2017 ANNUAL REPORT

AUTISM SCIENCE FOUNDATION

PRESIDENT'S LETTER

Dear Friends:

The past year was an exciting one for ASF and the field of autism science, which continues to deliver incredible new insights and actionable information. Now more than ever, we have to keep pushing forward, leveraging new knowledge and putting it to work for people with ASD and their families.

I was honored to again be part of the process of developing the new Interagency Autism Coordinating Committee (IACC) Strategic Plan, a blueprint to guide federally funded autism research and service provision. Importantly, it recommends focusing research on sex differences in ASD, specifically under-diagnosis, the female protective effect and unique challenges faced by girls/women with ASD. ASF's Autism Sisters Project is at the forefront of these efforts.

ASF continues to catalyze important progress. We maximize our impact by investing in the most promising science and the researchers who are taking innovative approaches to big challenges. While the science is at the center of our mission, it is always at the service of those who rely on science to help make their lives better in tangible ways.

Here are some important highlights from the past year:

ASF doubled its annual grant funding, awarding almost $1 million in new awards, and provided funding for more research trainees than any other private autism research organization.

Three new multiyear research grants will expand the Autism Sisters Project to the University of California at San Francisco and the Broad Institute in Cambridge, MA. This ASF initiative explores the female protective effect by studying autism families with an undiagnosed sister. The Autism Science Foundation, together with three international partner organizations, launched the first multinational survey on experiences in employment, designed to identify needs, gaps in services and opportunities for employers. The results will be used to develop a policy brief around employment in autism.

In March, the fourth annual ASF Day of Learning featured top autism scientists, clinicians and service providers giving TED-style talks to the stakeholder community on topics ranging from the development of diagnostic technologies and new biomarkers to how best to help people with ASD develop practical daily living skills.

Thomas Insel, the president and co-founder of Mindstrong Health and former director of the National Institute of Mental Health, has joined the ASF Board of Directors. We are delighted that Dr. Insel, who served on our Scientific Advisory Board, will play an expanded role in charting the course for our organization.

The ASF Weekly Science Podcast, hosted by Chief Science Officer Alycia Halladay, was named by Healthline as one of the Best Autism Podcasts of 2017. Each week, Dr. Halladay breaks down important news and emerging trends in the field of autism research.

Thank you for your continued support of ASF and its mission. We look forward to another year of progress, both in the lab and in the lives of those we strive to help every day.

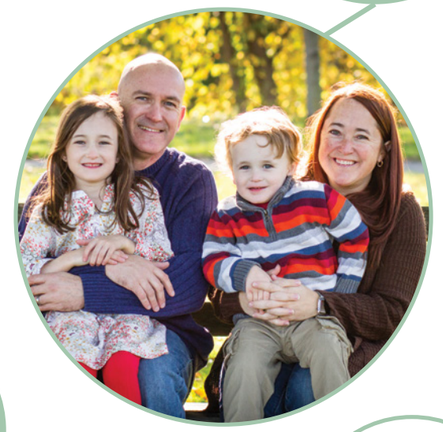
Sincerely,

Alison Singer
President, Autism Science Foundation

ACCOMPLISHMENTS

In 2017, our eighth year of operation, the Autism Science Foundation:

- More than doubled our annual grant funding, awarding almost $1 million.
- Provided funding for more research trainees than any other private autism research organization.
- Launched phase 2 of the Autism Sisters Project, providing grants to query the new database of unaffected sisters.
- Produced the autism community's fourth annual TED talks, featuring distinguished autism scientists from across the country.
- Provided substantial support to the largest group of researchers studying and sharing data on early signs of autism, the Baby Siblings Research Consortium.



- Expanded enrollment in Autism BrainNet, registering over 1,000 new individuals to donate portmortem brain tissue.
- Offered travel grants for stakeholders to attend major scientific conferences.
- Hosted 50 weekly science podcasts with more than 134,000 downloads, offering deeper explanation and context about autism research news for families.

- Posted over 400 up-to-the-minute news items on Facebook and Twitter, providing critical information for families about significant new scientific discoveries.
- Formed AGENDA, a coalition of genetically defined autism advocacy groups to foster collaboration.
- Organized an international cohort to conduct research on best practices in employment in autism, with a policy brief to be released in 2018.

FUNDING SCIENCE

In 2017, ASF awarded almost $1 million in grant support primarily through its pre- and postdoctoral fellowships, research accelerator awards, and undergraduate summer research grants. Below are summaries of the projects funded by these awards.

POSTDOCTORAL FELLOWSHIPS

Dr. Michael Breen and Dr. Joseph Buxbaum
Icahn School of Medicine at Mount Sinai

Developing peripheral blood and neuronal biomarkers for autism using a genetically defined subtype

This project will begin to determine the potential validity of a blood-based biomarker for autism by comparing gene expression in blood and induced pluripotent stem cells from the same patients, particularly individuals with a severe form of autism characterized by a mutation of the SHANK3 gene. This work will begin to identify new markers in blood samples that will improve diagnosis and also identify new drug targets that will enhance the development of new treatments.

Dr. Michelle Failla and Dr. Carissa J. Cascio
Vanderbilt University

Understanding the pain response in people with autism

This study will examine verbal and nonverbal responses to mild pain stimuli in adults with ASD. The findings will help clinicians understand pain sensitivity in those with autism so that new strategies to assess and manage pain can be developed.

Dr. Isabella Rodrigues Fernandes and Dr. Alysson Renato Muotri
University of California, San Diego

Screening for new autism treatments using cells in a dish

Using a cell culture system that more accurately models the complexity of the human brain compared to just single brain cells, this study will screen several potential therapeutic drugs in a system where the cells have an autism-related mutation and examine not just the effect on neurons, but on other brain cells as well. This will impact the way molecules for treatment are identified, screened and then moved into clinical trials.

Dr. Dorothea Floris and Dr. Adriana Di Martino
New York University Medical Center

Characterizing the female and male brain in autism

Using two neuroimaging databases, this project will examine functional magnetic resonance imaging data to better understand which areas of the brain are over-connected or under-connected in autism. By combining databases of imaging data, the project will be able to include enough females to understand how brain connectivity in males and females with autism is similar and different. Ultimately, this will inform future development of gender- and sex-specific diagnostic criteria and interventions.

Dr. Eitan Kaplan and Dr. Robert F. Hevner
Seattle Children's Hospital

Determining the genetic and environmental factors influencing brain development

In this study, researchers will explore how the development of new brain cells is affected by the combination of a genetic mutation in a known autism risk gene, TBR1, and the environmental factor of an immune response during pregnancy. This novel approach will better describe the role of each risk factor—genetic and environmental—separately and together on the formation of the cerebral cortex, a brain region known to be involved in ASDs.

Dr. Carol Wilkinson and Dr. Charles A. Nelson
Boston Children's Hospital

Developing biological markers for more severely affected individuals

Nearly half of all children with fragile X syndrome (FXS) meet criteria for ASD, and virtually all have cognitive and language difficulties ranging from mild to severe. This project will use noninvasive brain activity measures to understand what happens in the brains of young children with FXS during response to sensory stimuli, and how this correlates with symptoms often seen in autism. These brain-based markers will then be used in future clinical trials as objective measures for targeted outcomes.

This fellowship is supported by both the Autism Science Foundation and the FRAXA Research Foundation.

PREDOCTORAL FELLOWSHIPS

Songjun William Li and Dr. Ziv Williams
Boston University School of Medicine

Exploring the possibility of deep brain stimulation in autism

This project seeks to understand the specific areas of the brain that would be targets of deep brain stimulation therapy. Researchers will examine cell function during social interaction tasks and provide stimulation to these same neurons to understand how particular circuits affect behavior. This will lead to a better understanding of the therapeutic potential of deep brain stimulation.

Elizabeth Sharer and Dr. Jed Elison
University of Minnesota

Defining the female protective effect in infants with ASD

This will be the first study to investigate the female protective effect in infants who show behaviors of concern, as compared to those who develop typically and those who are later diagnosed with ASD. The goal is to provide more personalized diagnostic and treatment approaches for females with autism.

Christina Vallianatos and Dr. Shigeki Iwase
University of Michigan

Illustrating the importance of epigenetics in the sex bias of ASD

The KDM5C gene is associated with autism and intellectual disability in males, but this study will examine its effects in females, focusing on behavioral features, brain development and "downstream" gene expression. This research will shed light on the mechanisms of possible gene/environment interactions in females.

ACCELERATOR AWARDS

The accelerator grant mechanism is designed to rapidly fund novel findings, provide resources to build on an existing project or to explore unexpected opportunities.

Dr. Antoinette Sabatino DiCriscio, Dr. Christa Martin and Dr. Vanessa Troiani

Geisinger Clinic

Pupil response in individuals with ASD and known copy number variations

Individuals with autism differ on how the pupil in their eye expands and contracts in response to light. This funding will allow researchers to conduct pupillometry assessments on people with autism who are already undergoing other assessments, including behavioral and genetic analysis, in order to better understand autism subgroups.

Dr. John Strang

Children's National Medical Center

Longitudinal follow-up to adolescent social skills and executive functioning intervention studies

This award will support an additional four months of post-treatment follow-up to an ongoing study examining two different public-school administered interventions for adolescents with autism—one targeting social skills and the other executive function skills. The longer-term follow-up will enable the team to collect data on both the immediate and longer-term impacts of these interventions and allow researchers to obtain qualitative feedback from parents and individuals with ASD about their experiences with the interventions.

UNDERGRADUATE GRANTS

ASF is the only autism organization to offer summer grants to undergraduate researchers, encouraging the best and brightest students to launch a career in autism science.

Danielle Dennis and Dr. Rebecca M. Jones

Cornell University

Danielle focused on understanding how the brains of individuals with autism function in tasks involving impulsivity. This helped improve intervention targets, specifically for adolescents who struggle with impulse control.

Jacqueline Emerson and Dr. Geraldine Dawson

Duke University

Jacqueline examined a new EEG-based method to measure how people with autism react to sensory stimuli. This helped improve our understanding of what contributes to different restricted and repetitive behaviors so they can be better treated.

Christopher Esposito and Dr. Matthew D. Lerner

Stony Brook University

Christopher surveyed individuals with autism, family members, employers and service providers on experiences, needs and ways to improve employment opportunities.

Edward Gao and Dr. Stephen Sanders

University of California, San Francisco

This research used a novel analytic tool developed by Google/Verily Life Sciences to study the genetic makeup of thousands of families, with the goal of identifying new genes associated with autism. Adaptations to this tool help better identify a specific type of genetic change that has recently been discovered in ASD.

Ellyn Pueschel and Dr. Inna Fishman

San Diego State University

This project combined images from existing brain atlases to create a template of the typically developing infant brain so that it can be compared to the brains of infants at risk for autism.

Laura Thomas and Dr. Jed Elison

University of Minnesota

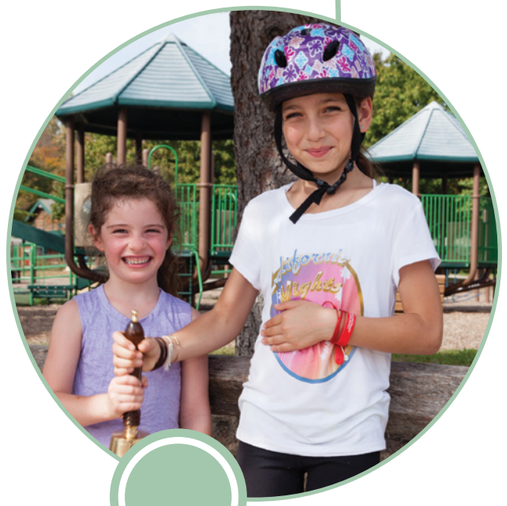
This project studied the function of the amygdala—an area of the brain known to be affected in adults with autism—during an attention task in toddlers to help better determine the biological basis of attention problems in young children, which may be responsive to early treatment.

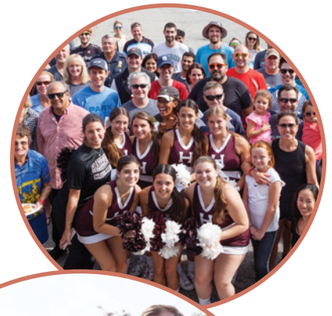
> Thanks to my ASF grant, I am able to devote more of my time and energy to what I am passionate about—improving our understanding of the neurobiology of autism so that, as a clinician, I can one day offer more effective therapies for the children and families I care for.
>
> **— DR. CAROL WILKINSON, BOSTON CHILDREN'S HOSPITAL**

Autism Sisters PROJECT

An Initiative of the AUTISM SCIENCE FOUNDATION and THE HILIBRAND FOUNDATION

In 2017, ASF launched phase 2 of its Autism Sisters Project by providing grants to query the database that was developed in phase 1. The goal of this project is to understand why females seem to be protected from autism and to harness this protective effect to protect both males and females from the most debilitating aspects of autism. In this new phase, researchers from the Broad Institute and the University of California, San Francisco will be analyzing both genetic and behavioral data from thousands of individuals worldwide. In addition, the project continues to seek new families with a child with autism and a non-diagnosed sister to contribute DNA (saliva sample) to our growing database.





WALL STREET RIDES FAR FOR AUTISM RESEARCH

Our third annual Wall Street Rides FAR (For Autism Research) drew a huge crowd and raised more than $270,000 to fund ASF scientific initiatives. Riders enjoyed bicycling through beautiful foliage in Westchester County in the fall. Join us for our fourth annual Ride FAR on October 13, 2018.

ASF President Alison Singer, Ride FAR Chairs Melissa Moo Harkins and Bryan Harkins at Wall Street Rides FAR

All smiles at the Autism Speaks Walk in Phoenix

autism BRAIN NET

In 2017, 1,222 new people registered with Autism BrainNet to donate postmortem brain tissue for autism research. Autism BrainNet's repository has contributed to critical findings in autism neurobiology, helping us understand how the brain develops connections and how those connections change with age.

Melissa Miller and Carolyn Hare registered families for Autism BrainNet at IMFAR 2017 in San Francisco

BSRC AUTISM BABY SIBLINGS RESEARCH CONSORTIUM

The Baby Siblings Research Consortium (BSRC) is a multidisciplinary, collaborative community of researchers and clinicians committed to understanding the developmental origins and earliest signs of autism spectrum disorder (ASD) by studying infants at familial risk for ASD. Over the last decade, through rigorous studies conducted by investigators across multiple sites, the BSRC has identified both behavioral and biological markers of risk and disrupted development prior to the age of diagnosis, sparking studies of early intervention in infancy. The BSRC shares this knowledge with investigators, parents and clinicians who provide care to communities and families, and shares research strategies with all those investigating high-risk populations.

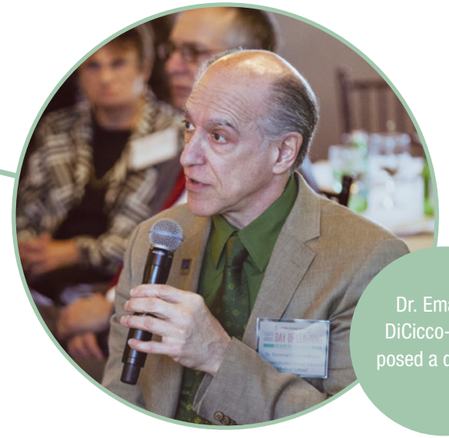
AUTISM SCIENCE FOUNDATION

FOURTH ANNUAL DAY OF LEARNING

Our fourth annual TED-style autism conference featured talks by top autism researchers from across the country for a full day of learning. More than 325 parents, individuals with autism, general and special education teachers, students and other stakeholders participated.

Dr. Amy Lutz of the EASI Foundation discussed the housing crisis for adults with autism

Dr. Wendy Chung of the Simons Foundation gave an overview of where we are in understanding the heterogeneity of the autism spectrum



Dr. Emanuel DiCicco-Bloom posed a question

Autism self-advocate Paul Morris introduced Amy Lutz of the EASI Foundation

Louise Southern and Dr. Aleck Myers of the Autism Society of North Carolina with ASF Board Chair Gregg Ireland, and Lori Ireland

Dr. David Mandell of the University of Pennsylvania described new methods to improve communication between teachers and parents of children with autism

Dr. Robert Schultz from the Children's Hospital of Philadelphia shared insights on new technologies being used to improve autism diagnosis

Dr. James McPartland of Yale with members of his lab

Marc, Liane and Jonathan Carter; Liane received the Caryn Schwartzman Spirit Award in recognition of her outstanding efforts as an autism advocate

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