



**Testimony**  
**Before the Subcommittee on Labor, Health**  
**and Human Services, Education, and**  
**Related Agencies**  
**Committee on Appropriations**  
**United States Senate**

**“Autism Research, Treatments, and**  
**Interventions”**

*Statement of*  
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Mr. Chairman and Members of the Subcommittee:

I am pleased to address the state of autism spectrum disorder research and include a brief review of the prevalence of the disorder, research findings, and recent initiatives at the National Institutes of Health (NIH).

Autism spectrum disorder (ASD) is a group of complex neurodevelopmental disorders that range in severity and that are characterized by social impairments, communication difficulties, and restricted, repetitive, and stereotyped patterns of behavior. The most recent Centers for Disease Control and Prevention estimate for ASD prevalence indicates that 1 in 150 children in the United States is affected by the disorder—more than a 10-fold increase from the early 1990s.<sup>1</sup> While much of this increase appears to be due to factors such as the use of broader definitions for ASD, better diagnostic tools, or increased ascertainment, recent research demonstrates that none of these factors fully explain the increase in ASD prevalence. Whatever the cause, scientists, clinicians, and families now agree that ASD has now become an urgent public health challenge, with enormous financial and societal costs. Estimates of the combined direct and indirect costs to care for all Americans with ASD during their lifetimes exceed \$34 billion,<sup>2</sup> with estimated costs for each person over his or her lifetime totaling \$3 million.<sup>3</sup> Families often incur large debts for medical and education services that public programs or medical insurance do not cover. Beyond the financial costs, ASD often leads to profound emotional hardships for persons with the disorder and their families. As more children with ASD become adults with ASD, access to services and lack of accommodation is a growing challenge.

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<sup>1</sup> Centers for Disease Control and Prevention. Prevalence of Autism Spectrum Disorders: Autism and Developmental Disabilities Monitoring Network, 14 Sites, US, 2002. *MMWR* 56 (SS-1), 2007.

<sup>2</sup> Ganz, ML. *Arch Pediatr Adolesc Med*. 2007 Apr;161(4):343-9.

<sup>3</sup> Ibid.

Matching the increasing public health urgency, NIH research funding for ASD has increased progressively over the past decade, reaching \$118 million in fiscal year (FY) 2008, an increase of nearly 6-fold from FY 1998. What has been the yield from this investment? I will summarize the research findings in three areas: diagnosis, causes, and treatment. A fundamental insight and challenge is the heterogeneity of ASD. While we use one diagnostic category, research increasingly demonstrates that ASD covers many disorders, with different causes and possibly requiring different treatments.

Diagnosis: Early diagnosis is critical because earlier interventions are associated with the best outcomes. Research has found that by age 2 children with ASD show unusual patterns of eye contact compared with typically developing children. Recent studies of children at high risk for ASD indicate the potential for even earlier detection. Simple tests of joint attention or responding to spoken name suggest that diagnosis at 12-14 months of age may be possible for many children. Ongoing research using neuroimaging and serum samples is looking for a biomarker that might permit diagnosis even earlier.

Causes: Scientists are looking for genetic and environmental causes across the autism spectrum. In the past 2 years, genetic research has proven especially informative, as more than 50 variations in the genome, alone or in combination, have been linked to ASD. Importantly, several new, rare mutations have been discovered. Along with known genetic disorders that cause ASD, such as Fragile X and Rett Syndrome, these new mutations may collectively account for 10 to 15% of ASD cases. These rare mutations and the many common variations which confer risk for ASD have one striking thing in common – nearly all of the genes implicated are critical for brain development. In fact, most are closely linked in the developing synapse – the connection between neurons – suggesting that ASD can now be approached as a synaptic

disorder and that new treatments can be developed for specific synaptic targets.

Just as with other complex medical disorders, ASD research increasingly focuses on the interaction of environmental factors with genetic vulnerability. For ASD, the research evidence has pointed to prenatal environmental factors as most salient. While there is increasing research into environmental factors that might contribute, thus far no one factor appears to explain the large number of, or apparent increase in, cases of ASD.

Treatment: In addition to breakthroughs in the diagnosis and causes of ASD, recent research has shed light on the treatment of ASD. NIH-supported randomized, controlled trials of behavioral treatment approaches have shown positive effects, and early behavioral interventions have been found to improve functional capabilities and reduce the severity of challenging symptoms.<sup>4</sup> Additionally, NIH has supported double blind randomized controlled trials of pharmacological treatments. For example, the atypical antipsychotic medication risperidone was shown to be better than placebo for reducing aggression, self-harming behavior and other serious behavioral problems, without impairing the cognitive skills of children with ASD.<sup>5</sup> Conversely, a multisite controlled trial to evaluate the efficacy of the antidepressant citalopram to treat the occurrence of stereotyped, repetitive behaviors in children with ASD found that this medication worked no better than placebo.<sup>6</sup> Double blind, placebo controlled trials are essential for assessing treatments for ASD. Positive effects are frequently observed with new experimental interventions for ASD, but equivalent effects are often seen with placebo. Only by including rigorous controls can we attribute clinical improvement to the experimental intervention.

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<sup>4</sup> Rogers, SJ, & Vismara LA, *J Clin Child Adol Psychol*. 2008; 37(1): 8-38.

<sup>5</sup> Aman MG, Hollway JA, McDougle CJ, Scahill L, et al. *J Child and Adolesc Psychopharmacol*. 2008 Jun; 18(3): 227-236.

<sup>6</sup> King BH, Hollander E, Sikich L, McCracken JT, et al. *Arch Gen Psychiatry*. 2009;66(6):583-590.

NIH will play a major role in the Administration's new initiative to significantly increase services and research into the causes of and treatments for ASD. Prior to this initiative, NIH will be using funding from the American Recovery and Reinvestment Act of 2009 (Recovery Act) as an opportunity to fuel further research on ASD, including its underlying biology, methods for earlier and more effective diagnosis, and improvements in treatment. The new [Interagency Autism Coordinating Committee \(IACC\) Strategic Plan for Autism Spectrum Disorder Research](#), released in January 2009, provides the scientific goals and benchmarks for this endeavor. The Combating Autism Act of 2006 (CAA) requires the IACC to develop and annually update this Plan. The IACC is currently in the process of monitoring the implementation of the Plan and gathering information to update the document in January 2010. With the arrival of the Recovery Act funds, we will be jumpstarting many of the short-term objectives in the Plan, utilizing economic recovery to support science that facilitates the best possible outcomes for individuals with ASD and their families.

NIH recently issued a series of Recovery Act funding opportunity announcements (FOAs) to address ASD, entitled "[Research to Address the Heterogeneity in Autism Spectrum Disorders](#)." This collaborative effort among several NIH Institutes and Centers is the largest single funding opportunity for ASD research in NIH's history. The FOAs encouraged applications for two-year projects that address ASD measurement, identification of biomarkers and biological signatures, immune and central nervous systems interactions, genetics/genomics, environmental risk factors, and ASD intervention and treatment. Participating NIH Institutes intend to contribute over \$60 million of Recovery Act funds to support many of the grant applications received in response to this initiative. Additionally, NIH will be supporting ASD research with Recovery Act funding through the [Challenge Grants in Health and Science](#)

[Research](#) Program ([RFA-OD-09-003](#)) and the Grand Opportunity grants ([RFA-OD-09-004](#)).

Targets for these grants included improving access to services by individuals with ASD and their families and expanding NIH's National Database for Autism Research (NDAR) in order to accelerate the availability of new data for the ASD research community. NIH has recently completed the scientific peer review of the Recovery Act applications. The advisory councils for each NIH Institute and Center are currently in the process of evaluating the reviewed applications in order to guide final funding decisions, which are expected shortly.

Finally, NIH will continue to build its investment in ASD research via its base budget, which supports a broad range of individual grants for research and training related to ASD, a new intramural program for ASD research, and the Autism Centers of Excellence (ACE) program. The ACE program focuses on identifying the causes of ASD and developing new and improved treatments. An example of the kinds of innovative research emerging from the ACE program is the Early Autism Risk Longitudinal Investigation ([EARLI](#)). Coordinated by researchers at the Drexel University ACE network, EARLI will explore the impacts and interplay of environmental factors and genetic predisposition in the cause of ASD. About 1,200 mothers of children with ASD will be followed as soon as they become pregnant again and throughout the early life of the new baby. Through extensive data collection on a number of possible ASD environmental risk factors and biomarkers, the study holds great promise in advancing understanding of the causes and progression of ASD.

In summary, ASD is a developmental disorder that affects too many families; research represents our best hope for making a difference for them. We at NIH are determined to continue to use the best available tools, to fund excellent and innovative science, and to encourage input from--and dialogue with--parents, teachers and individuals with ASD. Only in

this way, and only with your continued support, will we be able to continue to fuel the vital research that we believe will reveal the mysteries of ASD and lead to prevention and effective treatments.

I appreciate the interest of the members of this Subcommittee on ASD research and look forward to answering your questions.