Externally-led patient-focused drug development meeting

September 20, 2021

Collaborators

littleairwaysbigvoices.org

#littleairwaysbigvoices  #asthma  #PFDD
Little Airways, Big Voices aims to bring the voice of patients and families impacted by asthma in childhood to the forefront of drug development and research.

Learn more at littleairwaysbigvoices.org
The Allergy & Asthma Network, American Lung Association, American Partnership for Eosinophilic Disorders, and Asthma and Allergy Foundation of America are collaborating on the Little Airways, Big Voices initiative. There are multiple ways to participate in this initiative, including attending the virtual externally-led patient-focused drug development (EL-PFDD) meeting on September 20, 2021, taking an online survey, and submitting written comments.

The goal of the EL-PFDD meeting is to provide a forum for patients and families to share their lived experience with managing childhood asthma and their perspectives on current and future treatment options with the U.S. Food and Drug Administration (FDA) and other stakeholders – including medical product developers, health care providers, and federal partners. We hope that the patient insights collected through this project will effectively engage families in drug development, inform new treatment strategies, and improve health outcomes.

What is a Patient-Focused Drug Development Meeting?

The FDA established the patient-focused drug development (PFDD) initiative in 2006 to collect patient insights on specific diseases and their impact on daily life, as well as treatment options. PFDD meetings are designed to connect patients and families directly with the FDA, as well as drug researchers and developers. In addition to the FDA hosting PFDD meetings, patient advocacy organizations are encouraged to host externally-led PFDD meetings so that the FDA can gain insights on a greater number of conditions. The meetings and resulting reports help the FDA identify what is important to patients and what people want treatment to look like.

About Asthma in Children

Asthma is a chronic lung disease of the airways. It causes episodes of coughing, wheezing, chest tightness, and shortness of breath. Increased mucus, inflammation in the airways, and bronchial constriction or muscle spasms make it difficult to breathe. Symptoms can be mild or severe, and sometimes life-threatening.

Asthma is the most common chronic respiratory condition in children. It affects approximately 5 million children (ages 0-17) in the United States.

Asthma in children can be especially serious because children have smaller airways than adults. It is a leading cause of missed school and work days, as well as emergency department visits, and one of the top indications for hospitalization in children.

Because children may not always understand or recognize asthma and its symptoms, parents face a variety of challenges as they try to help their children breathe. Although treatment options have increased significantly, there are still patients for whom these options are not effective or accessible.

Voice of the Patient Report

The Little Airways, Big Voices project team will use information from the EL-PFDD meeting, survey, and written comments to develop and share a Voice of the Patient report in 2022 about living with and managing asthma in childhood. We hope this report will inform future drug development and regulatory decisions.
INTERIM SURVEY RESULTS

In preparation for the September 20, 2021, Little Airways, Big Voices EL-PFDD meeting, the initiative collaborators fielded a survey to capture a broad set of experiences. As of September 14, 2021, 35 individuals responded to the survey. All respondents are caregivers of a child with asthma. One in four (26%) also report having asthma.

Demographics: Respondents reported a nearly even split when indicating the biological sex of the person with asthma (54% male, 46% female). Respondents were also well distributed across the United States. Half (49%) were reporting for a child between 5-11 years old.

Diagnosis: Over half (54%) of respondents report their child was diagnosed with asthma more than 5 years ago; one in ten (9%) report still being within the first year of diagnosis. Four in five (80%) report their child has some form of persistent asthma, with nearly half (46%) having moderate or severe persistent asthma. Respondents manage a variety of asthma types, with 69% reporting their child has allergic asthma. Over half (54%) indicate their child has viral induced asthma, and two in five (40%) report their child has exercise-induced asthma.

Impact of Asthma: When asked which areas of life have been most negatively impacted by asthma, five areas were selected by more than 30 percent of all respondents: physical health (69%), work/school (57%), sports/exercise (57%), emotional health (31%), and self-confidence (31%). The bar graph below further describes the impact of asthma on school, work, and participating in physical or social activities.
Satisfaction with Current Treatments: While three in four (75%) respondents say they are “somewhat” or “very” satisfied with their child’s current asthma medicines and a vast majority (86%) say their child’s asthma medication is effective in reducing asthma symptoms, three in five (60%) say their medicine is expensive, and one in five (20%) say their child’s current medicine causes negative side effects.

Looking Ahead: Aside from a complete cure, the most important result a treatment could provide is less asthma symptoms (74%), followed closely by reduced quick-relief or rescue inhaler use (66%). Three in five (60%) also say reduced asthma-related emergency room/hospital visits, reduced need to visit the doctor for asthma, and less worrying about asthma are ideal outcomes. When asked which factors were most important when considering a new course of treatment for their child, the most important factors are effectiveness (86%) and side effects (74%). The bar graph below shows additional factors that are taken into consideration when deciding on a treatment.
### AGENDA

**Externally-Led Patient-Focused Drug Development (PFDD) Meeting on Childhood Asthma**

September 20, 2021, 10:00 a.m. - 3:00 p.m. EST

<table>
<thead>
<tr>
<th>Time</th>
<th>Session/Activity</th>
<th>Organizer/Presenter</th>
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</thead>
<tbody>
<tr>
<td>10:00 - 10:05 a.m.</td>
<td>Welcome and Opening Remarks</td>
<td>Sanaz Eftekhari, Vice President of Research, Asthma and Allergy Foundation of America, Kenneth Mendez, MBA, President and CEO, Asthma and Allergy Foundation of America</td>
</tr>
<tr>
<td>10:05 - 10:20 a.m.</td>
<td>FDA Introduction of PFDD Initiative</td>
<td>Stacy Chin, MD, Clinical Team Leader, Division of Pulmonary, Allergy, and Critical Care, Center for Drug Evaluation and Research, U.S. Food and Drug Administration</td>
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<tr>
<td>10:20 - 10:35 a.m.</td>
<td>Clinical and Treatment Overview</td>
<td>Albert Rizzo, MD, FACP, Chief Medical Officer, American Lung Association</td>
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<tr>
<td>10:35 - 10:40 a.m.</td>
<td>Discussion Format Overview</td>
<td>James Valentine, JD, MHS, Meeting Moderator</td>
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<tr>
<td>10:40 - 10:45 a.m.</td>
<td>Getting to Know You Polling</td>
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<tr>
<td></td>
<td><strong>Session 1 Living With Asthma: Symptoms and Daily Impact</strong></td>
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<tr>
<td>10:45 - 11:10 a.m.</td>
<td>Session 1 Panelist Statements</td>
<td>Patient vignettes will show the daily impacts of managing childhood asthma.</td>
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<tr>
<td>11:10 a.m. - 12:30 p.m.</td>
<td>Audience Discussion and Polling</td>
<td>Moderated discussion among meeting participants via Zoom, telephone, or written comments will provide additional perspectives on how asthma impacts daily life.</td>
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<tr>
<td>12:30 - 1:00 p.m.</td>
<td>Lunch Break</td>
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<tr>
<td>1:00 - 1:05 p.m.</td>
<td>Discussion Format Overview</td>
<td>James Valentine, JD, MHS, Meeting Moderator</td>
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<tr>
<td></td>
<td><strong>Session 2 Perspectives on Current and Future Approaches to Asthma Treatment</strong></td>
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<tr>
<td>1:05 - 1:25 p.m.</td>
<td>Session 2 Panelist Statements</td>
<td>Patient vignettes will discuss current treatment options and hopes for future treatments for childhood asthma.</td>
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<tr>
<td>1:25 - 2:40 p.m.</td>
<td>Audience Discussion and Polling</td>
<td>Moderated discussion among meeting participants via Zoom, telephone, or written comments will provide additional perspectives on current and future treatment options for childhood asthma.</td>
</tr>
<tr>
<td>2:40 - 2:50 p.m.</td>
<td>Meeting Summary</td>
<td>Tonya Winders, MBA, President and CEO, Allergy &amp; Asthma Network</td>
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<tr>
<td>2:50 - 3:00 p.m.</td>
<td>Next Steps and Closing Remarks</td>
<td>Mary Jo Strobel, Executive Director, American Partnership for Eosinophilic Disorders</td>
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</tbody>
</table>

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#littleairwaysbigvoices  #asthma  #PFDD
Session 1  Living With Asthma: Symptoms and Daily Impact

1. Of all the symptoms of asthma, which 1-3 symptoms have the most significant impact on your/your child’s life?
   • Which symptoms most affect you or your loved one now?
   • Were there other symptoms that were more significant at other times?
   • Were there other symptoms that you noticed (where you suspected something was off, or when looking back after diagnosis you now believe to be related to asthma)?
   • How does asthma affect you or your loved one’s best days and worst days? Describe your or your loved one’s best days and worst days.

2. How have your or your loved one’s symptoms changed over time? How has the ability to cope with the symptoms changed over time?

3. Are there specific activities that are important to you or your loved one that you or they cannot do at all or as fully as you or they would like because of asthma?
   • How does asthma affect your or your loved one’s daily life? What are the challenges?
   • How does asthma affect life activities (school/work, learning abilities, self-sufficiency, living situation, activities, etc.)?
   • If you or your loved one could do one activity that you or your loved one currently is unable to, what would it be?

4. What do you fear the most as you or your loved one gets older? What worries you most about your or your loved one’s condition?
   • What capabilities are you most concerned about you or your loved one potentially losing while growing older?
   • What frustrates you most about your or your loved one’s condition?

Session 2  Perspectives on Current and Future Approaches to Treatment

1. What are you currently doing to manage your or your loved one’s asthma symptoms?
   • Which specific asthma symptoms do the treatments address?
   • How has this treatment regimen changed over time and why?
   • How satisfied are you with available treatments when it comes to the route of administration (for example, shots, pills, oral treatments, inhaled treatments)? For treatments that are mostly done at the doctor’s office, would you prefer to have them available for administration at home?
   • What challenges do you or your loved one face when it comes to taking medicines as prescribed?
   • Are there any challenges differentiating between medicines (such as controller inhalers vs quick relief inhalers)?

2. How well do these treatments treat the most significant symptoms of asthma?
   • How well do these treatments improve the ability to do specific activities that are important in daily life?

3. What are the most significant downsides to your or your loved one’s current treatments and how do they affect daily life? (Examples of downsides may include bothersome side effects, going to the hospital for treatment, etc.)

4. Short of a complete cure, what specific things would you look for in an ideal treatment for asthma? When considering a new treatment, what factors are important to you?
Session 1 Living With Asthma: Symptoms and Daily Impact

Monique Cooper and Javan Allison
Miami, FL
Javan Allison is 13 years old. He was diagnosed with asthma at age 2. After spending a week in the ICU due to asthma, Javan and his mother Monique Cooper now advocate and raise awareness by sharing their experiences.

Regan Lloyd
Fairfax, VA
Regan Lloyd is a 17-year-old high school senior. She was diagnosed with asthma as a toddler after worsening allergic symptoms. She recounts her journey, how asthma has impacted daily life, severe reactions she’s suffered, and how she tried several medications and treatments over the years to control her asthma.

Nicole Sutton
Tampa, FL
Nicole Sutton is the parent of Nicholas, a 5-year-old boy who was diagnosed with asthma around age 2. She shares her family’s journey with asthma, triggers, and the impact of COVID-19 on asthma-related decisions, such as Nicholas attending school.

Del Principe Family
Bridgewater, NJ
Anne Marie, Larry, and Adrianna Del Principe join us to share the story of their beloved daughter and sister, Alessandra, who was diagnosed with asthma as a teenager. Together, the family recounts the tragic circumstances of the fatal asthma attack Alessandra succumbed to in the spring of 2021 and lament on the need for asthma severity awareness and adherence to treatment plans.

Esther, Chidiogo, and Chidubem Igboerika
Fresno, CA
Esther Igboerika is a school nurse who has two children with asthma. Her son Chidubem was diagnosed at age 4 and her daughter Chidiogo at age 5. Together they share their thoughts on asthma symptoms and the impact the condition has on their daily lives.
Session 2 Perspectives on Current and Future Approaches to Treatment

Jolene MacDonald
South Portland, ME
Jolene MacDonald has an 11-year-old son who has asthma. She talks about the challenges with asthma treatment that her family has experienced, the different doctors involved in her son’s care, and strategies that have helped.

Shreaya Madireddy
Dayton, OH
Shreaya Madireddy is a high school senior who was diagnosed with asthma at age 8. She describes the treatments she has been prescribed to help stabilize her asthma, the restrictions that asthma has placed on daily activities, and how the condition has impacted her quality of life.

Ryan Piansky
Atlanta, GA
Ryan Piansky is a 21-year-old college student who has lived with asthma since age 2. When he was 18, he was diagnosed with a phenotype of asthma called eosinophilic asthma. He joins us to share the impact asthma has on his life, the side effects from the various treatment options he has used, and how he ultimately arrived at a treatment plan to manage his asthma.

Colleen and KE Plunkett
New York, NY
KE Plunkett is an 18-year-old college freshman who has suffered from asthma since birth. He has been hospitalized many times for asthma. His mom, Colleen, recounts KE’s worst asthma attack and discusses the pros and cons to treatments he has had over the years.
MODERATORS & SPEAKERS

MODERATORS

Sanaz Eftekhari
Vice President of Research, Asthma and Allergy Foundation of America

Sanaz Eftekhari is the Vice President for Corporate Affairs and Research at the Asthma and Allergy Foundation of America (AAFA). Sanaz leads tactical and strategic conversations with business partners and stakeholders across multiple industries, including patient advocacy organizations, pharmaceutical companies, medical societies, research firms, and government agencies. She works closely with various stakeholders to collect patient insights, develop programs, and increase awareness of asthma, food allergies, and atopic diseases through a variety of social and communications channels. She currently oversees multiple projects to encourage successful patient-centered research and elevate the patient voice. In 2019, Sanaz partnered with five organizations to cohost More Than Skin Deep, an externally-led patient-focused drug development (EL-PFDD) initiative for atopic dermatitis. Sanaz has also co-authored several of AAFA’s key research reports, such as Asthma Disparities in America, My Life With Food Allergy, My Life With Asthma, and Atopic Dermatitis in America Summary Report.

James Valentine, JD, MHS
Associate, Hyman, Phelps, and McNamara, PC

James Valentine assists medical product industry and patient advocacy organization clients in a wide range of regulatory matters, including new drug and biologic development and approval issues. He also works with clients on clinical trials operations and compliance matters. Prior to 2014, he worked in the FDA’s Office of Health and Constituent Affairs (previously Office of Special Health Issues) where he facilitated patient input in benefit-risk decision-making and served as a liaison to stakeholders on a wide range of regulatory policy issues. He administered the FDA Patient Representative Program, facilitated stakeholder consultations during the reauthorization of PDUFA and MDUFA, helped launch the Patient-Focused Drug Development program, and developed the FDA Patient Network. He also worked at the Center for Drug Evaluation and Research’s (CDER) Office of Regulatory Policy where he coordinated the implementation of the medical gases certification scheme that was established in FDASIA, and handled a variety of postmarket safety issues including REMS and safety labeling changes.
SPEAKERS

Stacy Chin, MD
Clinical Team Leader, Division of Pulmonary, Allergy, and Critical Care, Center for Drug Evaluation and Research, U.S. Food and Drug Administration

Dr. Stacy Chin is a lead medical officer and associate director for biomedical informatics in the Division of Pulmonology, Allergy, and Critical Care within the Office of Inflammation and Immunology, Office of New Drugs, Center for Drug Evaluation and Research at the FDA. She joined the FDA 9 years ago after completing a fellowship in Allergy and Immunology at Duke University Medical Center in 2012. She completed her residency training in pediatrics at Children’s National Medical Center in 2009 and received her medical degree from Georgetown University Medical Center in 2006. Although originally from Arkansas, she now calls Washington, D.C. home.

Kenneth Mendez, MBA
President and CEO, Asthma and Allergy Foundation of America

Kenneth Mendez, MBA, became President and Chief Executive Officer of the Asthma and Allergy Foundation of America (AAFA) in early 2018. He came to AAFA from AdvaMed, the world’s largest medical technology association, where he served as Senior Executive Vice President and Chief Revenue Officer for 12 years. Since joining AAFA, he has led the organization in establishing a new multi-year strategic plan that emphasizes dramatically reducing the impact of asthma and allergies on the underserved. He has an MBA in marketing from Columbia Business School and a BA in American History and American Art from Harvard College. He also has a personal stake in AAFA’s mission as he and two of his children manage asthma and allergies.

Albert Rizzo, MD, FACP
Chief Medical Officer, American Lung Association

Dr. Albert Rizzo, as Chief Medical Officer (CMO) for the American Lung Association, is the organization’s senior medical authority. Dr. Rizzo has long been a key medical advisor to the American Lung Association, a member of the Lung Cancer Expert Medical Advisory Panel and a leading media spokesperson for the Association. In his role as CMO, Dr. Rizzo plays a key role in multiple areas of their mission, including the American Lung Association’s Lung HelpLine, research, including the Airways Clinical Research Centers and the Awards and Grants program, as well as advocacy, communications, development, and health promotions. Dr. Rizzo is also the former chief of the Section of Pulmonary and Critical Care Medicine at Christiana Care Health System in Newark, Delaware and continues to practice as a member of Christiana Care Pulmonary Associates. He is board certified in internal medicine, pulmonary, critical care, and sleep medicine and is a clinical assistant professor of medicine at Thomas Jefferson University Medical School in Philadelphia where he obtained his medical degree and completed his residency in internal medicine. Dr. Rizzo received his specialty training at Georgetown University Hospital in Washington, D.C. He is a member of the American Thoracic Society, a Fellow of the American College of Chest Physicians, a Fellow of the American College of Physicians, a Diplomat of the American Board of Sleep Medicine, and a Fellow of the American Association of Sleep Medicine.
Mary Jo Strobel
Executive Director, American Partnership for Eosinophilic Disorders

Mary Jo Strobel has nearly 25 years of professional experience in the nonprofit sector with a specific focus on patient advocacy for allergic conditions. She lives near Washington, D.C. and serves as the Executive Director of the American Partnership for Eosinophilic Disorders (APFED), having been in this role since 2012. She has a broad background and direct experience in a multitude of initiatives related to patient education, disease awareness, advocacy, and research. She collaborates with research investigators, facilitates patient experience data, advises on recruitment strategies, and contributes to research design and protocol. She has co-authored numerous research abstracts and manuscripts that have been published in journals. In addition to invitational speaking engagements around the U.S. and abroad to share insights and information to patients, providers, industry, government agencies, and other nonprofits, she also represents APFED as a member of the Consortium of Eosinophilic Gastrointestinal Disease Researchers; the Rare Disease Clinical Research Network Coalition of Patient Advocacy Groups; the Lay Organization Strategic Partnership of the American Academy of Allergy, Asthma & Immunology; and in several national and global collaboratives to identify and address unmet needs of patients with eosinophil-associated disease.

Tonya Winders, MBA
President and CEO, Allergy & Asthma Network

Tonya Winders, MBA, is currently the President and Chief Executive Officer of the Allergy & Asthma Network, the leading patient advocacy organization dedicated to ending the needless death and suffering due to asthma, allergies, and related conditions. Tonya has over 20 years experience in leadership roles within the allergy and asthma industry. From sales and marketing leadership to managed markets access, she has worked tirelessly to ensure patients have access to effective diagnostic and treatment tools. Tonya serves on several expert panels including the American Thoracic Society Public Advisory Roundtable and Global Initiative for Asthma international guidelines. Furthermore, she serves on the board of directors for the Alliance to Prevent Legionnaires Disease and the American Respiratory Care Foundation and is the current president of the Global Allergy & Airways Patient Platform, representing more than 50 patient organizations throughout the world. Personally, Tonya is the mother of five children, four of whom have asthma and/or allergies, ranging in age from 15-22 years old. She enjoys spending time with her husband of more than 20 years Brian Winders and cheering on her children in various sports.

Submit Written Comments
Share your experiences with asthma in childhood by submitting written comments.

littleairwaysbigvoices.org/take-action
DISCUSSION STARTERS

We are deeply grateful to the following community members for sharing their insights and kicking off live discussions:

**Session 1**

- Charmayne Anderson  
  Caregiver
- Mrs. K.A. Hoskinson and her son Charles  
  Family managing asthma
- Ty-Eisha Rivera  
  Caregiver
- Jennifer Roeder  
  Caregiver
- Shiv Sewlal  
  Living with asthma

**Session 2**

- Amanda Eisen  
  Caregiver
- Nicole Gyimah  
  Caregiver
- Andrea Jensen  
  Caregiver
- LaJoy Johnson-Law  
  Caregiver
- SPC Okino Thompson  
  Living with asthma
ACKNOWLEDGMENTS

Thank you to the following individuals for supporting the Little Airways, Big Voices initiative:

Larry Bauer of Hyman, Phelps, & McNamara, PC for strategic counsel about PFDD meetings, presenting at the August 11, 2021, community webinar introducing EL-PFDD meetings, and supporting patient and caregiver panelists as they prepared their testimony.

Taylor Desens of Taylor Does Marketing for designing the Little Airways, Big Voices logo, website, and social media graphics in preparation for today’s meeting.

John Dudley, Eric Quigley, & Kyle Rhoderick of Dudley Digital Works for creative and technical services to broadcast the meeting to a live remote audience and preserve it for later on-demand viewing.

Jennifer Roeder of Roeder Creative for strategic communications and outreach support for the initiative, and the design of this program. In partnership with the collaborative, she will assist writing the Voice of the Patient report.

U.S. Food and Drug Administration for their commitment to incorporating the patient voice in drug development and evaluation. We would especially like to thank William Lewallen of the Center for Drug Evaluation and Research for his guidance and support as we planned this meeting.

James Valentine of Hyman, Phelps & McNamara, PC for strategic counsel about PFDD meetings, preparing panelists to convey their experiences, and moderating today’s meeting.

ADDITIONAL SUPPORT

Michele Cassalia
Director of Partnership and Alliances
Asthma and Allergy Foundation of America

Hannah Jaffee
Research Analyst
Asthma and Allergy Foundation of America

Joy Meyer
National Vice President, Health Promotion
American Lung Association

Cara Schmitt
Program and Services Coordinator
American Partnership for Eosinophilic Disorders

The Allergy & Asthma Network, American Lung Association, American Partnership for Eosinophilic Disorders, and Asthma and Allergy Foundation of America’s community and marketing/communications teams for outreach support in preparation for today’s meeting.
The organizations involved in the **Little Airways, Big Voices** initiative each have a history of providing support for patients and caregivers with asthma. Together, the organizations bring significant expertise, experience, and influence.

The collective history of hosting events bringing patients, caregivers, and patient advocates together with other healthcare stakeholders shows a commitment to elevating the patient voice in research and drug development.

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**Allergy & Asthma Network** is the leading nonprofit patient education and advocacy organization for people with asthma, allergies, and related conditions. The patient-centered network unites individuals, families, healthcare professionals, industry, and government decision makers to improve health and quality of life for Americans with asthma and allergies. The organization specializes in making accurate medical information relevant and understandable to all while promoting evidence-based standards of care.

Learn more: allergyasthmanetwork.org

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**American Lung Association** is the leading organization working to save lives by improving lung health and preventing lung disease, through research, education, and advocacy. Their work is focused on four strategic imperatives: to defeat lung cancer; champion clean air for all; improve the quality of life for those with lung disease and their families; and create a tobacco-free future.

Learn more: lung.org

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**American Partnership for Eosinophilic Disorders (APFED)** is a non-profit organization whose mission is to passionately embrace, support, and improve the lives of patients and families affected by eosinophil-associated diseases through education and awareness, research, support, and advocacy.

Learn more: apfed.org

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Founded in 1953, the **Asthma and Allergy Foundation of America (AAFA)** is the oldest and largest non-profit patient organization dedicated to saving lives and reducing the burden of disease for people with asthma, allergies and related conditions through research, education, advocacy, and support. AAFA offers extensive support for individuals and families affected by asthma and allergic diseases, such as food allergies and atopic dermatitis (eczema). Through its online patient support communities, network of local chapters and affiliated support groups, AAFA empowers patients and their families by providing practical, evidence-based information, and community programs and services.

Learn more: aafa.org
Thank you to the following sponsors for helping to make the Little Airways, Big Voices initiative possible.

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