2018
ANNUAL REPORT

angelman
SYNDROME FOUNDATION
give them a reason to smile.

DEAR FRIENDS AND FAMILY,

What a year! Over the last 12 months, we have accomplished so much: a record-breaking Walk, groundbreaking new research, and four new Angelman Syndrome Clinics in the U.S. And it's all because of our tremendous community of supporters. With new clinics in New York, Nashville, Houston and Chicago, we are able to accelerate research that is catapulting us closer to a cure while offering more AS families the treatments, therapies and support they desperately need.

Each year, our supporters amaze me with their generosity and commitment to helping the ASF find a cure for Angelman syndrome. Now, with these additional clinics, we can accelerate research discoveries while continuing to improve the quality of life for even more people with AS *now*. To all our supporters: thank you, from the bottom of my heart. Your generosity is felt throughout the AS community, and for that I remain forever grateful.

Eileen Braun, Executive Director
Angelman Syndrome Foundation

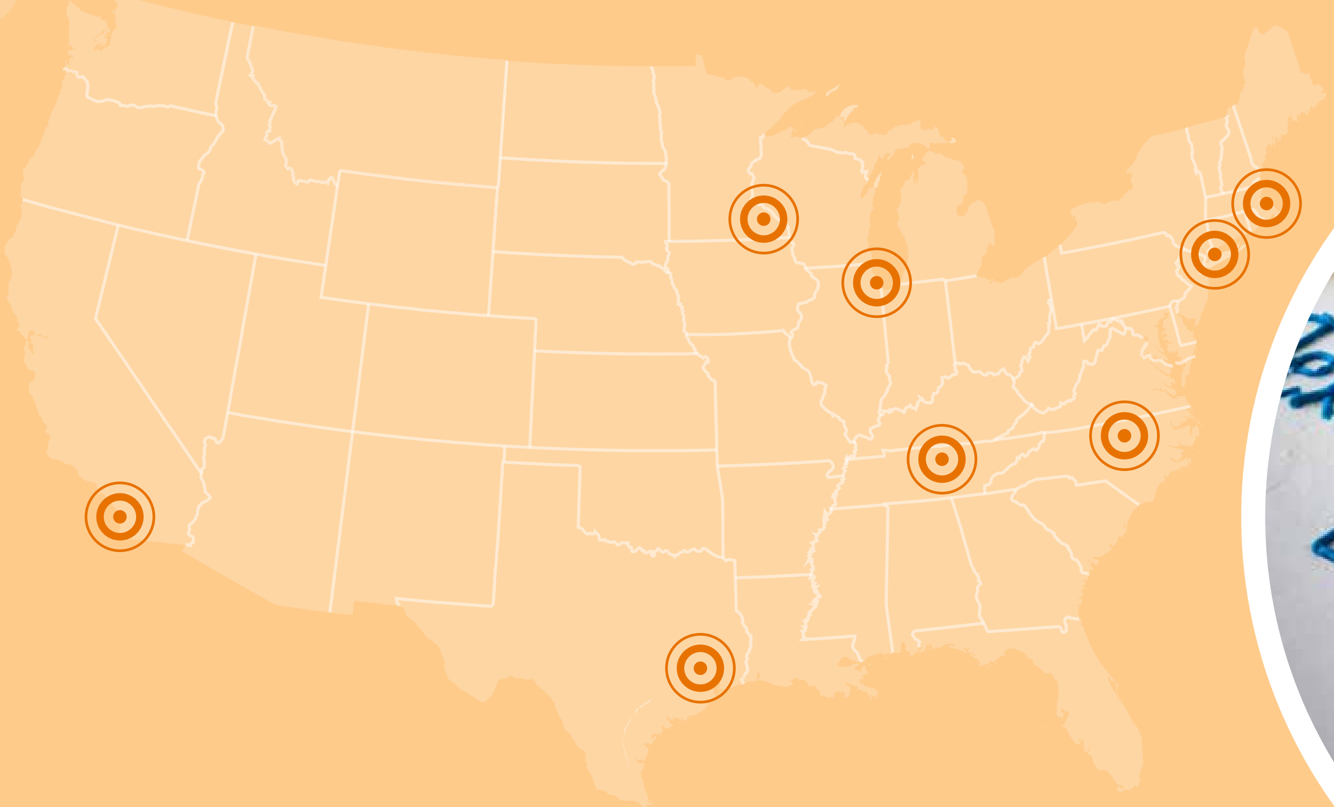
ASF AS CLINICS:

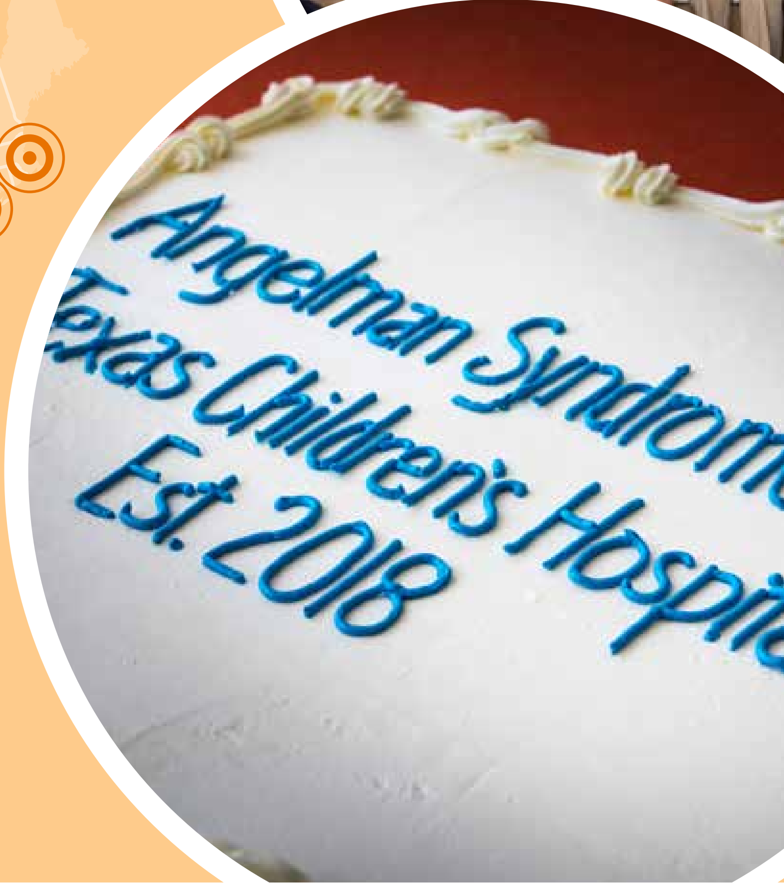
Angelman syndrome occurs in approximately 1 in 15,000 live births, which means there are many thousands of people in the world whose quality of life could be improved by having an ASF AS Clinic nearby. The dream of opening more clinics in the U.S. and around the globe is coming true because of continued support from our generous donors. With more ASF AS Clinics, more families can access specialized care while creating a network of clinics that can support and accelerate research and clinical trials to find a cure for AS.

> **“INDIVIDUALS WITH ANGELMAN SYNDROME HAVE EXTREME CHALLENGES OBTAINING THE CARE THEY NEED AS THEY GROW INTO ADULTS,”**
>
> **Dr. Elizabeth Berry-Kravis, co-director of the Angelman Clinic at Rush Hospital**

CHANGING LIVES, ONE CLINIC AT A TIME.

Thanks to the generous support of people in our community, four new ASF AS Clinics opened this year! Now, with eight locations across the United States, we have doubled the number of AS families we can help. The passion, commitment and expertise that each clinic brings to the AS community is truly making a difference in the lives of those with AS now and in the future.





NEW CLINICS. NEW HOPE.

VANDERBILT UNIVERSITY
MEDICAL CENTER

Vanderbilt University

"We want to improve the standard of living for our patients and their caregivers by using our knowledge and expertise to change lives,"

— Dr. Jessica Duis,
Director of the ASF Angelman Syndrome Clinic

RUSH UNIVERSITY
MEDICAL CENTER

Rush University Medical Center

"Our partnership with the ASF has allowed us to provide the comprehensive medical services that improve patient and families' quality of life,"

— Dr. Cesar Ochoa-Lubinoff,
Co-director of the ASF Angelman Syndrome Clinic

Texas Children's
Hospital

Texas Children's Hospital

"Thanks to the help of ASF, we are proud to provide very specialized care for individuals with Angelman syndrome,"

— Dr. Gary Clark,
Chief of Neurology at Texas Children's

NYU Langone
Health

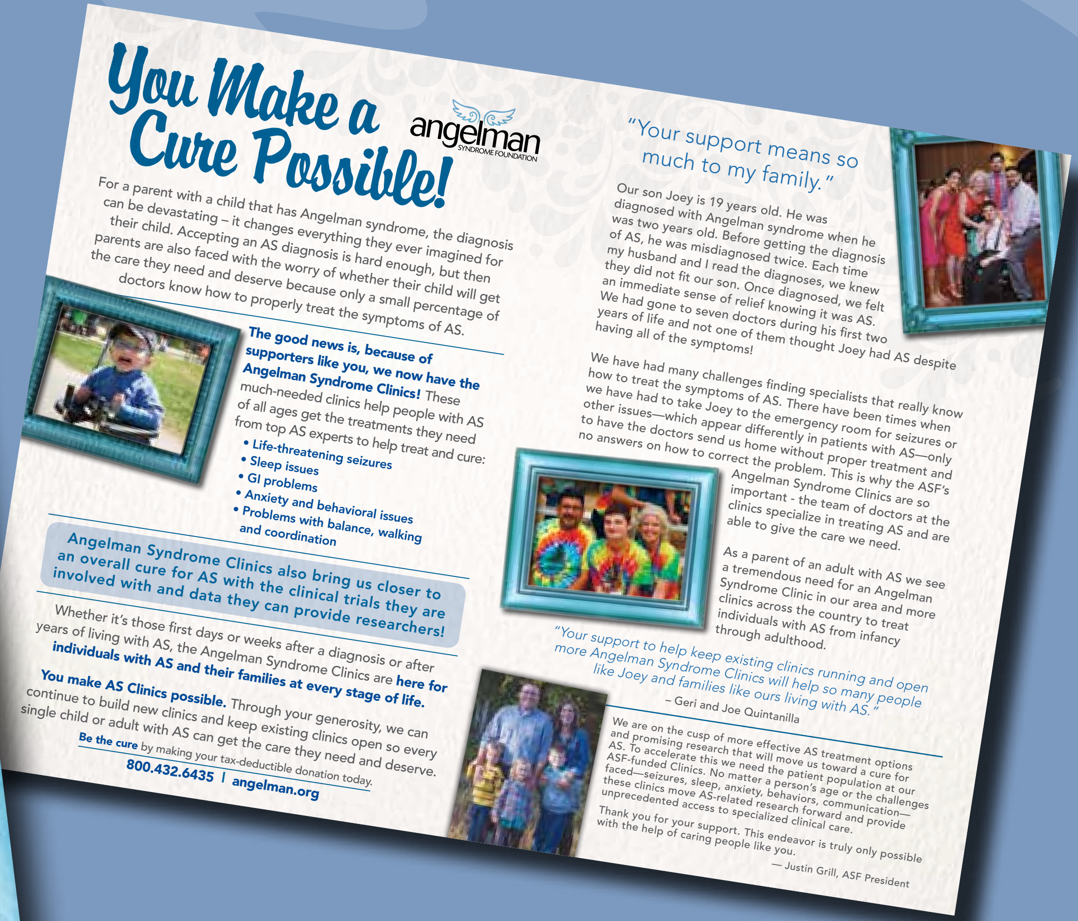
NYU Langone is in the approval process.

"The ASF AS Clinic is improving the lives of patients and accelerating AS research, which is helping us develop new and effective treatments for symptoms of AS,"

— Dr. Miles,
Head of the ASF Angelman Syndrome Clinic

BE THE CURE

Nearly 700 donors opened their hearts for our Be the Cure year-end campaign, helping us raise more than $223,000 – which played a huge role in the opening of the new clinics!



You Make a Cure Possible!

angelman SYNDROME FOUNDATION

For a parent with a child that has Angelman syndrome, the diagnosis can be devastating – it changes everything they ever imagined for their child. Accepting an AS diagnosis is hard enough, but then parents are also faced with the worry of whether their child will get the care they need and deserve because only a small percentage of doctors know how to properly treat the symptoms of AS.

The good news is, because of supporters like you, we now have the Angelman Syndrome Clinics! These much-needed clinics help people with AS of all ages get the treatments they need from top AS experts to help treat and cure:

- **Life-threatening seizures**
- **Sleep issues**
- **GI problems**
- **Anxiety and behavioral issues**
- **Problems with balance, walking and coordination**

Angelman Syndrome Clinics also bring us closer to an overall cure for AS with the clinical trials they are involved with and data they can provide researchers!

Whether it's those first days or weeks after a diagnosis or after years of living with AS, the Angelman Syndrome Clinics are **here for individuals with AS and their families at every stage of life.**

You make AS Clinics possible. Through your generosity, we can continue to build new clinics and keep existing clinics open so every single child or adult with AS can get the care they need and deserve.

Be the cure by making your tax-deductible donation today.

800.432.6435 | angelman.org

"Your support means so much to my family."

Our son Joey is 19 years old. He was diagnosed with Angelman syndrome when he was two years old. Before getting the diagnosis of AS, he was misdiagnosed twice. Each time my husband and I read the diagnoses, we knew they did not fit our son. Once diagnosed, we felt an immediate sense of relief knowing it was AS. We had gone to seven doctors during his first two years of life and not one of them thought Joey had AS despite having all of the symptoms!

We have had many challenges finding specialists that really know how to treat the symptoms of AS. There have been times when we have had to take Joey to the emergency room for seizures or other issues—which appear differently in patients with AS—only to have the doctors send us home without proper treatment and no answers on how to correct the problem. This is why the ASF's Angelman Syndrome Clinics are so important - the team of doctors at the clinics specialize in treating AS and are able to give the care we need.

As a parent of an adult with AS we see a tremendous need for an Angelman Syndrome Clinic in our area and more clinics across the country to treat individuals with AS from infancy through adulthood.

"Your support to help keep existing clinics running and open more Angelman Syndrome Clinics will help so many people like Joey and families like ours living with AS."

– Geri and Joe Quintanilla

We are on the cusp of more effective AS treatment options and promising research that will move us toward a cure for AS. To accelerate this we need the patient population at our ASF-funded Clinics. No matter a person's age or the challenges faced—seizures, sleep, anxiety, behaviors, communication—these clinics move AS-related research forward and provide unprecedented access to specialized clinical care.

Thank you for your support. This endeavor is truly only possible with the help of caring people like you.

— Justin Grill, ASF President

RESEARCHING OUR WAY TO A CURE.

ASF-Funded Research is leading the charge toward exciting advancements and discoveries, like validating a new tool to diagnose AS in the first few weeks of life, which will improve quality of life faster by allowing treatments to be administered right away. The ASF AS Clinics are accelerating research as well by collecting important data from a large AS population, conducting research studies, and providing a clinical setting for future clinical trials. ASF-funded research is progressing into biomarkers and outcome measures, gene editing, gene therapy, small molecule treatments, and more! But none of this would be possible without our community of generous donors working to change the future of those with AS.

NEW ASF-FUNDED RESEARCH:

World’s Largest Newborn Screening for AS

— Dr. David Godler, Murdoch Children’s Research Institute in Melbourne Australia

ASF-funded research is assisting Dr. Godler in validating a newborn screening tool for AS that will diagnose newborns in weeks, rather than years. This will be critical, because the earlier we can apply life-changing treatments, the better the results will be. Watch the video to learn more.

Crispr/Cas9 Gene Editing Tool

— Dr. Mark Zylka, University of North Carolina – Chapel Hill

New ASF-Funded Research by Dr. Zylka is utilizing Crispr/CAS9 technology to help find a cure for AS! This one-time treatment could lead to a lifetime effect – a CURE! Watch the video to learn more.

PUBLISHED ASF-FUNDED RESEARCH STUDIES:

ASF-funded research is improving lives and earning top recognition among the world's leading AS researchers. What's more, researchers have used ASF funding to secure additional funding based on success. For example, a white matter study that began with a $200,000 investment from ASF led to a $3.14 million National Institutes of Health (NIH) grant.

A number of pivotal research papers were published this year that ASF had a role in supporting. A few highlights include:

1. **Ube3a reinstatement mitigates epileptogenesis in Angelman syndrome model mice**
   — Ben Philpot, Ph.D., University of North Carolina – Chapel Hill
2. **Gene Therapy in Angelman Syndrome**
   — Steven Gray, Ph.D., University of Texas Southwestern Medical Center
3. **Speech generating devices in children with Angelman syndrome: An effectiveness trial**
   Dr. Anjali Sadhwani – Boston Children's Hospital
4. **Angelman syndrome in adolescence and adulthood: A retrospective chart review of 53 cases**
   Ronald L. Thibert, DO, MsPH, MassGeneral Hospital for Children
5. **A behavioral test battery for mouse models of Angelman syndrome: a powerful tool for testing drugs and novel Ube3a mutants**
   — Ype Elgersma, Ph.D., Erasmus University Medical Center

ON AVERAGE, EVERY $100 DONATED FOR RESEARCH TO ASF GROWS TO $1,500 FOR AS RESEARCH.

CURE CLUB

This special group opens their hearts monthly with a donation that truly makes a difference. Each gift adds up to big impact for AS families, and we are so grateful for their generous support. Learn more about the Cure Club here.

> "BEING A PART OF THE ASF CURE CLUB ALLOWS ME TO SUPPORT A FOUNDATION THAT HAS DONE SO MUCH FOR OUR DAUGHTER AND HER ANGEL.
>
> – VICKI SPICKELMIRE
> GRANDMA OF ANGEL JACKSON MOORE

BOOTS ON THE GROUND. HOPE FOR THE FUTURE.

The love and support we feel every year from our community champions is unmatched. Their dedication to helping the ASF find a cure for Angelman syndrome warms our hearts. In 2018, individuals across the country helped raise more than $243,000 through grassroots fundraisers like the 2018 Chicago Marathon, Quinn's Hot Cocoa for a Cure, Chip Away at AS, and more!

BOOTS ON THE GROUND. HOPE FOR THE FUTURE.

2018 ANGELMAN SYNDROME FOUNDATION WALK – 1,265,950

Walk participants from all over the country came together to raise money for AS research, education and family support services. Each year we are blown away by the turnout from our amazing community! It's truly a day to remember.

BOOTS ON THE GROUND. HOPE FOR THE FUTURE.

QUINN'S HOT COCOA FOR A CURE – $80,846

Last year, Quinn started his hot cocoa for a cure to raise money for his twin sister Emma, who has AS. This year, Quinn doubled his proceeds by selling real and virtual cups of cocoa for $4 a cup. Quinn's goal is to sell 5 million cups – that's $20 million toward a cure!

BOOTS ON THE GROUND. HOPE FOR THE FUTURE.

2018 CHICAGO MARATHON – $58,726

The Windy City Angels didn't walk, but ran 26.2 miles to raise thousands of dollars so that our loved ones can get the expert care they desperately need at ASF AS Clinics. We are so thankful for their dedication and commitment to changing the lives of those with AS.

BOOTS ON THE GROUND. HOPE FOR THE FUTURE.

FACEBOOK FUNDRAISERS – $98,315

Fundraisers created on Facebook helped mobilize our networks of family and friends. Through birthday fundraisers, #GivingTuesday and more, these generous people proved that every dollar raised counts!

BOOTS ON THE GROUND. HOPE FOR THE FUTURE.

SKATING ANGELS – $13,635

This charity hockey event, held in Dedham, Massachusetts, is dedicated to spreading awareness for Angelman syndrome and raising support for innovative research and critical care.

BOOTS ON THE GROUND. HOPE FOR THE FUTURE.

CHIP AWAY AT AS – $7,881

In celebration of Stetson's birthday, Chip Away at AS hosts an annual golf tournament, lunch and birthday celebration. All funds raised go to ASF research.

BOOTS ON THE GROUND. HOPE FOR THE FUTURE.

TRIVIA NIGHT - $4,777

They put the FUN in fundraising with Trivia Nights, drawing crowds out to test their pop culture knowledge while giving back to the AS community.

AS FAMILY WEEKEND AT CENTER FOR COURAGEOUS KIDS

A TIME TO BE YOURSELVES, WHILE BEING TOGETHER.

For the third year in a row, our generous supporters have allowed families to enjoy a weekend at CCK for free. For three days, 30 AS families were able to take a break from the doctors appointments and worry. They enjoyed stress-free time together sharing traditional camp activities in an environment that is accepting of everyone.

> "YOU GAVE US THE BEST FAMILY WEEKEND WE EVER HAD. THANK YOU FOR MAKING THIS POSSIBLE AND GIVING OUR FAMILY AN UNFORGETTABLE WEEKEND." – BRAD

FAMILY RESOURCE TEAM

The ASF Family Resource Team is a group of compassionate AS experts helping families handle a variety of needs, from finding general resources for your child, to solving Individual Education Plan (IEP) issues, to finding medical equipment and other helpful products.

This group serves as a lifeline for AS families across the country. In 2018, they spent more than 100 hours providing one-on-one support to families, and they've spent a total of 1,612 hours with families since 2014.

Meet the Team

Lizzie Sordia

Dr. Eric Wright

Michelle Harvey

SCIENTIFIC ADVISORY COMMITTEE (SAC)

A group of 17 Angelman syndrome researchers and professionals responsible for overseeing research grants funded by the ASF. Members donate their time and talent to review all research applications submitted to the ASF for funding.


Stormy Chamberlain, PhD
SAC Chair

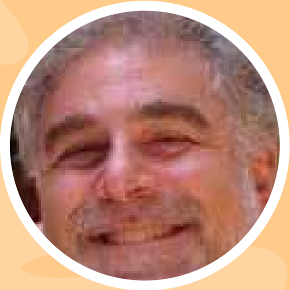

Arthur Beaudet, MD


Charles Williams, MD


Ben Philpot, PhD


Dan Harvey, PhD


Michael Ehlers, PhD


Steven Katz, MD


Jane Summers, PhD


Marc Lalande, PhD


Mark Nespeca, MD


Wen-Hann Tan, MD


Fred Pritzker, JD


Ron Thibert, DO, MsPH


Coral Thompson, BSc., RT


Katharine Grugan, PhD


Lora Meerdo, PhD


Erin Sheldon, M.Ed

BOARD MEMBERS

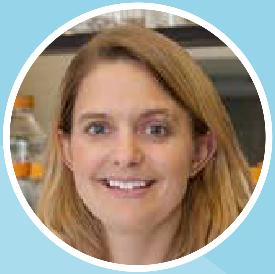
We are honored to have this amazing leadership team steering the future of the AS community. Their wisdom and experience in organizational growth is leading to amazing discoveries on behalf of AS families everywhere.

Justin Grill
President

Tim Bousum
Vice President

Kathy Rokita
Treasurer

Michael Cecere
Secretary



Stormy Chamberlain
SAC Chair

Dan Harvey
Director

Shannon Pruitt
Director

Jim Kubicza
Director

Kyle Rooney
Director

Eric Hamberg
Director

Amanda Moore
Director

Elizabeth Jalazo
Director

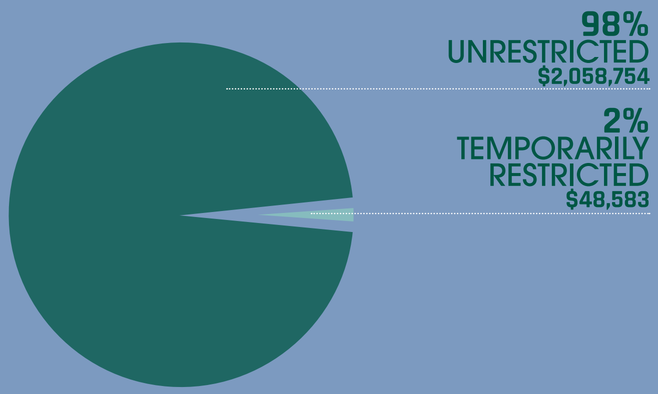
Eric Wright
Director

Anna Blanding
Director

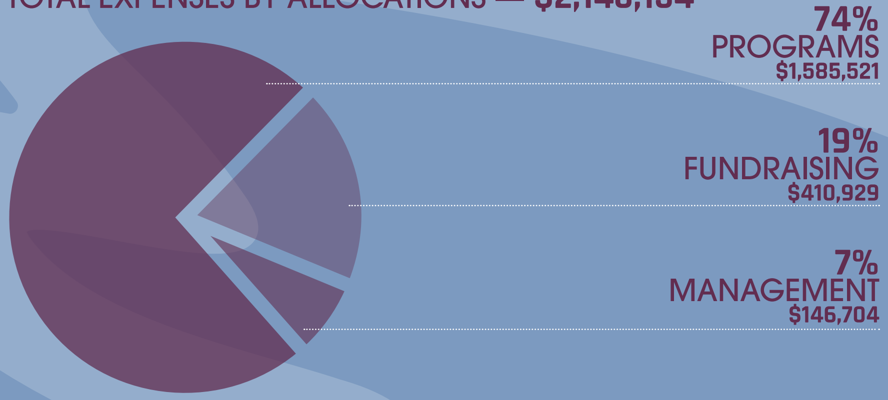
FINANCIAL SUPPORT

Everything we do is supported by our community of generous donors. In 2018, that community made a daily difference in the lives of those with AS and their families, and they are bringing us closer to a cure!

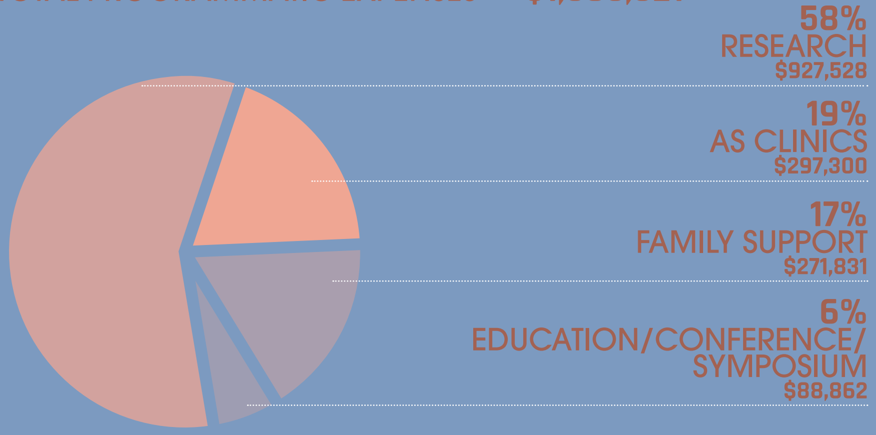
TOTAL REVENUES — **$2,107,337**



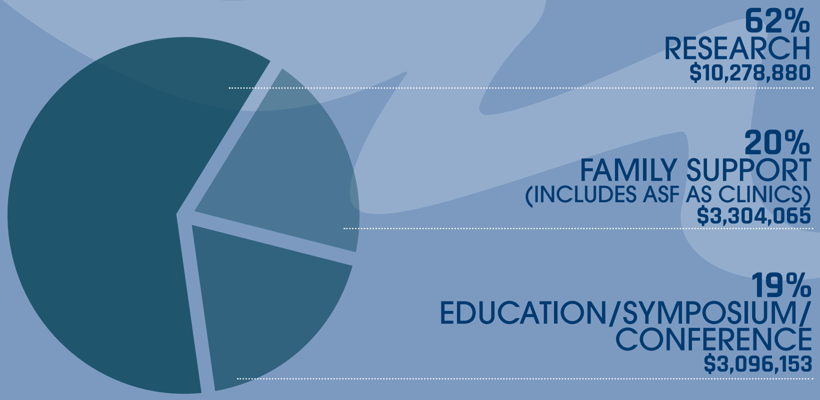
TOTAL EXPENSES BY ALLOCATIONS — **$2,143,154**



TOTAL PROGRAMMATIC EXPENSES — **$1,585,521**



TOTAL OF ALL CONTINUED PROGRAMMATIC EXPENSES — **$16,679,098**
(SINCE INCORPORATION - 26 YEARS)



THANK YOU FOR CHANGING SO MANY LIVES.

Thanks to our incredible supporters, my family and the thousands of other families living every day with AS are able to see hope for the future. In 2018, they once again blew us away with their generosity. Together, we were able to continue reaching more families with the expert care they need while we aggressively and actively search for a cure. The future is bright for the AS community and we have all of our contributors to thank for it.

With humble gratitude,
Justin Grill, President
Angelman Syndrome Foundation

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give them a reason to smile.