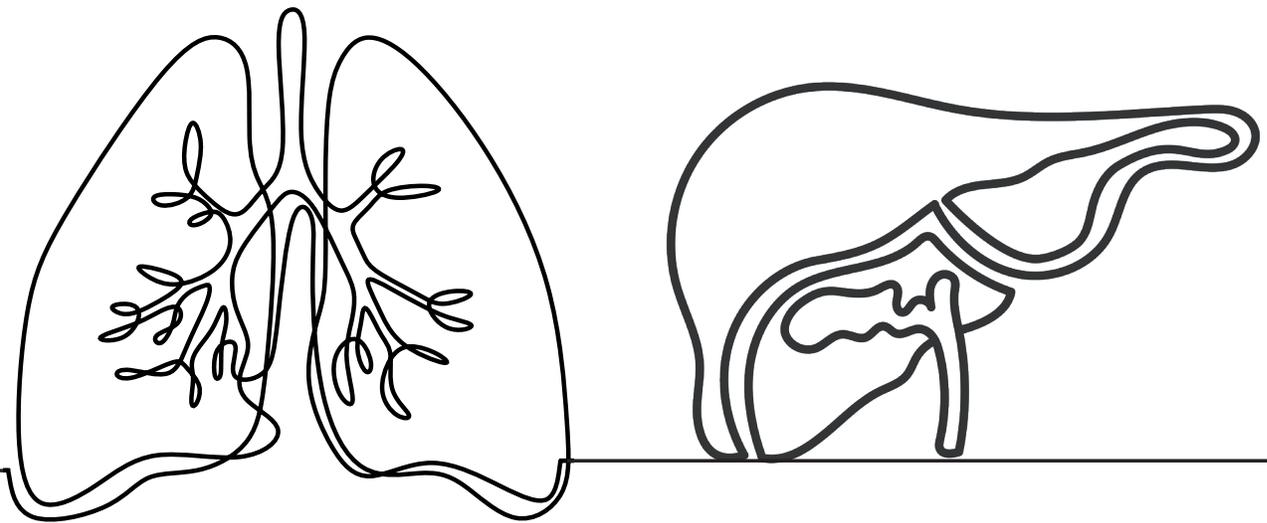


Virtual 21st Gordon L. Snider Critical Issues Workshop

Exacerbations and Pulmonary Comorbidities



January 29, 2021 from 9 am to 1 pm ET
& January 30, 2021 from 9 am to 12:30 pm ET



Exacerbations and Pulmonary Comorbidities 21st Gordon L. Snider Critical Issues Workshop

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MEETING OVERVIEW

Welcome to the 21st Gordon L. Snider Critical Issues Workshop entitled, “Exacerbations and Pulmonary Comorbidities.”

The last 50-60 years have seen substantial progress in the identification and management of Alpha-1 Antitrypsin Deficiency. Over the last 25 years, individuals with Alpha-1 and their families have significantly benefited from the comprehensive disease management program offered by AlphaNet that has proven to extend the average life expectancy.

The Foundation’s contribution to the body of knowledge regarding Alpha-1 and its international research investments have brought us closer to the cure.

The rarity of the condition continues to impact on clinical care with many remaining unanswered questions. Early on, the Foundation set up a research registry to overcome the lack of significant cohorts which can hamper rare disease understanding and research. Our improved clinical research registry will facilitate the in-depth exploration of these issues. Recent investigator and patient-driven questions continue to influence both clinical and research efforts to develop more comprehensive knowledge. This workshop is the result of many of those questions which we will explore today.

The goal of the workshop is to discuss more recent knowledge addressing some of these issues and to highlight where real progress has been made and where knowledge in patient management remains sub-optimal. In particular, the role and effect of acute exacerbations and disease subtypes on patients’ health and long-term outcomes are to be addressed. The program will include presentations followed by open discussion.

The Foundation would like to thank the workshop’s co-chairs, Professor Robert Stockley, Dr. Alan Barker and Ms. Jean McCathern, for their input into the conference program and for inviting and securing the participation of a group of experts to present data and partake in the ensuing discussions. We would also like to thank the members of the planning committee and staff of the Alpha-1 Foundation for all of their hard work in putting this workshop together.

Following the workshop, the Foundation will work with the co-chairs to generate action items, a white paper and possible publication.

We look forward to making this a meaningful and memorable event!



Miriam O’Day
President & CEO
Alpha-1 Foundation



Jeanine D’Armiento, MD, PhD
Chair, Board of Directors
Alpha-1 Foundation

AGENDA

DAY 1

Welcome

09:00am – 09:05am M. O'Day (Alpha-1 Foundation) and M. Brantly (University of Florida)

Co-Chair Introduction

09:05am – 09:10am R. Stockley (Co-Chair, University Hospitals Birmingham), J. McCathern (Co-Chair, Highspire, PA), and A. Barker (Co-Chair, Oregon Health & Science University)

Pulmonary Exacerbations

09:10am – 09:20am **What is an Exacerbation? The Patient Perspective**
H. Stagl (Beacon, NY)

09:20am – 09:35am **What is an Exacerbation? The Physician Perspective**
M. Campos (University of Miami)

09:35am – 09:50am **What is the Role of Chronic Inflammation in Exacerbations?**
D. Sin (University of British Columbia)

09:50am – 10:05am **Prevention of Exacerbations**
P. Calverley (University of Liverpool)

10:05am – 10:25am **What is the Role of Bronchiectasis in COPD Exacerbations? Exacerbation: Comparison Between AATD and Usual COPD**
R. Stockley (University Hospitals Birmingham)

10:25am – 10:55am **Moderated Discussion**
R. Stockley (University Hospitals Birmingham)

10:55am – 11:15am **Break**

Comorbidities

11:15am – 11:30am **Cardiovascular**
E. Sapey (University Hospitals Birmingham)

11:30am – 11:45am **Bronchiectasis**
J. Chalmers (University of Dundee)

11:45am – 12:00pm **Sleep Apnea**
T. Siddharthan (University of Miami)

12:00pm – 12:15pm **Pulmonary Hypertension**
M. Dalabih (National Jewish Health)

12:15pm – 12:25pm **Coping With Alpha-1**
J. McCathern (Highspire, PA)

12:25pm – 12:55pm

Moderated Discussion

A. Barker (Oregon Health & Science University)

Wrap up - Day 1

12:55pm – 01:00pm

M. O'Day (Alpha-1 Foundation)

DAY 2

Welcome

09:00am – 09:05am

M. Brantly (University of Florida)

Morbidities

09:05am – 09:15am

Liver – The Patient Perspective

G. Collett (Bend, Oregon)

09:15am – 09:30am

Liver – The Physician Perspective

V. Clark (University of Florida)

09:30am – 09:45am

Panniculitis

N. McElvaney (Royal College of Surgeons in Ireland)

09:45am – 10:00am

Frequently Asked Questions (treatment: MWFtx, Azithromycin)

M. Brantly (University of Florida)

10:00am – 10:30am

Moderated Discussion

R. Stockley (University Hospitals Birmingham)

10:30am – 10:40am

Break

Usual COPD vs. Alpha-1 COPD

10:40am – 10:55am

The Anatomy in Usual COPD vs. Alpha-1 COPD

K. Chapman (University of Toronto)

10:55am – 11:15am

The Differences in Treatment Approaches, Augmentation Therapy, Inhaled Treatment

Panel Discussion: R. Stockley (University Hospitals Birmingham), R. Sandhaus (National Jewish Health), C. Strange (MUSC)

11:15am – 11:45am

Moderated Discussion

R. Stockley (University Hospitals Birmingham)

Concluding Remarks

11:45am – 11:55am

M. O'Day, M. Brantly, R. Stockley, J. McCathern, A. Barker

SPEAKER BIOS

Alan F. Barker, MD

Oregon Health and Science University

Dr. Alan Barker is Professor of Medicine, Pulmonary and Critical Care, at Oregon Health and Science University, Portland. With a career interest in COPD, he focused on alpha-1 antitrypsin deficiency beginning in 1988 with participation as an investigator in the landmark NHLBI Alpha-1 Antitrypsin Registry. Since then, he has collaborated on many clinical research studies regarding Alpha-1. In addition to being one of the Alpha Docs in the Northwest, he has organized and hosted 25 annual forums for alpha-1 individuals, families, and friends. In the past 20 years the link with alpha-1 has expanded his expertise to other rare respiratory conditions that have yielded insights to common lung diseases.



Mark L. Brantly, MD

University of Florida

Dr. Mark Brantly is a Professor of Medicine and Molecular Genetics and Microbiology, Department of Medicine Vice-Chair for Research at the University of Florida College of Medicine in Gainesville, where he is also the Alpha-1 Foundation Research Professor. He is currently serving as the Scientific Director of the Alpha-1 Foundation.



Dr. Brantly runs a translational research laboratory focused on better understanding the molecular basis of alpha-1-antitrypsin deficiency and developing novel therapies and biomarkers for alpha-1-antitrypsin deficient individual. Dr. Brantly also maintains an international reference laboratory for alpha-1-antitrypsin levels, phenotype, and genotype analysis.

Dr. Brantly earned his medical degree from the University of Florida College of Medicine in Gainesville. He completed a residency in internal medicine at Eastern Virginia Medical School in Norfolk, where he was also Chief Medical Resident, and a fellowship in pulmonary disease at the National Institutes of Health in Bethesda, Maryland. Dr. Brantly was a senior researcher at the NIH Clinical Center for 16 years.

Dr. Brantly's clinical and research interests include alpha-1-antitrypsin deficiency lung and liver disease and chronic obstructive pulmonary disease. Dr. Brantly has published more than 170 manuscripts, book chapters, editorials, and books during his career. He is a member of the American Thoracic Society and the American Society for Cell Biology and is a reviewer for many journals, including CHEST, the American Journal of Respiratory and Molecular Cell Biology, and the American Journal of Respiratory and Critical Care Medicine and an editor of Genetics and Molecular Biomarkers.

Peter Calverley, MBChB, DSc, FRCP, FRCPE, FMedSci, FERS, ATSF

University of Liverpool

Professor Peter Calverley is Emeritus Professor of Respiratory Medicine at the University of Liverpool, UK and continues to work in the Institute of Ageing and Chronic Disease. He trained at the University of Edinburgh, was a travelling fellow at the Meakins-Christie Laboratories at McGill University, Montreal 1982–3 and subsequently moved to Liverpool, becoming a full professor in 1995. His research interests focus on the patho-physiology and management of chronic obstructive pulmonary disease. He has published over 350 papers and several textbooks as well as lecturing widely on these topics.



Prof. Calverley was Associate Editor of Thorax till 2010 and has been an Associate Editor of the American Journal of Respiratory and Critical Care Medicine since 2015. He has chaired the scientific committee of the British Lung Foundation, the British Sleep Society and the Clinical Physiology Assembly of the ERS and was a member of the ERS Executive Committee.

He helped develop the GOLD initiative for COPD, and chaired its Dissemination Committee and Scientific Committee. He was President of the British Thoracic Society in 2006, co-chaired the External Reference Group advising Ministers on the content of the COPD Clinical Service Strategy and chaired the Respiratory Specialty groups of the UK Comprehensive Clinical Research Network until 2015. Prof. Calverley is a Fellow of both the London and Edinburgh Colleges of Physicians and was elected to the Fellowship of the Academy of Medical Sciences in 2011, the European Respiratory Society in 2014 and the American Thoracic Society in 2020.

Michael A. Campos, MD, FACP
University of Miami



Dr. Michael Campos is a Professor of Clinical Medicine at the University of Miami School of Medicine, Miami, Florida, USA. He received both his bachelor's degree in biology and medical degree from the Universidad Peruana Cayetano Heredia, Lima, Peru in 1994 and completed his training in Internal Medicine, Critical Care Medicine and Pulmonary Medicine at the University of Miami/Jackson Memorial Medical Center, Miami, Florida. In 2003 he joined the faculty at the University of Miami School of Medicine. In addition to his academic career, Dr Campos is Section Chief, Pulmonary Medicine of the Miami Veterans Administration Medical Centre, Miami, Florida.

Dr. Campos is principal investigator in many research trials focusing on tobacco smoking, electronic cigarettes and COPD. He has particular interest in alpha-1 antitrypsin deficiency (AATD), and has performed trials sponsored by NIH, the Alpha-1 Foundation and Industry. Particular interests focus on bronchial epithelial alpha-1 antitrypsin, the role of AAT in inflammation, the microbiome, the effects of double dose augmentation therapy and the long-term outcomes of patients with AATD.

James Chalmers, MBChB, PhD
University of Dundee



Professor James Chalmers is the British Lung Foundation Chair of Respiratory Research and Consultant Respiratory Physician at the School of Medicine, University of Dundee. His clinical and research interests are in respiratory infections, particularly bronchiectasis and COPD. He chairs the European Bronchiectasis Registry (EMBARC) and has authored more than 270 studies in the field. He chaired the 2017 European Respiratory Society Bronchiectasis Guidelines, the 2020 ERS Inhaled Corticosteroids Withdrawal Guidelines and currently chairs the ERS COVID19 task force. Prof. Chalmers' research group studies inflammation and the microbiome in respiratory disease and is funded by grants from the Wellcome Trust, Medical Research Council, Scottish Government, EU and the pharmaceutical industry.

Kenneth R. Chapman, MD, MSc, FRCPC, FACP, FERS

University of Toronto

Dr. Kenneth Chapman is Director of the Asthma and Airway Centre of the University Health Network, President of the Canadian Network for Respiratory Care and Director of the Canadian Registry for Alpha-1 Antitrypsin Deficiency. A graduate of the University of Toronto and a former member of the faculty at Case Western Reserve University, he is now a Professor of Medicine at the University of Toronto. Dr. Chapman is an internationally respected researcher in the field of asthma, COPD and airway diseases; his publications have appeared in the New England Journal of Medicine and the Lancet. The titles of more than 600 publications ranged from "Emergency medical services for outdoor rock music festivals" to "The effect of posture on thoracoabdominal movements during progressive hyperoxic hypercapnia in conscious man". With more the 19,000 citations to his work, Professor Chapman is in the top 1% of cited medical researchers. He chaired Canada's first Consensus Conference to establish Canadian guidelines for the management of COPD. Professor Chapman was the inaugural holder of the CIHR-GSK Research Chair in Respiratory Health Care Delivery at the University Health Network and was one of five Canadian respiratory physicians awarded Fellowship in the European Society in its Inaugural year.



Virginia Clark, MD, MS

University of Florida

Dr. Virginia Clark is an associate professor of medicine at the University of Florida where she serves as the program director of the gastroenterology training program. Her areas of clinical and research interest are liver disease in adults with alpha-1 antitrypsin deficiency and fatty liver disease. She is currently serving on the board of directors for the Alpha-1 Foundation.



Gordon Collett

Bend, OR

Mr. Gordon Collett was diagnosed with Alpha-1 in September 2007 after several years of doctors searching for the reason his liver was dying. It took the diagnosis of his brother in 2007 of Alpha-1 for Gordon to finally be tested for this disease. As a result of his liver being the most affected organ, he was granted a liver transplant in February 2008, which eliminated the Alpha-1 from his system. He lost about 20% of his lung function to the disease before the transplant. Mr. Collett holds a master's degree in Computer Science from Oregon State University. After working for several large companies, he decided to specialize in Internet services and has been a self-employed website designer and software consultant for over 15 years. Mr. Collett lives in Bend, Oregon with his wife of 29 years. He enjoys photography, with biking and hiking the beautiful high desert country of Central Oregon.



Mohammad Dalabih, MD

National Jewish Health

Dr. Dalabih is an Assistant Professor at National Jewish Health in Denver. He finished his pulmonary fellowship with a focus on pulmonary hypertension at the University of Arizona before joining the pulmonary hypertension program at National Jewish Health in 2019. His primary interests are pulmonary hypertension in patients with chronic lung disease and exercise-induced laryngeal obstruction.

Jean McCathern

Highspire, PA

Ms. Jean McCathern has been active in the Alpha-1 community since her diagnosis as a ZZ genotype in 2004. She became a support group leader for Central PA until she stepped down to take a position as an AlphaNet Coordinator. She worked as a Coordinator for 7 years and now acts as an information technology consultant for AlphaNet. She edited the update to the Big Fat Reference Guide in addition to her other duties at AlphaNet. Ms. McCathern has been involved in many research studies concerning Alpha-1. She is also active with the Alpha-1 Foundation, serving on a committee since 2009. She is currently a member of the Medical and Scientific Advisory Committee. Ms. McCathern served in the US Air Force for 25 years and subsequently worked with the Navy and then with the Pennsylvania Department of Transportation before joining AlphaNet. She has one daughter and three grandchildren. Her oldest grandson is serving in the Air Force. She lives in Highspire, Pennsylvania. She enjoys doing genealogy, gardening, and fishing.



Noel G. McElvaney, MD, BCh, BAO, FRCPI, FRCPC

Royal College of Surgeons in Ireland

Professor Gerry McElvaney is Chairman of the Department of Medicine and Professor of Medicine in the Royal College of Surgeons in Dublin, Ireland. Prof. McElvaney received his medical education at University College Dublin and completed his postgraduate internal medicine training at the Mater Misericordiae Hospital, St. Laurence Hospital and Jervis street Hospital in Dublin before pursuing a pulmonary Fellowship in Vancouver, Canada. Following that, Prof. McElvaney worked in the Pulmonary Branch, NHLBI, NIH, Bethesda and in Cornell University-Rockefeller University Hospital, New York. He returned to Ireland in 1996. Prof. McElvaney's unit has a well-established track record in research in Alpha-1 antitrypsin deficiency and Cystic Fibrosis and their work on lung defenses has led to interactions with pharmaceutical companies interested in translational research. For example, in 1999 Beaumont Hospital was the first site worldwide for intravenous administration of transgenic alpha-1 antitrypsin to individuals with Alpha-1 Antitrypsin Deficiency. In 2003, Prof. McElvaney founded the Alpha One Foundation of Ireland and subsequently received funding from the Irish Department of Health and Children to set up an Alpha-1 research unit to further research into the condition. He also established the first targeted detection program in Europe.



Miriam A. O'Day

Alpha-1 Foundation

Ms. Miriam O'Day has been the president and CEO of the Alpha-1 Foundation since February of 2019. Her experience with the Alpha-1 patient community, the research and medical community, and public policy at the federal level, extends over twenty years. Ms. O'Day has been involved in government relations, strategic planning and communications by representing many organizations including the Alpha-1 Foundation, the Immune Deficiency Foundation (IDF), the COPD Foundation, the U.S. COPD Coalition, NTM Info and Research, the American Association for Respiratory Care and the National Organization for Rare Disorders (NORD). Ms. O'Day served as the former Senior Director of Public Policy for the Alpha-1 Foundation. In that role she managed legislative and regulatory collaboration with the COPD Community and worked on a wide variety of issues with the Centers for Medicare and Medicaid Services (CMS) including supplemental oxygen and access to pulmonary rehabilitation.



Robert A. Sandhaus, MD, PhD, FCCP

National Jewish Health

Dr. Robert “Sandy” Sandhaus graduated from Haverford College in Pennsylvania with a degree in molecular biology. He received a PhD in cell biology and an MD from Stony Brook University in New York. He completed his residency in internal medicine at the Beth Israel Deaconess Medical Center in Boston and a pulmonary fellowship at the University of California San Francisco (UCSF). He has been board certified in internal medicine, pulmonary disease, and critical care medicine. Following his pulmonary fellowship and a post-doctoral research fellowship, he joined the UCSF medical faculty and subsequently the medical faculty at the University of Colorado School of Medicine where he founded the Alpha-1 Antitrypsin Deficiency Program at National Jewish Health in Denver 40 years ago. He remains its director.



His early research focused on the role of white blood cell proteases in the prevention and promotion of lung disease. This work led to a special clinical interest in patients with the genetic deficiency of Alpha-1 Antitrypsin. In addition to his ongoing academic career, Dr. Sandhaus has worked in research and development in the biopharmaceutical industry. He ran clinical development programs at Cortech, NeXstar Pharmaceuticals, and Gilead Sciences. Dr. Sandhaus has served on the Boards of Directors of the Alpha-1 Association, the Alpha-1 Foundation, AlphaNet, the Alpha-1 Project (TAP), the Association for the Accreditation of Human Research Protection Programs, Global Implementation Solutions, and the Osteogenesis Imperfecta Foundation. For the past 20 years, he has been the Executive Vice President and Medical Director of AlphaNet and the Clinical Director of the Alpha-1 Foundation, two not-for-profit organizations serving the Alpha-1 community.

Elizabeth Sapey, BSc, MBBS, PhD, FRCP

University Hospitals Birmingham

Professor Liz Sapey is an academic respiratory physician at the University of Birmingham and University Hospitals Birmingham NHS Foundation Trust. She graduated from The Royal London Medical School and undertook a PhD at the University of Birmingham while completing specialist clinical training. Prof. Sapey’s research interests focus on chronic respiratory inflammation associated with neutrophil activation and recruitment. Her interests span translational science and moving new or repurposed therapies into early phase clinical trials. Her group focus on Alpha 1 Antitrypsin Deficiency and COPD related tissue damage and poor bacterial clearance.



Prof. Sapey is also the Director of the HDR-UK Health Data Research Hub in Acute Care, called PIONEER. She runs a programme to collect, link and use routine health data to understand disease, its presentation and potential response to treatments more readily. Prof. Sapey is the Managing Director of Birmingham’s NIHR Clinical Research Facility and Chair of the British Thoracic Society Science Committee. She has achieved much of this while working part time, bringing up her two children.

Trishul Siddharthan, MD

University of Miami

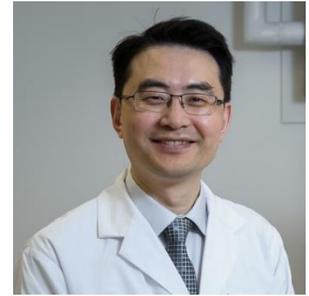
Dr. Trishul Siddharthan is an Assistant Professor of Medicine in the Division of Pulmonary and Critical Care Medicine at the University of Miami. His research over the past decade pertains to risk factors, health outcomes and management of chronic respiratory disease among in low- and middle-income settings, with a focus on obstructive lung disease among non-smokers. His current funded research includes identifying the effectiveness of treatment for COPD among non-smokes in LMICs and assessing the prognostic value of lung imaging for acute and chronic lung injury.



Don Sin, MD

University of British Columbia

Dr. Don Sin is the Director of the Centre for Heart Lung Innovation (HLI), and a Professor of Medicine at University of British Columbia (UBC) in Vancouver, Canada. He holds a Tier 1 Canada Research Chair in COPD and the De Lazzari Family Chair at HLI. He has published more than 500 peer-reviewed papers and has an H-index of 98. He has served on the Global initiative for chronic Obstructive Lung Disease (GOLD) scientific committee since 2009 and is the section editor for the European Respiratory Journal and an editorial board member of the American Journal of Respiratory and Critical Care Medicine. Dr. Sin obtained his medical degree at University of Alberta and completed his post-doctoral research fellowship at University of Toronto. His research focus is using “omics” data to discover novel biomarkers of disease activity and new therapeutic targets to reduce hospitalization and mortality in patients with COPD.



Heide Stagl

Beacon, NY

Ms. Heide Stagl was diagnosed with COPD in August 2001. In 2016, during one of her PFT exams, it was mentioned to her about a new test that was available regarding something called Alpha-1, so she decided to take it. A week later her doctor called and wanted to do the test again, he was not sure of the results and wanted to make sure the reading was correct. They did a second test and it showed that she was indeed an SZ Alpha-1. Ms. Stagl has four children, two of which have tested as carriers, one is an MZ and the other is an MS. Her other two children opted to not be tested at this time. She lives in Beacon, NY with her husband and is a loving grandmother to her most recent and closest living grandchild at the age of 8. In 2014, she retired from being an Administrative Assistant with the HealthSearch Group to help care for her youngest grandson. They enjoy cooking and gardening together, and she is enjoying watching him become a proficient horseback rider. Her 7 other grandchildren are grown and already starting their families!



Robert Stockley, MD, DSc, FRCP, FERS

University Hospitals Birmingham

Professor Robert Stockley is Professor of Medicine at the University of Birmingham and established the Lung Immunochemical Research group in 1980s to study the cell biology and biochemistry of COPD. He has a longstanding interest in COPD phenotypes with particular reference to airway inflammation, proteinases and antiproteinases. He established a registry for AATD patients in the UK in 1996 and has followed up more than 1,300 patients for over 20 years. He also helped establish and chair the Alpha-1 International registry. Prof. Stockley has been a member of the Executive Committee and is currently a member of the Scientific Committee of the Global Initiative for Chronic Obstructive Lung Disease (GOLD). He initiated and established the International meeting on COPD held every two years in Birmingham, UK from 1998 until 2018. He has edited 7 books and has more than 450 publications.



Charlie Strange, MD

Medical University of South Carolina

Dr. Charlie Strange is Professor of Pulmonary and Critical Care Medicine, at the Medical University of South Carolina in Charleston and Medical Director for AlphaNet. Dr. Strange received his undergraduate degree at Davidson College in Davidson, North Carolina, and his medical degree at the Medical College of Virginia in Richmond. He has been at the Medical University of South Carolina for > 35 years. Dr. Strange directed the Alpha-1 Foundation Research Registry from 2000-2019 enrolling > 7500 individuals with alpha-1 antitrypsin deficiency (AATD) to invite for community research study invitations. He directed the Alpha-1 Foundation Genetic Counseling Center from 2007-2019 supporting healthcare providers, patients, and individuals considering testing for AATD. Dr. Strange is the author or co-author of more than 300 publications, presentations, and book and computer chapters that focus on rare lung diseases, registry science, COPD, quantitative CT methodology, and AATD. In 2019 he joined AlphaNet as Medical Director.



The Alpha-1 Foundation would like to acknowledge the following sponsors for their generous support:

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

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