angelman™
SYNDROME FOUNDATION

2022 ANNUAL REPORT

DEAR ASF COMMUNITY,

This year has been a special year as we have been able to celebrate 30 years of Angelman Syndrome Foundation serving our community.

Reflecting through the year, we had a chance to meet with the founders of this organization. We are so humbled by their leadership and thankful for the vision they cast 30 years ago.

Now as we reflect on an amazing year and look forward, we are proud to say we have made tremendous progress on our mission. New family supports have been added, multiple ASF Clinics have been created and millions of dollars in research have been given to get us closer to therapeutic treatments.

We are so thankful for you—each of you who have felt the power of our mission and have been compelled to get involved. Thank you for organizing walks, special events in your hometowns, Facebook fundraisers and so much more. Thank you to each of those who have raised their hands and answered the call to volunteer for a committee, support a newly diagnosed family or volunteer at our conferences. It is because of you that we can serve the community and meet each individual where they are in their journey.

We still have work to do. Our journey isn't complete until we've overcome every challenge our loved ones with Angelman syndrome experience. Until then, we promise we will never stop.

Sincerely,
KYLE ROONEY, Board of Directors President
AMANDA MOORE, CEO

Celebrating 30 Years of Service to the AS Community

2022 MILESTONES

1. LAUNCHED LADDER LEARNING NETWORK

In a strategic collaboration with Dup15q Alliance, we established the first network of medical providers specializing in 15q syndromes. Cross-disorder investigation is, and will remain, key to driving discovery.

2. OPENED FOUR NEW ASF CLINICS

Many families expressed difficulty finding doctors who understand the complexities of AS. We now have a total of 25 ASF Clinics worldwide to connect those who have experience treating AS to families who need it most.

3. FUNDED SEVEN RESEARCH PROPOSALS

Research is key to improving the lives of individuals with AS. In 2022, we funded more than $1 million in studies for ASO treatments, gut health, parent-infant interaction, motor skills impairments and more.

4. ESTABLISHED THE JACOB PRITZKER FELLOWSHIP PROGRAM

As need for medical providers specializing in AS continues to grow, we established this fellowship to support a junior investigator or clinical training program for an early-career physician.

5. HOSTED THE BIGGEST ASF FAMILY CONFERENCE EVER!

We reunited the ASF community at Kalahari Resorts in Texas for the most largely attended ASF Family Conference in history. We welcomed 1,000+ attendees, 50+ speakers and 245+ industry members.

6. LAUNCHED THE ASF PODCAST

As another way to help families on their journey, we launched the ASF Podcast. Discussion from experts touch on topics ranging from genetics to siblings stories to clinical treatments for seizures.

7. LAUNCHED STRATEGIC PARTNERSHIPS

Knowledge comes when forces combine. We established partnerships with Ambit Care, PAN Foundation and other Angelman syndrome organizations across the globe. (See Page 12)

8. SUPPORTED 135 FAMILIES WITH THE ASF FAMILY FUND

This year, we provided $252,853 to support families with items needed to improve quality of life. Awards included items like safety beds, strollers, AAC devices and travel grants to visit an ASF Clinic.

9. WALKED TO RAISE MORE THAN $1.2 MILLION!

Our annual ASF Walk drew 7,158 registrants across 48 locations nationwide. These "game changers" not only raised awareness and funds, they continued to keep our growing community strong.

FAMILY SUPPORT

SUPPORTING ASF FAMILIES FOR 30+ YEARS

ASF is committed to supporting families on their journey through its abundance of resources and programs. Through grants, committees, contacts, checklists, planning tools, webinars, meetings and more, we steadfastly provide support when and where families need it most.

2022 FAMILY SUPPORT HIGHLIGHTS

ASF FAMILY CONFERENCE:
1,000 ATTENDEES — the largest turnout to date

COUNSELING SERVICES:
PROVIDED 759 free counseling sessions for AS families

ASF FAMILY FUND:
135 grants awarded, totaling **$252,853**

ASF FAMILY CHAMPIONS:
54 generous family volunteers

PODCAST:
Launched Season 1 with **8** episodes

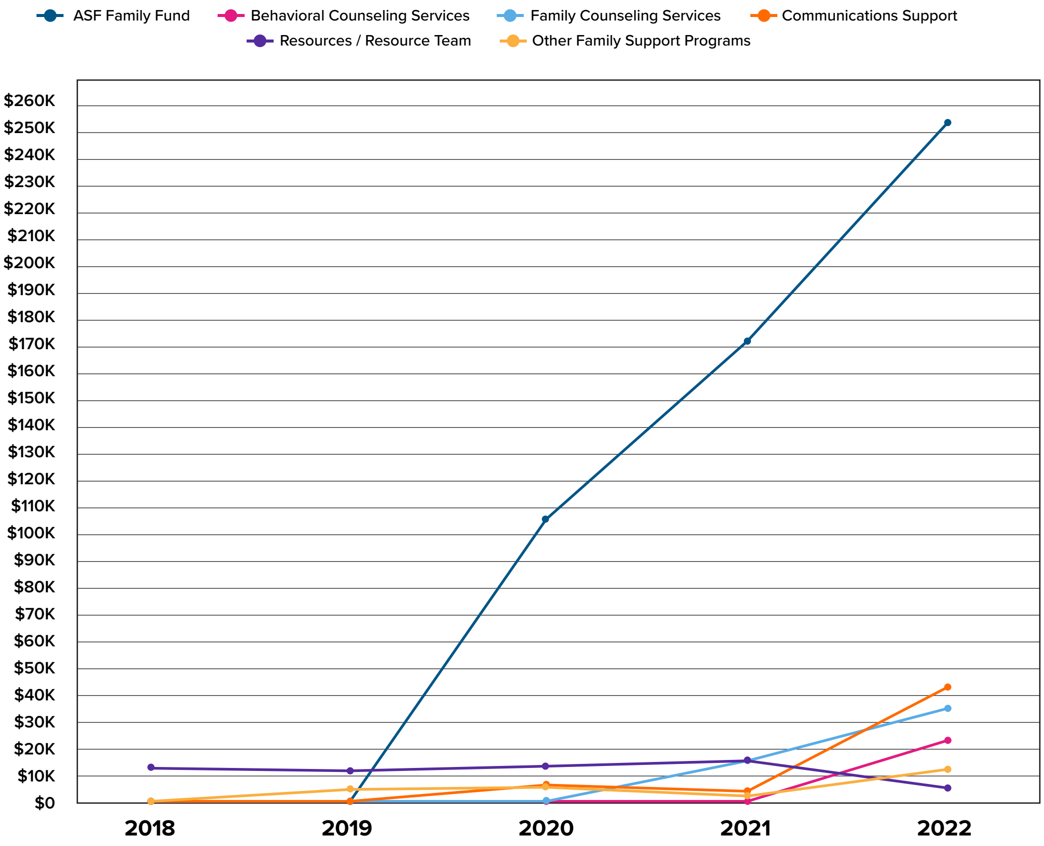
"I was not scared when my sister with AS had a seizure for the first time. I knew what to do because I learned it at the ASF Conference during the Sib Shops." — Mason Chesser, Sibling

Adults with Angelman syndrome and their families have unique needs. Families often feel alone and afraid due to the scarcity of support and services for adults. ASF, through adult services, is dedicated to using a pragmatic approach to continue to work toward meeting the needs of our adults with AS and their families."
— Robin Wilkerson, Parent

"I struggle to find the right words to express our thanks for the support from the ASF Family Fund. As we age, it gets harder to manage some of the simplest things in life, like going for a walk as a family. ASF Family Fund helped us acquire a heavy duty portable power chair for David. Now we can enjoy walks together and not risk our own heath pushing him. He LOVES it!!"
— Dawn Bly, Parent

ASF FAMILY SUPPORT CONTINUES TO GROW

Core to our mission, we provided $372,293 to family support programs in 2022, with a significant focus on the ASF Family Fund to help improve quality of life.



ASF FAMILY SUPPORT PROGRAMS

ASF continues to expand its Family Support resources and programs to help families during all stages of life.

Read information on each at angelman.org/resources

ASF Family Fund

Counseling Services

Communications Support

Family Resource Team

Adult Services

Sibling Resources

Care Committee

Sunshine Committee

The Center for Courageous Kids

Mentoring Program

RESEARCH

ASF has invested more than $12.9 million in AS research to date, supporting projects worldwide in the quest to find treatments and ultimately a cure.

TYPES OF RESEARCH WE FUND

1. High-risk, high-reward strategies to find a cure, including topoisomerase inhibitors, ASOs, gene therapy and CRISPR
2. Clinical studies to alleviate symptoms and improve quality of life
3. Studies that help us learn about UBE3A and what it does in the brain

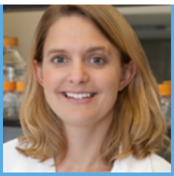
OUR FUNDING PHILOSOPHY

ASF focuses heavily on pilot funding. Essentially, we use a small amount of money to test drive an idea. If the test drive looks good, the researcher is then able to ask larger agencies, like the National Institutes of Health, Simons Foundation and other pharmaceutical companies to provide more funding to expand the project. Every pivotal idea started with pilot funding.

When we choose projects to fund, we do so with the knowledge that incremental studies enable the next big leaps. The $15 million in research we have funded has led to others contributing an additional +$200 million to the cause.

Instead leveraging all our resources on one huge idea, this year we funded seven studies. To use a sports analogy, not every study has to be a home run to make an impact. Four base hits also leads to a run scored.

Treatments resulting from our investment in research have helped individuals with AS learn to walk, communicate and live better lives today. But we're not stopping now. We believe AS can be cured, so that those we love can lead better lives tomorrow.



> "ASF has funded the novel ideas, that became bigger ideas, that then became therapeutics. Without this small amount of funding from ASF, advancements would not happen."
>
> — Stormy Chamberlain, PhD
> Section Head Neurogenomics at Roche
> Member of ASF Scientific Advisory Committee

ASF-FUNDED RESEARCH HIGHLIGHTS

+$15M	Research Investment since 1996**
	7 New Research Studies Funded in 2022
+$1M	In Research Funded in 2022*
45	Clinical Studies Funded at +$2.9 Million**
79	Translational/Applied Studies Funded at +$9.9 Million**

**Total includes first- and second-year grants from current and/or prior year funding rounds. Full award amount is not fully expensed during a fiscal year if the study is multi-years.*

***Cumulative research investment*

NEW STUDIES FUNDED IN 2022

Twice a year, scientists, clinicians and educators from all over the world bring their creative project ideas to ASF in the form of grant proposals. The ASF Scientific Advisory Committee reviews the proposals and chooses to fund projects that are likely to provide the biggest impact on the daily life of individuals with AS and/or an eventual cure.

Newly funded studies in 2022 include:

- Adapting The Parent and Infant Inter(X)Action Intervention for Angelman Syndrome
- Angelman and The Gut
- Recruitment and Deep Pheno typing of Infants with Angelman Syndrome to Enable Early Treatment
- Pilot Study to Understand Skilled Motor Impairments in Angelman Syndrome
- ASO Treatments for a Better Understanding of AS Pathophysiology and Optimizing Therapeutic Efficacy

ASF RESEARCH STEPPING STONES

Since funding its first clinical trial in 1996, ASF-funded research has continued to lay the foundation for future discovery.

UBE3A

ASF funded the first studies of the UBE3A gene. Impairment of this gene causes AS, and these studies helped us better understand how, when and where UBE3A is made. Incremental studies have since followed to understand UBE3A targets, to understand its gene and protein regulation and to understand the structure of the protein.

MOUSE MODELS

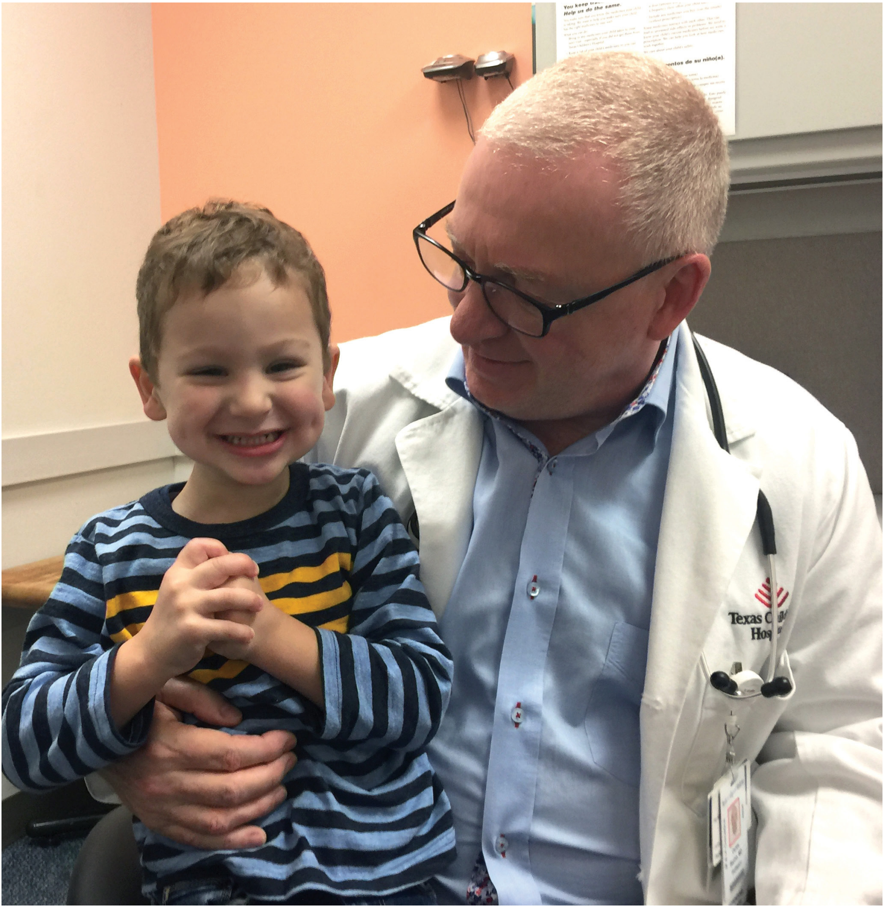
ASF funded the first mouse models to help with safe testing of potential therapies and to show how different brain regions contribute to AS symptoms. ASF-funded mouse studies proved that earlier restoration of UBE3A would give better outcomes following genetic therapies.

GENE THERAPY

ASF funded the first gene therapy study. This led to funding two more innovative twists to make an optimal gene therapy. These studies, along with the gene activation strategies, have attracted several pharmaceutical companies to develop potential therapies for AS.

ASO STUDIES

ASF funded the first antisense oligonucleotide (ASO) studies proving that the therapeutic approach would work, which led directly to the three clinical trials by Ionis, Roche and Ultragenyx/Genetx. These studies will help answer questions like: How much ASO and how much UBE3A protein is needed? Is ASO treatment required throughout life? When does ASO treatment need to start?



angelman SYNDROME FOUNDATION Clinics

FOR PATIENTS. FOR PROVIDERS. TOGETHER, TOWARD THE CURE.

In 2022, ASF and the DUP15q Alliance partnered to launch the LADDER Learning Network to provide high-quality medical care for individuals with AS or Dup15q. The network connects those who practice evidence-based medicine with a high level of excellence for these two rare syndromes. Then, we ensure as many families have access to it as possible through ASF Clinics or Dup15q Clinics worldwide.

2022 ASF CLINICS HIGHLIGHTS

4 NEW
ASF Clinics
Opened

25 ASF CLINICS
Now Exist
Worldwide

50,000 DATA POINTS
In The LADDER
Database

ESTABLISHED
AS Center of Excellence
Designation

LADDER LEARNING NETWORK ESSENTIAL FUNCTIONS

1. CONNECTS PATIENTS TO CARE

Connects AS/dup15q individuals to comprehensive care from highly engaged, highly specialized medical experts who have demonstrated a passion for and an ability to specifically treat these rare disorders.

2. CONNECTS PROVIDERS TO ONE ANOTHER

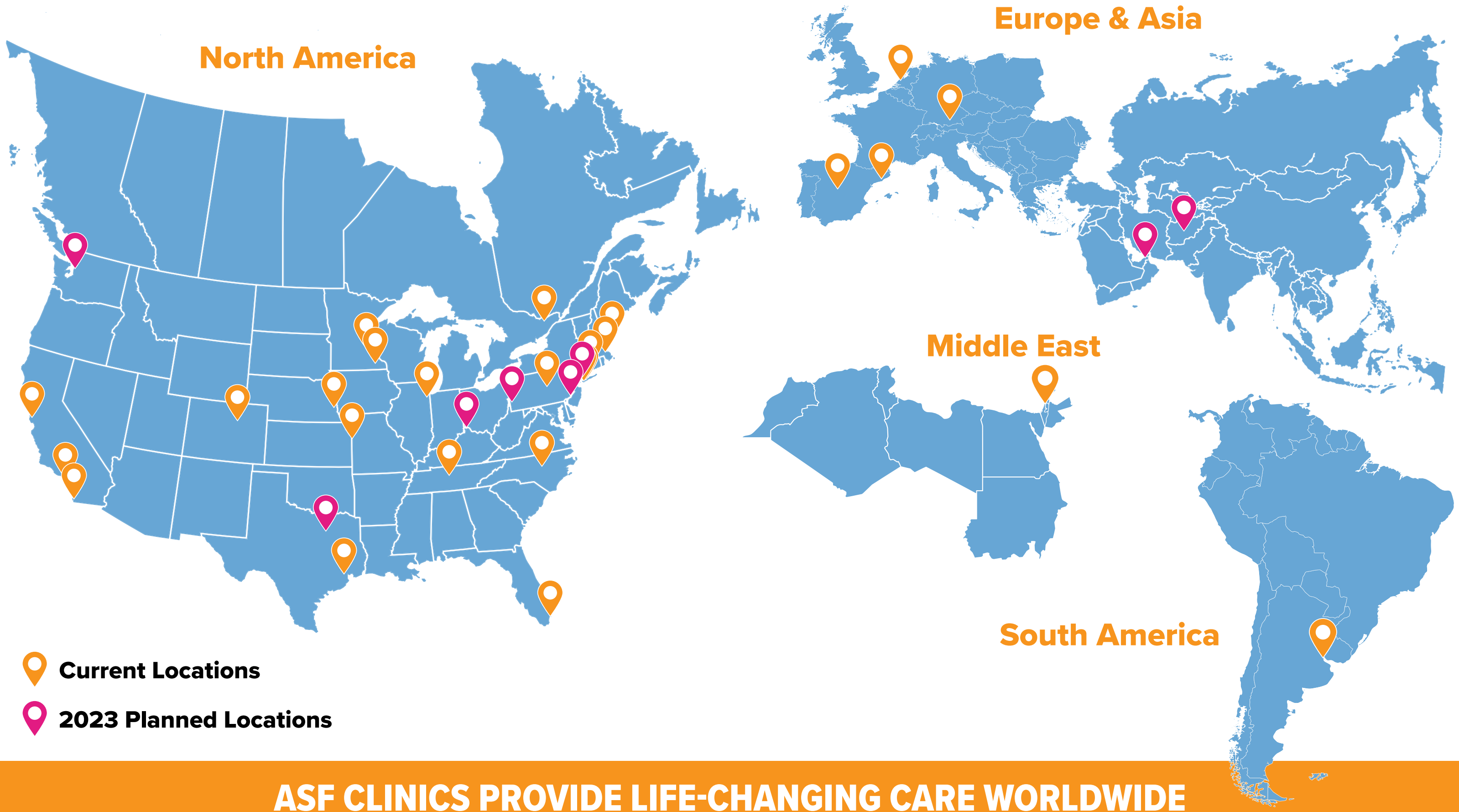
Serves as an avenue for communication among 40+ AS/dup15q experts across the globe to share knowledge, discuss challenging cases and to progress toward standardizing care.

3. SUPPORTS CLINICAL TRIALS

Provides a foundation to support clinical trials by having established sites with experts and patients in place to conduct those trials when they become available.

4. OPERATES LADDER DATABASE

Operates the global network of patient-powered data, to enhance understanding of both syndromes. Increased insight into data from AS/dup15q individuals informs better treatment options and will pave the way for a cure.



ASF CLINICS PROVIDE LIFE-CHANGING CARE WORLDWIDE

WITH SEIZURES CONTROLLED, ASF CLINICS HELPED OUR DAUGHTER GROW

“Emily had her first seizure at a year old. Before visiting the ASF Clinic at Children's Hospital Colorado, she was having multiple seizures in a 24-hour period once a month. At the clinic, she saw a team of doctors, therapists and other specialists who focus on caring for kids with AS.

They gave Emily a new seizure medication and developed a care plan to reduce her seizures and aid her development. She hasn’t had a seizure since November 2021. It is a dream come true!”

— The Waibel Family

“ASF Clinics are the ultimate resource for the AS community. Families can access expert care, access clinical trials and access future treatments for those living with AS. Clinics are one of the key resources needed for this community, and we are so thankful that ASF answered that call.”

Ron Thibert, DO, MSPH
ASF Clinician at Massachusetts
General Hospital

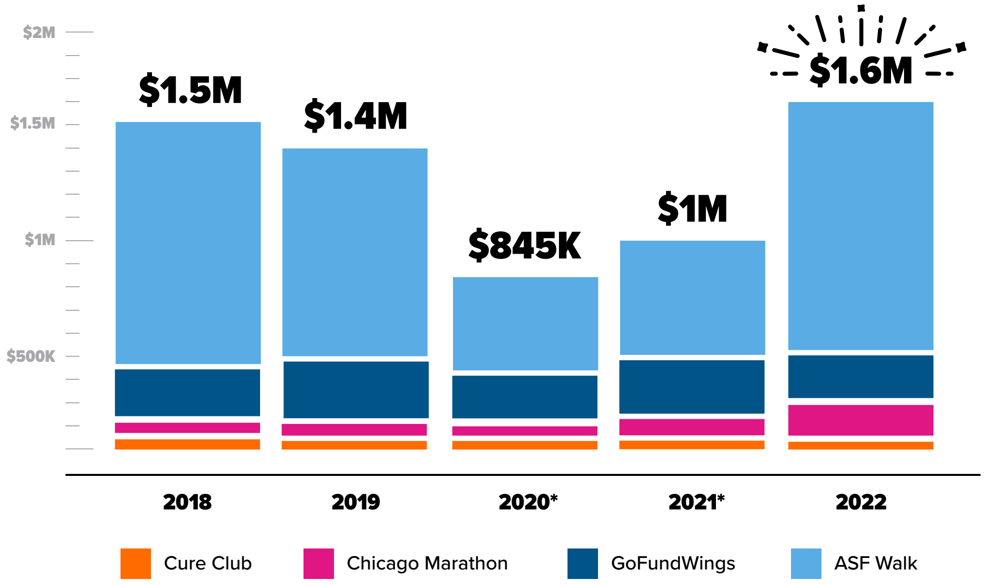
FUNDRAISERS

ASF WALK | *Raised $1,259,601*

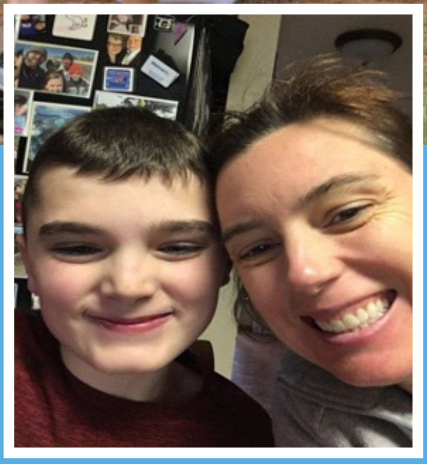
The 2022 annual ASF Walk drew 7,158 registrants across 48 locations nationwide. It raised the most funds since 2018 and continues to be ASF's largest fundraiser. These "game changers" not only raised awareness and funds, they continued to keep our growing community strong.

FAMILIES MAKE AN IMPACT

Without our community, none of the breakthrough work we do would be possible. Angelman families gave back in a big way in 2022, raising more than $1.6 million through fundraising opportunities. The passion and drive of these fundraisers move us even closer to life-changing treatments for AS.



*Fundraising efforts affected by Covid-19 pandemic



"When our family participated in our first ASF Walk in 2014, it was in Naperville, Il. My son Gavin was three years old. We felt so vulnerable as parents of a child with a lifelong disability. I couldn't even talk about Angelman syndrome without crying. Our family and many friends were at the Walk to support us. After that first event, I said, 'Someday we'll host an ASF Walk in our hometown of Bettendorf.' Ten years since receiving Gavin's diagnosis, 2023 is our year. We aren't raw and vulnerable anymore. We are loud and proud of all that Gavin has accomplished and hope to pass on that same spirit to others." — Julie Stabb, Parent

GOFUNDWINGS

Raised $190,316

Fundraisers organized by families and friends collectively raised more than $190,000 in 2022. Fueled by their love for someone with AS, they've done great things for ASF and had fun while doing it!

SOME INSPIRING FUNDRAISERS

Quinn's Hot Cocoa For A Cure
Raised $266,000*
Los Angeles, CA

Chip Away at AS Golf Tournament
Raised $44,716*
Atlanta, GA

Labor Day Tractor Cruise
Raised $49,260*
Everest, KS

24 & 27 Hours of Crossfit
Raised $21,392*
Parker, OH

Centercourt for Lacrosse Tournament
Raised $12,203*
Mount Olive, IN

Suds For Silas
Raised $11,763*
Nashville, TN

Cumulative totals to date

BANK OF AMERICA CHICAGO MARATHON

Raised $125,974.16

25 runners joined the Windy City Angels team at the 2022 Bank of America Chicago Marathon. Together, they raised nearly $126,000.

Raised $39,840

The ASF Cure Club is a special group of givers who make a huge impact by setting up recurring monthly donations. As we know, those smaller gifts really add up! In 2022, the ASF Cure Club raised the most it has since 2018. Incremental donations from 88 members provided nearly $40,000 in funding to ASF in 2022. Since the program began in 2016, it has raised nearly $195,000.

BOARD, COMMITTEE & ADVISORY MEMBERS

BOARD OF DIRECTORS

Kyle Rooney, President
Lesley McCallister, Vice President
Michael Cecere, Treasurer
Pete England, Secretary
Eric Wright, Director
Anna Blanding, Director
Susan Ravellette, Director
Jim Lamb, Director
Shannon Moyer, Director
Rebecca Burdine, SAC Chair
Marianne Benet, Director
Andrew Oberwager, MD, CFA, Director
Charles Winslow, Director
Melinda McBride, Director
John Sugden, Director
David Routh, Director
William Rakoczy, Director
Kathy Rokita, Past President
Dan Harvey, Non-voting Consultant

SCIENTIFIC ADVISORY COMMITTEE

Rebecca Burdine, PhD, Chair
Stormy Chamberlain, PhD
Arthur Beaudet, MD
Charles Williams, MD
Ben Philpot, PhD
Michael Ehlers, MD, PhD
Dan Harvey, PhD
Jane Summers, PhD
Wen-Hann Tan, MD
Mark Nespeca, MD
Ron Thibert, DO, MSPH
Katharine Grugan, PhD
Elizabeth Berry-Kravis MD, PhD
Joseph P Horrigan, MD
Anjali Sadhwani, PhD
Jason Yi, PhD
Martin Scheffner, PhD
Christy Zigler, PhD
Anne Wheeler, PhD
Andrew Oberwager, MD, CFA

PARENT ADVISORY COMMITTEE

Tim Bousum
Jim & Jenn Kubicza
Stacey Davis
Kendra Garding
Karen Hill
Morgan Leao
Jennifer Marshall
Steven Mastrocola
Alyson Sinclair
Chelsea Smith
Nate Smith
Courtney Swafford
Regina Uribe

COMMUNICATION ADVISORY COMMITTEE

Dan Harvey, PhD
Stephen Calculator, PhD
Jane Summers, PhD
Erin Sheldon, M.ED
Penelope Hatch, PhD
Carole Zangari, PhD, CCC-SLP
Rose Sevcik, PhD
Courtney Castelli, EDS

OUR PARTNERS

ASF is proud to partner with groups and organizations whose values and services align with our mission.

Ambit Care
Angelman Syndrome Alliance
Angelman UK
Autism Brain Net
Canadian Angelman Syndrome Society
DEE-P Connections
Dup15q Alliance
EveryLife Foundation
Families in United Arab Emirates
FAST
Global Genes
IONIS Pharmaceuticals
National Organization for Rare Disorders
OVID Therapeutics
Parent Empowerment Workshops
PAN Foundation
PTC Therapeutics
Rare Epilepsy Network
Rare and Ready
Roche/Genentech
Ultragenyx

THANK YOU TO OUR VISIONARY LEADERS

Fred Pritzker Visionaries $250,000+

Renee Pritzker and Fred Pritzker*

Betty J. Shaw Benefactors $100,000+

Ionis Pharmaceuticals

Harry Angelman Champions $50,000+

Ultragenyx Pharmaceutical, Inc

Heroes Giving Hope $10,000+

Anonymous Donor, Antonieta Arango, Arthur and Marjorie Beaudet, Janet and Michael Bell, Biogen U.S., Four Apple Cross, LLC, Genentech, Inc., Gloria Gibson, Merewyn and Alan Harrington, HundredX Inc., J's and G's Golf, James and Renee Kizziar, Bonnie and Dennis Knoedler, Michael and Emily Leighton, James and Ana Libby, Agatha and Steve Luczo, Lesley and Drew McCallister, Tim and Gretchen McCarty, Tom and Ruthann McCarty, Ovid Therapeutics, Clint and Lia Perryman, Radius Health, Rakoczy Molino Mazzochi Siwik, LLP, David and Jenny Routh, Texas Children's Hospital, Bryan Schnick and Allyson Tole, Alexander and Sierra Smith, Victor and Clara C. Battin Foundation

Sustainers $5,000+

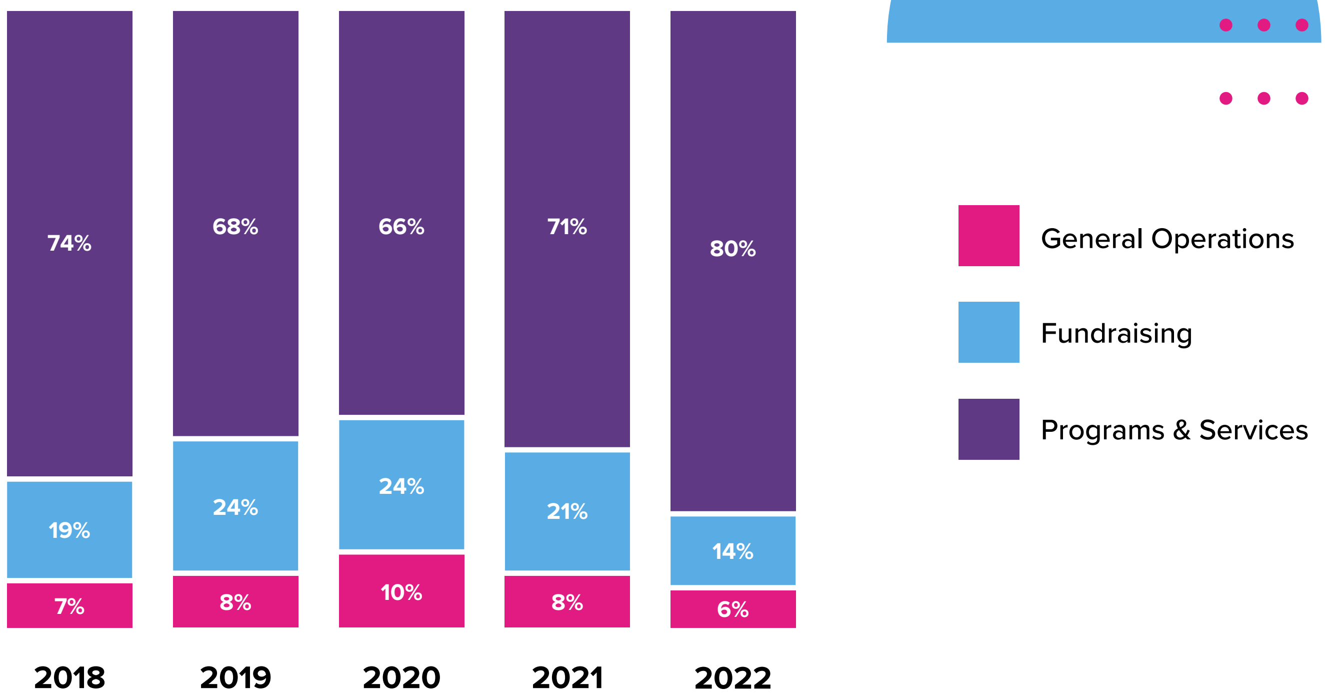
Acco Brands USA, LLC, Anonymous Donor, John and Christine Bakalar, Barbara L. Griffith Fund, Lincoln Benet, Anna Blanding and Garfield Pilliner, William and Katherine Burford, Richard and Ann Carr, Michael and Lori-An Cecere, James and Susan Edberg, Randy and Betty Eisenbach, Enterprise Holdings Foundation, Eubanks Humphrey Engineering, EveryLife Foundation for Rare Diseases, Genetx Biotherapeutics, LLC, Dan and Karen Harvey, Christian and Hilary Hommes, David Lloyd Kennell and Mary Clare Lindsay, Lea Family Fund, David and Laurie Morren, Janet and William Neal, John R. Niepold, Christian Pache and Anna Hsu, William A. Paridon*, Clint W. Perryman P.C., Richard Piluso, John and Mary Pipal, Wayne and Jana Pott, PTC Therapeutics, Inc., Deirdre and Mark Rajkowski, Susan and Steven Ravellette, Martin and Angela Reggette, David Rosenblatt and Karen Hoerst, Jonathan Ruckdeschel, Dudley Ryan, Pamela Scholten, Setpoint Integrated Solutions, Inc., John Sugden and Ana Monaldi, Jonathan Timbers, UAW Local 2209, U.S. Anesthesia Partners

Donations received Oct 2021 - Sept 2022

**in memoriam*

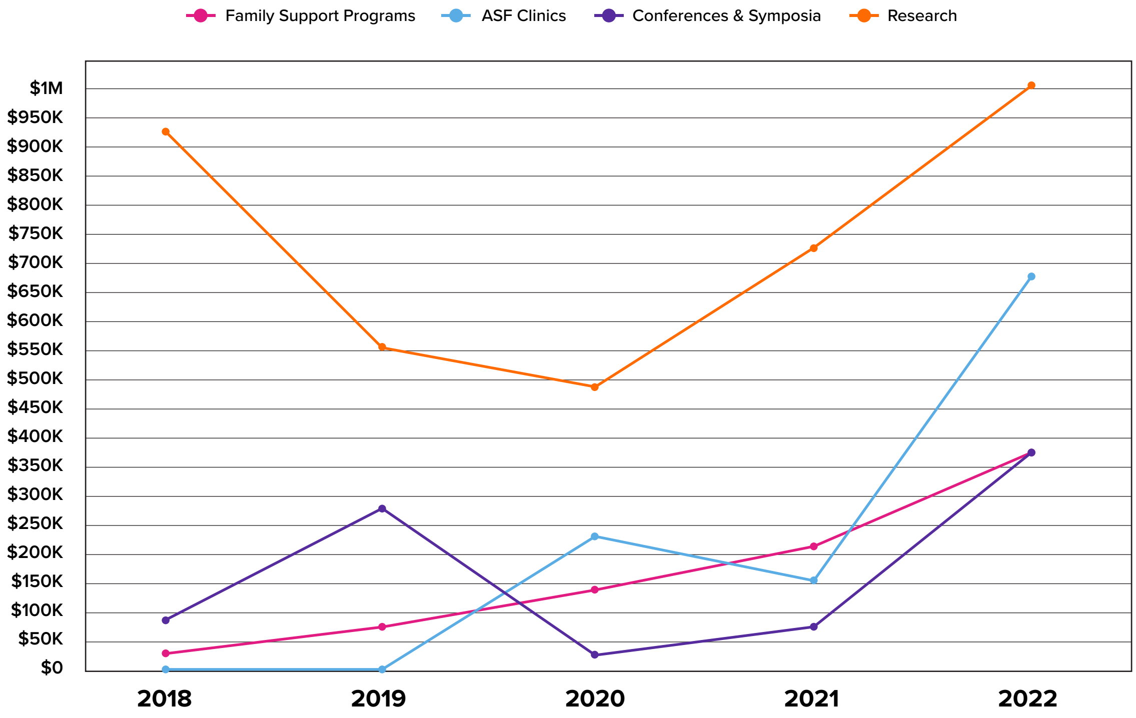
WHERE YOUR CONTRIBUTIONS GO

80% of every dollar spent in 2022 funded programs and services core to our mission.



HISTORICAL TRENDS

Annual expenses allocated to our core Programs and Services continue to show our commitment to family support, educating our community and to scientific discovery.



FINANCIAL BREAKDOWN

STATEMENT OF FINANCIAL POSITION
FOR THE YEAR ENDED SEPTEMBER 30, 2022 AND 2021

Current Assets:	2022	2021
Cash & Cash Equivalents	$1,214,351	$918,772
Pledges Receivable, Current Portion	$10,000	------
Investments	$786,039	$897,812
Prepaid Expenses	$49,326	$21,321
Total Current Assets	**$2,059,716**	**$1,837,905**

Property & Equipment (at cost):	2022	2021
Office Furniture, Fixtures, & Equipment	$2,921	$2,921
Computer Hardware	$14,216	$11,216
LADDER	$533,285	$533,285
Website	$24,000	$24,000
Less - Accumulated Depreciation	($424,490)	($267,142)
NET Property & Equipment	**$149,932**	**$304,280**

Other Assets:	2022	2021
Pledges Receivable, Long-Term Portion	$40,000	------
TOTAL ASSETS	**$2,249,648**	**$2,142,185**

Current Liabilities:	2022	2021
Accounts Payable	$216,417	$60,901
Accrued Payroll Expenses	$49,357	$45,771
Total Current Liabilities	**$265,774**	**$106,672**

Long-Term Liabilities:	2022	2021
Note Payable	------	$87,000
Total Liabilities	**$265,774**	**$193,672**

NET Assets:	2022	2021
Total Current Assets	**$1,983,874**	**$1,948,513**
TOTAL LIABILITIES & NET ASSETS	**$2,249,648**	**$2,142,185**

STATEMENT OF ACTIVITIES
FOR THE YEAR ENDED SEPTEMBER 30, 2022 AND 2021

PUBLIC SUPPORT & REVENUE

	2022	2021
Contributions	$2,336,706	$1,377,328
Biennial Conference	$152,724	$96,121
Special Events *(Net of related expenses which includes in-kind revenue of $206,733 and $73,086 for 2022 and 2021 respectively)*	$1,204,052	$647,841
Net Investment Return (Loss)	($107,929)	$125,990
Other Income	$3,626	$11,552
Forgiveness of PPP Loan	$87,000	$87,000
Loss on Disposal of Fixed Assets	------	($1,067)
Net Assets Released From Restrictions - Satisfaction of Program Restrictions	------	------
TOTAL PUBLIC SUPPORT & REVENUE	**$3,676,179**	**$2,344,765**

FUNCTIONAL EXPENSES

Program Services	2022	2021
Family Support	$1,464,448	$710,897
Research	$1,061,322	$739,010
Biennial Conference & Symposium	$375,340	$69,231
TOTAL PROGRAM SERVICES	**$2,901,110**	**$1,519,138**
Management & General	$170,322	$219,898
Fundraising	$421,937	$519,810
TOTAL FUNCTIONAL EXPENSES	**$3,640,818**	**$2,111,397**

NET ASSETS

	2022	2021
Change In Net Assets	$35,361	$233,368
Net Assets, *Beginning Of Year*	$1,948,513	$1,715,145
Net Assets, *End Of Year*	$1,983,874	$1,948,513

Financial information presented here has been summarized from financial statements audited by Dugan + Lopatka dated September 30, 2022 and 2021.

The mission of the Angelman Syndrome Foundation is to advance the awareness and treatment of Angelman syndrome through education and information, research, and support for individuals with Angelman syndrome, their families and other concerned parties. We exist to give all of them a reason to smile, with the ultimate goal of finding a cure.

Info@Angelman.org | 1-800-432-6435 | 3015 E. New York St. Suite A2 #285, Aurora, IL 60504

©2023 Angelman Syndrome Foundation. All Rights Reserved.