



**Written Statement  
Subcommittee on Wellness and Human Rights  
House Committee on Government Reform  
United States House of Representatives**

**NIH Autism Research Activities**

*The Future Challenges of Autism:  
A Survey of the Ongoing Initiatives*

*Written Statement of*

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I am Dr. Ann Wagner, Chief of the Autism Interventions Research Program in the Division of Services and Interventions Research (DSIR) at the National Institute of Mental Health (NIMH), a component of the National Institutes of Health (NIH). I am pleased to submit this written statement for the record on behalf of the NIH. In addition to my role in DSIR, I am actively involved in the coordination, planning, and oversight of autism research activities at NIH, many of which involve inter-Institute collaborations. For example, I am a member of the NIH Autism Coordinating Committee (NIH/ACC). I have been an active participant in the organization and implementation of the NIH centers program called for in the Children's Health Act of 2000 (P.L. 106-310), which we have named the Studies to Advance Autism Research and Treatment (STAART) Centers. I also serve as the Executive Secretary of the Department of Health and Human Services (HHS) Interagency Autism Coordinating Committee (IACC) created under a provision of the Children's Health Act of 2000.

Thank you for an opportunity to discuss NIH research initiatives on autism spectrum disorders. I am a clinical psychologist who came to NIH after a career of providing clinical services for children with autism and their families. As a clinician I have seen firsthand the desire for more definitive empirical answers to clinical questions that can lead us to effective treatments. Over the course of my clinical career I got to know hundreds of families affected by autism spectrum disorders and have a deep appreciation for their urgent need for answers about how best to treat – better yet, to prevent – this serious developmental disorder.

The Children's Health Act of 2000, P.L. 106-310, signed into law on October 17, 2000, authorized the Secretary of HHS to conduct additional activities relevant to autism

and pervasive developmental disorders, including expansion, intensification, and coordination of activities of the NIH with respect to research on autism; developmental disabilities surveillance and research programs; expanded information and education activities; and, establishment of an Interagency Autism Coordinating Committee. The Act also requires that an annual report be prepared and submitted to Congress. The Secretary submitted the first such annual report in mid-2001, and the second annual report in November 2002, and the third report is in preparation. My testimony will emphasize the progress and expansion that has occurred over the past year with regard to research activities.

### **NIH Research Activities**

Within NIH, five institutes are members of the NIH Autism Coordinating Committee (NIH/ACC), an internal body made up of all of the institutes that are conducting autism-related research. Members include NIMH, the National Institute of Child Health and Human Development (NICHD), the National Institute of Neurological Disorders and Stroke (NINDS), the National Institute on Deafness and Other Communication Disorders (NIDCD), and the National Institute of Environmental Health Sciences (NIEHS). In addition, a staff representative from the National Institute of Allergy and Infectious Diseases (NIAID) participates in meetings. The NIH/ACC functions in a coordinating role for autism research activities funded and conducted by the NIH Institutes.

Over the past six years NIH autism research dollars have risen from approximately \$22 million in 1997 to \$74 million in 2002. Today I will emphasize major activities involving research networks and centers, but it is important to bear in mind that

NIH also funds individual autism research projects in the areas of diagnosis, genetics, neuroimaging, neurobiology, psychopharmacological and behavioral interventions, and services research.

***Research Units on Pediatric Psychopharmacology and Psychosocial Interventions (RUPP-PI)***

The Research Units on Pediatric Psychopharmacology (RUPP) network was established in 1996/1997 with an initial focus on the multi-site clinical trials of medications that are commonly used in children without adequate data on safety and efficacy. This network has provided a model for designing and implementing multi-site clinical trials with children and adolescents with autism spectrum disorders. In 2002 the network was renewed and expanded to include psychosocial and behavioral interventions as well as pharmacology, and renamed to reflect that change. The Research Units on Psychopharmacological and Psychosocial Interventions (RUPP-PI) sites that are focused on autism spectrum disorders are located at:

- Indiana University
- Kennedy Krieger Institute (Maryland)
- Ohio State University.
- University of California, Los Angeles
- Yale University (Connecticut)

***Children's Centers for Environmental Health and Disease Prevention***

NIEHS, in partnership with the U.S. Environmental Protection Agency (EPA), has continued its support of autism research through a national network of Centers for Children's Environmental Health and Disease Prevention Research. Two of the Centers, funded in August 2001, focus on possible environmental aspects of autism and related neurodevelopmental disorders. They are located at:

- University of California, Davis

- University of Medicine and Dentistry of New Jersey.

Within the past year, the UC-Davis Center has begun enrollment of autistic children in the first large-scale epidemiologic case-control study of environmental risk factors for autism. This study is collecting extensive information to assess a variety of environmental exposures in the prenatal and postnatal period. The Center at the University of Medicine and Dentistry of New Jersey has begun enrollment and evaluation of children in a unique study designed to provide a comprehensive evaluation of personal, residential and community environments of children with autism to identify any sources of harmful exposures and to develop strategies to minimize risk from such exposures. Several basic science projects conducted at this Center are using cellular and animal models to explore the interaction of model neurotoxicants such as lead and methyl mercury with aspects of early brain development and with the emergence of specific behavior patterns.

***The NICHD/NIDCD Network on the Neurobiology and Genetics of Autism***

Established in 1997 and renewed in 2002, the NICHD/NIDCD Network on the Neurobiology and Genetics of Autism currently consists of ten Collaborative Programs of Excellence in Autism (CPEAs). Each CPEA has a site-specific, multidisciplinary research program on the causes, brain substrates, functional characteristics, and clinical development of autism spectrum disorders. In addition, each site participates in some trans-Network collaborative studies for which no one individual site has the needed expertise and/or subject population. The CPEA network is now studying the world's largest group of well-diagnosed individuals with autism who have been characterized with regard to genetic and developmental profiles.

NIH has renewed the CPEA sites for an additional 5 years of funding. NICHD

and NIDCD plan to allocate \$60 million in this time period to sustain and enhance the CPEAs. A data-coordinating center (DCC) was established this year to expedite and maximize analysis of the data generated by the CPEA research projects as well as the STAART network. This DCC has started to provide administrative support for all network activities and implements a Web site to foster organization, communication and collaboration. The CPEA sites are located at:

- Boston University (Massachusetts)
- University of California, Irvine
- University of California, Los Angeles
- University of California, Davis
- University of Texas
- Yale University (Connecticut).
- University of Washington
- University of Pittsburgh (Pennsylvania)
- University of Rochester (New York)
- University of Utah

### ***Centers of Excellence***

The Children's Health Act of 2000 called for NIH to establish at least five Centers of Excellence in Autism Research. The goal was to establish several research centers that would bring together expertise, infrastructure, and resources focused on major questions about autism. A total of eight centers were funded, two in 2002 and six in 2003, and are named the Studies to Advance Autism Research and Treatment--STAART Centers Program. These centers are located at:

- University of North Carolina, Chapel Hill
- Yale University (Connecticut)
- University of Washington
- University of California, Los Angeles
- Boston University (Massachusetts)
- University of Rochester (New York)
- Kennedy Krieger Institute (Maryland)
- Mt. Sinai Medical School (New York).

The STAART Centers will contribute to the understanding of autism by investigating early detection through behavioral and/or biological markers, efficacy of early interventions, early course of core features, biological basis of the core deficits, efficacy of treatments for social deficits, efficacy trials for pharmacotherapy, genotypic and phenotype of response to treatment, and identification of susceptibility genes. Plans for collaborative projects include multi-site clinical trials within the STAART network, as well as interaction with the CPEA. A single data coordination center supports both CPEA and STAART network functions. These Centers have been awarded with 5-year grants, for approximately \$65 million in total. Coordination and collaboration between the STAART Centers and the CPEA Network is being facilitated in many ways. The single data coordinating center for both networks will facilitate the use of common measures, common databases and data sharing. A joint Steering Committee meeting will occur annually; the first of which is being planned for May 2004 in Washington, D.C. NIH has convened a group of Scientific Advisors, made up of senior scientists and public representatives, who are external both to the NIH and to the funded centers, for oversight and advice on research directions. Cross-network subcommittees and interest groups have been formed and are already active. Investigators at all centers within both networks have adopted these activities with enthusiasm and a strong collaborative spirit.

### ***Genetics and Tissue Resources***

NIH has undertaken several activities to increase the quality and availability of genetic and tissue resources to the autism research community. The NIMH Genetics Repository is a national resource that collects, stores, and distributes such materials broadly across the scientific community. Activities for 2003 include an administrative supplements program to promote data sharing in ongoing NIH autism projects. Four NIH Institutes and Centers committed \$2.25

million to support the collection and timely sharing of family data and DNA from their ongoing projects through the NIMH Autism Genetics Initiative (<http://nimhgenetics.org>). Eight applications were funded to provide data and materials on 500 new families through this program. Another administrative supplements program promotes data sharing in ongoing STAART Center projects. Through this program, additional clinical data and DNA on 100 new families, 500 unrelated affected individuals, and 150 autistic individuals enrolled in a new clinical trial will be shared with the scientific community through the NIMH Autism Genetics Initiative.

In addition, NINDS continues to support promising research in the genetics of autism, including core grant support for the development and expansion of genetics resources. This support will enhance the ability of research groups located in the United States, Canada, Europe, and Israel to work collaboratively to discover genes that contribute to autism. These research groups have created a genetic data bank that can be shared across laboratories, greatly increasing the power to discover genes involved in autism.

To take advantage of new molecular methodologies, NIMH, in collaboration with the autism community and other NIH Institutes, is stepping up efforts to establish brain bank collections to study autism. Activities in 2003 included an Autism Brain Banking Workshop in March that was sponsored by the NIH/ACC Institutes. This workshop evaluated current best practices for tissue collection and distribution and proposed effective new mechanisms and infrastructure needed to enhance tissue research in autism. Subsequently, NIMH, NINDS and NIDCD announced a joint effort in supplementing the Harvard Brain Tissue Resource Center (whose principal funding comes from NINDS and NIMH) for the creation and maintenance of a National Autism Brain Bank.

### ***Interagency Autism Coordinating Committee***

The Children's Health Act of 2000 authorized the establishment of an interagency autism coordinating committee to coordinate research and other efforts with regard to autism within the HHS. Secretary Tommy Thompson delegated the authority to establish the IACC to the National Institutes of Health (NIH) in April 2001. The NIMH at the NIH has been designated the lead for this activity. The Committee is established and has had five semi-annual meetings and a sixth scheduled for this Friday, November 21, 2003.

Government agencies represented include: NIH/ACC members (NIMH, NICHD, NIDCD, NIEHS, and NINDS), the Health Resource Services Administration (HRSA), the Centers for Disease Control and Prevention (CDC) (specifically, the National Center on Birth Defects and Developmental Disabilities), the Agency for Toxic Substances and Disease Registry (ATSDR), the Substance Abuse and Mental Health Services Administration, the Administration for Children and Families (specifically, the Administration on Developmental Disabilities), the Food and Drug Administration (specifically, the Center for Biologics Evaluation and Research), the Centers for Medicare and Medicaid Services, the Agency for Healthcare Research and Quality, and the Department of Education (ED) (specifically, the Office of Special Education and Rehabilitative Services). A summary of each meeting is posted when available on the NIMH Web site (see <http://www.nimh.nih.gov/events/interagencyautism.cfm>).

The IACC has established subcommittees on autism screening and the organization of autism treatment services. Both subcommittees are now working to coordinate activities among IACC members and with the relevant stakeholders in the medical and services communities.

### ***IACC Science Panel and Matrix Development***

The House and Senate conferees considering the FY2003 appropriations for the

Departments of Labor, Health and Human Services and Education, House Report 108-10, requested that the IACC “convene a panel of outstanding scientists to assess the field of autism research, and identify roadblocks that may be hindering progress in understanding its causes and best treatment options.” The final product was to be the development of a research matrix focusing on the causes and best treatment options for autism. In response to this request, the IACC convened a panel of science experts to document both roadblocks to understanding causes and best treatment options for autism, as well as goals and activities to overcome these roadblocks. A list of roadblocks was created, and the autism research matrix was designed to include goals and activities for the next 10 years. The goals and activities generally fall within the following categories: characterization of autism (i.e., phenotype), screening, early intervention, school and community interventions, specific treatments, neuroscience and epidemiology. The science panel suggested items for the matrix last July, and the membership of the IACC will approve the final version of the matrix on November 21.

### ***Autism Summit Conference***

In order to expand on the work of the IACC, particularly the work of the subcommittees on early screening and services, a national conference focusing on the federal Government’s role in biomedical research, early screening and diagnosis, and improving access to autism services began yesterday and continues through today at the Washington Convention Center in Washington, DC. This event is entitled “The Autism Summit Conference: Developing a National Agenda.” The goal of the meeting is to provide information to the public on Federal activities relevant to autism and to provide an opportunity to the public to share information with Federal agencies. This is another step in our progress toward a unified national agenda for Federal autism activities. The planning committee for this conference is composed of several Federal and public members of the IACC, and the meeting was officially co-sponsored by HHS

and DOE. Three broad themes will be addressed: a) a public presentation of the autism research matrix; a) screening and diagnosis for review of existing screening instruments and discussion of current clinical practices; and b) autism services throughout the lifespan including discussion of integrating services provided by different systems.

In summary, there has been considerable expansion of Federal programs to support research related to autism spectrum disorders. These activities adhere to applicable law protecting personal and medical data. Communication and coordination among Federal agencies is improved and exciting partnerships between public and private groups are being formed. Enhancing the quality and coordination of autism research activities across the Federal Government remains a high priority.