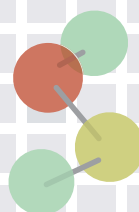
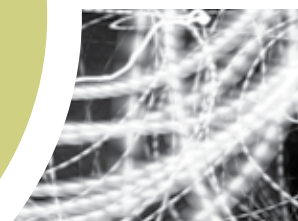
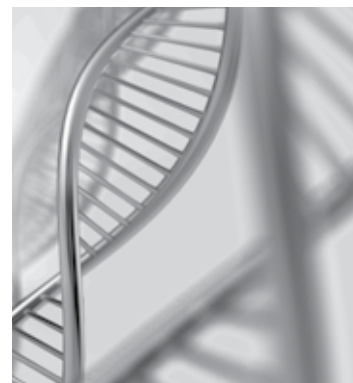
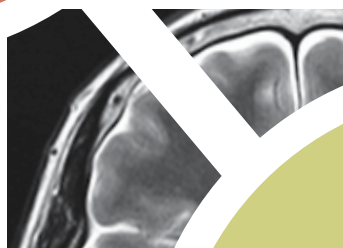


# 2013 ANNUAL REPORT



**AUTISM** SCIENCE FOUNDATION

# PRESIDENT'S LETTER

*Dear Friends:*

As I sit down to write my fourth annual president's letter, I can't help but focus on the fact that next year ASF will celebrate its fifth anniversary as an organization. We have accomplished so much in the past four years, but there is so much work still ahead of us. And while the goal of this annual report is to look back at our yearly accomplishments, like all our families, we can't help but look forward.

Looking forward has always been part of the standard operating procedure of families raising children with autism. Our kids gain new skills; we celebrate briefly and are already dreaming of their next accomplishment. We barely agree to one IEP and are thinking about the next one. At ASF, we are always looking forward for the next big scientific breakthrough that will help our family members, and looking ahead to figure out how to raise the money to fund the research to make it a reality.

This year, when we learned from investigators that one of the biggest rate limiting factors in autism research was lack of funds to exploit unexpected research findings, we quickly implemented a new rapid-review funding mechanism to provide "research enhancement grants" to enable scientists to quickly explore these promising, novel findings. We also launched our campaign to encourage more families to register to donate brain tissue for autism research, another rate limiting factor in autism research. With increased awareness of the need for tissue we are certain to see our families mobilize and register to make this important gift. And we celebrated the major milestone of surpassing the one million dollar mark in grants funded.

We could not do the work we do without your continued support and we are looking forward to continuing to work together to make real scientific progress that improves real people's lives. We look forward to the day when our family members with autism learn a new skill. We look forward to their smiles of success. And we look forward to seeing the results of our funded studies make a real difference in real people's lives.

Sincerely,



Alison Singer  
President, Autism Science Foundation



## ACCOMPLISHMENTS

In 2013, our fourth year of operation, the Autism Science Foundation:





# FUNDING SCIENCE

Funding autism research is at the core of ASF's mission, with over \$1.1 million funded in ASF's first four years. In 2013 alone, ASF awarded over \$350,000 to clever young investigators, with the brightest, most innovative ideas, to help them jumpstart their careers in autism research. These young scientists are mentored and trained by established autism researchers. In 2013, ASF introduced three new funding mechanisms: a 3-year early career award, a treatment grant, and research enhancement mini-grants. By funding some of the most promising young investigators, Autism Science Foundation is investing in the future of autism research.



### 3-YEAR EARLY CAREER AWARD

**Dr. Jill Locke**  
University of Pennsylvania

Multi-Site, Randomized, Controlled Implementation Trial of an Evidence-Based, Adult and Peer-Mediated Social Skills Intervention for Elementary School Children with Autism Spectrum Disorder

Dr. Locke works directly with children in schools in Philadelphia and New York to develop and test methods to improve their social skills. Her study involves training school personnel in a social skills intervention to improve children's engagement with peers during unstructured periods, such as recess and lunch, and then measuring the outcome of those interventions. She also examines the best ways to support public school personnel in their continued use of this intervention.

*"I am really fortunate to have the opportunity to work with schools to support students with autism with their social skills and peer relationships. My goal is to enable children with autism to utilize these interventions on a daily basis in their natural environment."*



### TREATMENT GRANT

**Dr. Alexander Kolevzon**  
Icahn School of Medicine at Mount Sinai

Human Clinical Trial of IGF-1 in Children with Idiopathic ASD

This grant enables Dr. Kolevzon to expand his existing FDA-approved human clinical trial of Insulin-Like Growth Factor-1 (IGF-1), which currently includes children with autism who have a known SHANK3 mutation to now include children with autism without this mutation. IGF-1 is known to promote synaptic maturation and plasticity and the SHANK3 / glutamate signaling pathway may be common in diverse forms of ASD. Results from this trial are expected to provide evidence that IGF-1 is safe and efficacious in targeting core symptoms of social withdrawal in children with ASD, with and without Shank3 deletion.

*"Preliminary results from our placebo-controlled pilot of IGF-1 in Phelan McDermid Syndrome are very promising and provide clear support for our methods and outcome measures. We have seen robust effects in some patients and look forward to increasing our sample size and including this cohort of patients with ASD but without Phelan McDermid Syndrome."*



### PREDOCTORAL FELLOWSHIPS

**Alexandra Bey & Dr. Yong-hui Jiang**  
Duke University

The Role of Shank3 in Neocortex Versus Striatum and the Pathophysiology of Autism

This project examines how different brain regions contribute to ASD-related behavior in a mouse model of autism. We are disrupting the gene Shank3, a well-established genetic cause of autism, in different brain regions and measuring sociability, communication, and repetitive behaviors in mice.

*"From my work so far, it is really exciting to see how disrupting Shank3 in just one brain region can recapitulate some of the behaviors seen in mice lacking Shank3 everywhere in the brain, supporting our idea that some regions control specific ASD-related behaviors. I am really looking forward to attending IMFAR this year and sharing my work, as well as hearing from other scientists and people whose lives are directly impacted by autism."*



### PREDOCTORAL FELLOWSHIPS

**Ezzat Hashemi & Dr. Veronica Martinez-Cerdeno**  
University of California, Davis

Alteration of Dendrite and Spine Number and Morphology in Human Prefrontal Cortex of Autism

This project will analyze pyramidal neurons in the prefrontal cortex of post-mortem human subjects with autism. Dendrites transfer electrical signals within the brain via their spines. To determine the role of dendrites and spines in disrupted signaling, we will quantify and analyze their morphology using Golgi staining and Neurolucida. This study will bring us a step closer to developing new treatments for autism and other neurodevelopmental disorders.

*"I am interested in studying neurodevelopmental diseases, mostly autism. During the past year I have been studying the role of cortical dendritic arbors and spines in the pyramidal neurons of autism patients. I am also studying interneuronal subtypes and their differing influence on autism."*



### PREDOCTORAL FELLOWSHIPS

**Jessie Northrup & Dr. Jana Iverson**  
University of Pittsburgh

Development of Vocal Coordination between Caregivers and Infants at Heightened Biological Risk for Autism Spectrum Disorder

From very early in life, even before babies begin saying words, infants and their mothers adjust the timing of their vocalizations when interacting with one another, much like two adults having a conversation. This type of turn-taking is an important developmental skill, and disruptions in that skill could be early indicators of later social-communication problems associated with ASD. My research examines the timing of vocalizations in interactions between infant siblings of children with ASD and their mothers throughout the first year of life in order to look for early warning signs of ASD and language delay.

*"I feel so grateful to ASF for giving me the opportunity to dedicate my time to trying to answer research questions that I am excited about and that I think are important to the autism community."*



### PREDOCTORAL FELLOWSHIPS

**Russell Port & Dr. Timothy Roberts**  
University of Pennsylvania

GABA and Gamma-band Activity: Biomarker for ASD?

The brain uses both chemicals and electrical signals to communicate, and there is evidence that both of these are affected in ASD, but this has never been tested simultaneously in the same subjects. The purpose of this study is to verify in the same subjects that both chemical and electrical signals are dysfunctional, and to determine whether their dysfunction is related to one another. Lastly using animal models that represent aspects of ASD, this study will try to discover the mechanistic basis for this interrelation.

*"ASD is truly devastating, but the recent advances in the understanding of these disorders improve our ability to help people with ASD. My project has the promise of discovering a fundamental biological substrate affecting people with ASD, and will hopefully lead to better treatment options."*



**POSTDOCTORAL FELLOWSHIPS**

**Dr. Aimee Badeaux & Dr. Yang Shi**  
Boston Children's Hospital

Molecular Characterization of Autism Gene CHD8 in Shaping the Brain Epigenome

During our months in the womb and in the first years of life, the neurons in our brain are rapidly dividing, growing and being pruned to form mature circuits. For this process to occur normally, the expression of our genes must be tightly timed and controlled. The focus of this research is to understand how a few molecules implicated in autism control the timing and location of gene expression in the developing brain, and how this process goes awry when these molecules are lost.

*"I work on chromatin regulation and gene transcription in the developing and mature brain. The merging of neuroscience and epigenetics is sure to bring great discoveries in the near future, and I feel so fortunate to be a part of this venture."*



**POSTDOCTORAL FELLOWSHIPS**

**Dr. Sara Schaafsma & Dr. Donald Pfaff**  
Rockefeller University

Sex-Specific Gene-Environment Interactions Underlying ASD

This study investigates whether early pre-natal stress can help explain why males are so much more likely to develop ASD than females. We are looking at the effects of a bacterial infection in the pregnant mother on male and female mice offspring with a mutation in a gene that is linked to ASD risk.

*"I very much like this project because I think finding the origin of the male bias in ASD incidence can really shine light on the development of ASD."*



**POSTDOCTORAL FELLOWSHIPS**

**Dr. Teresa Tavassoli & Dr. Joseph Buxbaum**  
Icahn School of Medicine at Mount Sinai

Developing a Sensory Reactivity Composite Score for the New DSM-5

In the DSM-5, sensory reactivity difficulties, such as being overwhelmed by touch, are a new criterion for Autism Spectrum Disorder (ASD). This project tests and develops ways of measuring sensory reactivity. This project has discovered that a significant proportion of children with ASD (60-80%) showed sensory reactivity issues using three sensory measures. Better sensory reactivity phenotyping is an essential first step for reducing phenotypic heterogeneity seen in individuals with ASD and will help identify barriers to functioning that can then be addressed.

*"My postdoctoral fellowship from the Autism Science Foundation has been essential for my research. With the help of ASF I am able to develop robust ways of measuring sensory reactivity issues, which will ultimately help to guide diagnosis and treatment."*



**RESEARCH ENHANCEMENT GRANTS**

**Dr. Gazi Azad**  
University of Pennsylvania

Partners in Schools: A Program for Parents and Teachers of Children with Autism

The goal of Partners in Schools is to develop and test a model that will improve parent-teacher communication for diverse children with autism attending urban public schools. Successful completion of this project will result in a new culturally sensitive tool for improving communication, which will promote consistency of interventions and enhanced student outcomes. This understanding is the necessary first step for future research targeting family-school partnerships for children with autism.

*"As a former teacher, the students I had the most impact on were the students whose parents I worked very closely with. I'm excited to have the opportunity to make a broader impact by establishing partnerships between parents and teachers, which will ultimately improve the lives of so many underserved children."*



**RESEARCH ENHANCEMENT GRANTS**

**Dr. Haruki Higashimori**  
Tufts University

Role of Astrocytic Glutamate Transporter GLT1 in Fragile X Syndrome

Recently, we discovered that Fragile X Syndrome (FXS) mice have fewer and more dysfunctional astrocytic glutamate transporter GLT1. This transporter performs critical functions for maintaining very low glutamate concentrations in the brain. Building on work done in his Autism Science Foundation postdoctoral fellowship, Dr.Higashimori will not expand his findings in the rodent brain and will test his theory using human brain tissue samples. The goal is to assess whether there is a similar alteration in human glutamate transporter in FXS and autism patients.

*"I am interested in deciphering the developmental role of astrocytes in the normal and pathologic brain. Understanding the mechanism of tripartite synapse formation during development will give a clear assessment of astrocyte contribution to normal or dysfunctional synapse formation."*



**RESEARCH ENHANCEMENT GRANTS**

**Dr. Adam Naples**  
Yale University

Cross-Modal Automated Assessment of Behavior during Social Interactions in Children with ASD

This study is exploring the brain activity of individuals with ASD during simulated social interactions. By monitoring where a person looks at an onscreen face, we can make faces return gaze or change facial expression. This research enhancement grant will allow us to extend this research beyond eye gaze. Faces will respond to other non-verbal behaviors, such as facial expression or posture. This will give us insight into the brain bases of successful and unsuccessful social interactions in ASD.

*"I'm thrilled to have the opportunity to develop new ways of studying social interaction in ASD. The collaboration supported by this award will allow me to create responsive, interactive neuroscience experiments that adapt to the participant. We will finally be studying social brain function in social contexts."*





## RESEARCH ENHANCEMENT GRANTS

**Dr. Aaron Shield**

Boston University

The Effects of Autism on the Sign Language Development of Deaf Children

This project is a nationwide study of autism in deaf children who use sign language to communicate. These children have never before been studied, and it has been found that there are interesting differences (as well as similarities) in the ways that autism affects their language, compared to hearing children. Hopefully, by studying this rare and unique population, we can better understand how autism affects language and cognition in both deaf and hearing children.

*"It's been a privilege as well as a challenge to conduct this study: a privilege, because of the way that parents, teachers, and schools have embraced my research and encouraged me to find the answers to difficult and novel questions; and a challenge, because of the significant methodological obstacles entailed in this work. It is exciting as a researcher to work in an area where there are real mysteries to be solved, but where unlocking those mysteries could have a real impact on people's lives."*



## RESEARCH ENHANCEMENT GRANTS

**Dr. Jessica Suhrheinrich**

University of California, San Diego

Use of Video Feedback to Enhance Teacher Training

Although evidence-based practices for educating children with autism are being applied and studied in school settings, there is less information about the best ways to train teachers to use these practices. In this project, teachers of students with autism receive training and on-going coaching in an adapted evidence-based practice. The ASF research enhancement funding will support an investigation of the benefit of immediate video feedback, as compared to verbal and written feedback alone, during classroom coaching sessions.

*"The ASF mini-grant funding has allowed us to explore how teachers benefit from different types of coaching while learning evidence-based practices for children with autism. This has provided a great opportunity to draw additional data from a large randomized training study and for early-career researchers to work through a full grant cycle, from proposal development to implementation and data analysis. Thank you ASF!"*



## RESEARCH ENHANCEMENT GRANTS

**Dr. Pamela Ventola**

Yale University

Sex Differences in the Neural Mechanisms of Treatment Response

This project is investigating the neural mechanisms of response Pivotal Response Treatment (PRT). Dr. Ventola uses fMRI and EEG to measure neural-systems level changes to identify biological markers that may predict the magnitude of treatment response.

Historically, girls with ASD have been understudied. This grant enables Dr. Ventola to specifically recruit girls in order to investigate both behavioral and neural-systems level differences to foster development of more effective treatments for girls and boys.

*"Girls with ASD have historically been under-represented in research, particularly treatment research. Therefore, I am excited to explore sex-based differences in response to PRT. I think this project will inform our ability to better understand how and why treatment works for both boys and girls with ASD."*

# WORKING WITH OUR COMMUNITY

In January, ASF created the first ever Autism Research Glossary so stakeholders can better understand the latest autism science. Many scientific articles contain technical terms and jargon, which can make it difficult for many stakeholders to understand the latest scientific findings. This tool defines many of the scientific terms used in autism research publications, allowing stakeholders to better comprehend the latest science that impacts their families.

In February, ASF co-sponsored the Autism in India conference with the Hans Foundation. This workshop served to share information about early intervention and increase autism awareness in India. This collaborative conference, held in New Delhi, India, brought together some of the brightest minds in autism research to discuss new findings in autism science. The Autism Science Foundation and the Hans Foundation hope to jointly promote research that will benefit families affected by autism in India as well as America<sup>1</sup>.

In June, ASF participated in Harlem Health Day and shared new information about the early signs of autism with the community. Dozens of organizations gathered to spread knowledge about healthcare and healthy living<sup>2</sup>.

*"Harlem Health Day was a great opportunity to connect with families in Harlem and with other organizations dedicated to improving health awareness. We shared English and Spanish printed materials about the early signs of autism. In addition to providing information to the community, we did a lot of listening. Neighborhood residents who stopped by our table shared their stories, as well as their opinions on how to better support families of children with autism in Harlem and neighboring communities."*  
-ASF Intern Stephanie Millman

Our Science and Sandwiches program brings small, informal groups of scientists together with stakeholders in the autism community to share ideas and, of course, to eat sandwiches. Scientists benefit from hearing about the day-to-day experiences of families, and families hear directly about the latest in autism research from the scientists themselves. From this dynamic combination, big new ideas are often born!

For example, in October, Science and Sandwiches featured Dr. Kevin Pelphrey, a cognitive neuroscientist, the Harris Professor in the Child Study Center, Professor of Psychology, and the Director of the Center for Translational Developmental Neuroscience at Yale University. Dr. Pelphrey led discussion on "How Treatments for Autism Change the Developing Brain."

In October, ASF sponsored the 8th Annual Rockland Autism Symposium, a day-long learning event for special education teachers, regular education teachers, parents and other stakeholders. We are proud to have co-sponsored the event for the fifth year in a row! Of particular interest to the teachers was a presentation

by Dr. Josh Diehl of Notre Dame describing how specially designed robots can be integrated into special education classrooms to provide additional "on task time" for children with autism spectrum disorders<sup>3</sup>.



1. Dr. Jill Locke meets with professionals and stakeholders at the Autism in India conference  
2. ASF Interns Stefan Hansen, Stephanie Millman, and Jesse Eaton at Harlem Health Day  
3. Alison Singer, Dr. Josh Diehl, and "Kelly" the Robot at the Rockland Autism Symposium



# WORKING WITH SCIENTISTS

In February, ASF's Scientific Advisory Board and other world-renowned autism researchers, clinicians, and stakeholders met in New York to review pre- and post-doctoral grant applications.<sup>1</sup> In 2013, ASF received more applications than in any prior year.

To kick off Autism Awareness month in April, President Barack Obama announced the BRAIN Initiative (Brain Research through Advancing Innovative Neurotechnologies), which committed \$100 million in 2014 to advance our knowledge of the brain's billions of neurons and gain greater insight into diseases like autism, potentially finding therapies for a variety of brain-based disorders<sup>2</sup>. ASF President Alison Singer joined advocates, scientists, and other leaders from around the country in East Room of the White House for the announcement. ASF has invested in brain-based research since its launch in 2009<sup>3</sup>.

In April, for Autism Awareness Month, ASF hosted weekly Twitter Chats with leading autism researchers, including Dr. Simon Baron-Cohen, Dr. Jill Locke, and Dr. David Amaral. Stakeholders were able to directly ask the world's top scientists their questions about autism.

In May, ASF co-sponsored the International Meeting for Autism Research in San Sebastian, Spain, where scientists from around the world gathered to present their latest findings<sup>4</sup>.

ASF Programs Manager Donielle Johnson presented her poster, "Service Needs of Adults with Autism" at IMFAR. This study identified 4 key areas that needed to be addressed to improve the lives of adults with autism, including instruction in "on the job" social skills and improved training for parents of adults with autism<sup>5</sup>.

1. ASF's Scientific Advisory Board meets to review pre- and post-doctoral grant applications
2. President Barack Obama announces the BRAIN Initiative
3. NIMH Director Dr. Thomas Insel, Dr. Eric Kandel, and Alison Singer at the BRAIN Initiative announcement
4. Press Conference at the 2013 International Meeting for Autism Research
5. Donielle Johnson presents ASF-sponsored research at IMFAR



# WORKING TOGETHER

We are so thankful for the many community volunteers who organize fundraising events to help support the Autism Science Foundation.

In April, Super Zumba instructor Gustavo Lopez of Dance2BFit in Mamaroneck, NY led adults and children of all ages and fitness levels in Zumba Mania -- a three-hour Zumba marathon that resulted in over 100 zumba-ers coming together to raise over \$2000 for autism research<sup>6</sup>.

The Scarsdale High Schools Autism Science Foundation Student Club hosted its annual silent auction to support autism research in May. In October, the students baked sweet treats for their annual Halloween bake sale<sup>7</sup>.

Scoring Goals for Autism is an annual soccer tournament fundraiser that benefits the Autism Science Foundation. Volunteers Erin Lopes and Tim Bak organize this one-of-a-kind indoor soccer tournament that offers competitive team play for adult players as well as a TOPSoccer skills and drills clinic for children and adolescents with disabilities including autism. In May, Erin and Tim hosted the third annual Scoring Goals for Autism tournament in Philadelphia<sup>8</sup>.

Since 2010, the AML Bikers for ASF group generously supports the Autism Science Foundation with an annual motorcycle ride fundraiser. The team rides 100 miles from the south shore of Long Island to the Montauk Lighthouse. In June, AML Bikers hosted their fourth annual ride to support autism research<sup>9</sup>!

In December, our annual year-end Recipe4Hope campaign featured the Millman family and the beautiful relationship between Alex, who is diagnosed with autism, and his older sister Stephanie (who works as an intern at the Autism Science Foundation!). The winter campaign showcased Alex and Stephanie's love of the beach, helping us all think warm thoughts during the snowy holiday season<sup>10</sup>.

6. Girls zumba-ing in support of ASF
7. Scarsdale High School's ASF Student Club hosts their annual Halloween bake sale
8. Soccer players at Scoring Goals for Autism
9. ASF board member Gregg Ireland and his wife Lori at ASF's Scientist Appreciation Dinner at IMFAR
10. Stephanie and Alex Millman at the beach, as featured in the 2013 Recipe4Hope campaign

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