



Written Testimony provided for the
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Good morning, Mr. Chairman. I am Dr. Geraldine Dawson, Chief Science Officer of Autism Speaks. I also serve as a Research Professor of Psychiatry at the University of North Carolina at Chapel Hill.

I am honored to appear before the Senate Appropriations Committee Subcommittee on Labor, Health and Human Services, at its second hearing on autism and the status of autism research, treatments and services. I want to thank this Subcommittee and you, Mr. Chairman, for your leadership in providing full funding of the Combating Autism Act over the last three years, and more specifically, for your most recent Fiscal Year 2010 appropriations bill.

Autism Speaks was founded in February 2005 by Bob and Suzanne Wright, grandparents of a child with autism. Since then, Autism Speaks has grown into the nation's largest autism science and advocacy organization, dedicated to funding research into the causes, prevention, treatments and cures for autism; increasing awareness of autism spectrum disorders; and advocating for the needs of individuals with autism and their families. We are proud of what we've been able to accomplish and look forward to continued success in the years ahead.

Here are a few facts about autism:

- According to the Centers for Disease Control and Prevention, autism is diagnosed in 1 in 150 children in the U.S., and 1 in 94 boys.
- A decade ago, experts estimated the prevalence of autism to be 1 in 2,500.
- This year more children will be diagnosed with autism than with AIDS, diabetes and cancer combined.
- Autism costs the American economy more than \$35 billion in direct and indirect expenses each year, according to a Harvard School of Public Health study. Caring for a child with autism is estimated to cost over \$3 million over that person's lifetime.

When Bob and Suzanne Wright founded Autism Speaks just four years ago, they were shocked that a disorder as prevalent as autism commanded so little in terms of resources devoted to research and treatment - when compared to other, less common disorders. That disparity continues, despite greater public and Congressional awareness, and despite the efforts of you and this subcommittee to direct additional resources into autism research.

- For example, leukemia affects in 1 in 25,000 people but receives research funding of \$310 million a year.
- Pediatric AIDS affects 1 in 8,000 children; its funding, \$255 million a year.
- Compare these figures with those for autism, which affects 1 in 150 children and yet NIH funding for Fiscal 2009 is estimated to be \$122 million.

To close the gap, Autism Speaks raises its own funds to support autism research. We hold walks and special events, and cultivate major giving opportunities for individuals as well as corporations and foundations all to help accelerate the pace of autism research. We are proud to be putting our hard-earned dollars to work to move forward in finding a cure for autism.

Through our grants (scientist-initiated requests for funding) and initiatives (targeted projects designed by Autism Speaks), we support research designed to answer questions poised in each of five targeted areas: What causes it? (Etiology); What is it? (Biology); How do you know if someone has it? (Diagnosis); How do we make it better? (Treatment); and How do we effectively disseminate best practices to the community? (Dissemination). Since its founding in 2005, Autism Speaks has committed over \$130 million toward autism research.

But we realize, Mr. Chairman, that our non-profit, no matter how successful we may be in raising funds, cannot match the resources that the Federal government could marshal in the campaign to find treatments and cures for autism. For that reason, we also worked together with thousands of families affected by autism to introduce, pass and have former President George W. Bush sign into law the Combating Autism Act in December 2006. This historic act, considered by some to be the most comprehensive piece of single-disease legislation ever passed by Congress, authorizes appropriations of \$920 million over the 5 year period, from Fiscal 2007 to 2011, for autism research, surveillance, awareness and early identification.

Combating Autism Act

Mr. Chairman, let me elaborate on each of the 3 sections of the CAA.

For the NIH, the funding increases are incremental, from \$100 million in Fiscal 2007 to an authorized level of \$158 million for Fiscal 2011. Most important, the Act directs NIH to spend those dollars strategically, according to a Strategic Research Plan devised by the Interagency Autism Coordinating Committee (IACC) with consumers and advocates comprising a third of its membership. The Act also directs NIH to increase its investment in research into potential environmental causes of autism.

For the CDC, whose autism portfolio would increase from \$15 million to \$21 million over the 5 year span, its increased funds are targeted for expansion of its awareness and intervention activities to reach new parents, health care professionals, and health care providers.

The Health Resources and Services Administration (HRSA) autism activities, which would increase from \$32 million in Fiscal 2007 to \$52 million by Fiscal 2011, are designated for creation of new and innovative state-based programs in autism education, detection and early intervention and for best practices research on autism interventions, an area in which Autism Speaks is at the forefront through a cooperative agreement between our Autism Treatment Network and HRSA.

Our investment in the Autism Treatment Network, a collaborative network of 15 hospitals providing care for children with autism, is now being leveraged by the federal government through this HRSA funding and through a developing collaboration with Dr. Susan Swedo's NIMH Intramural Research program on autism. The establishment of research networks by private funders, like Autism Speaks, has created the infrastructure that can now benefit both HRSA and the NIH, providing significant time and cost savings. The collaboration with the NIMH Intramural Research Program is an excellent example of a productive public-private partnership.

Autism Speaks also recently contributed \$5 million to expand and link two NIH-funded large-scale, multi-site studies investigating genetic and environmental risk factors for autism in 2,000 infant siblings of children with Autism Spectrum Disorders (ASDs). These are only a couple of examples of the many ways that Autism Speaks is collaborating with the NIH to facilitate research on the causes and treatment of autism.

President Obama and Autism

Congress' commitment to autism has now been matched by the President. Last year, Mr. Obama committed to increasing ASD funding for research, treatment, screenings, public awareness and support services to \$1 billion annually by the end of his first term in office. He also pledged to end insurance discrimination against people with autism. (Of course, Autism Speaks is now working to bring this promise to fruition through the health care reform process.)

President Obama fulfilled his campaign promises in the Fiscal 2010 budget he submitted to Congress in February of this year. That budget includes \$211 million in HHS funding for research into the causes of and treatments for ASD, screenings, public awareness, and support services.

Appropriation's Leadership

The authorizations and Presidential budgets will mean little if not matched by real dollars appropriated by your subcommittee, Mr. Chairman. And you have done a heroic job of identifying and targeting the resources to match the authorization targets set by the Combating Autism Act for HHS's autism activities.

In your Fiscal 2008 and 2009 bills, the Senate Appropriations Committee matched or beat the CAA authorized levels for CDC and HRSA. And in each of those years, you also provided the resources to NIH to create and fund the operational costs of the IACC, and to adhere to the research funding targets set by the Combating Autism Act. As of just last week, the Senate Appropriations Committee Fiscal 2010 bill provides \$71 million for CDC and HRSA's autism activities, the same levels proposed by the President.

On behalf of Autism Speaks, I also want to thank you for ensuring that \$2 million of the increase proposed for HRSA's autism activities will be targeted for expansion of research on evidence-based practices for interventions for individuals with autism and other developmental disabilities, for development of guidelines for those interventions, and for information dissemination, as directed by the CAA. As I mentioned earlier, this program, in particular, is already being leveraged to the benefit of other federal research.

State of Autism Research

Causes of autism. Most scientists agree that autism is caused by a combination of genetic susceptibilities which interact with environmental risk factors. We have learned that autism is a complex genetic disorder involving many genes. In recent years, studies have shown that several rare mutations confer significant risk for autism. While we have discovered some of the risk genes for autism, we still know little about the potential environmental risk factors. We also have come to understand that there are many different causes of autism, and that any one cause will likely only explain a minority of cases. Piece by piece, we must discover each of these causes so that effective treatments and prevention will be possible.

To identify the genetic and environmental risk factors for autism, it will be necessary to conduct large scale population-based studies that broadly examine both genetic susceptibilities and environmental exposures. The NIH National Children's Study (NCS), a prospective longitudinal study of 100,000 children in the US, potentially provides such an opportunity and should be fully utilized to increase our understanding of autism. Autism Speaks is currently advising the staff of the National Children's Study on ways the study could be leveraged to better understand how genetic and environmental risk factors may increase risk for autism. Autism Speaks also is funding expansion of two NIH-supported Autism Center of Excellence Network studies to include the capture of a number of environmental exposures and the analysis of how they interact with genetic vulnerability. In addition to funding large scale studies of risk factors, continued investment in large scale data bases and biorepositories, such as the National Database for Autism Research, the Autism Genetic Resource Exchange, and the Autism Tissue Program, will be crucial for understanding the causes of this complex disorder. By identifying the causes, we will be able to identify individuals at risk for the disorder as early as possible and develop methods for treating and preventing autism.

Biological mechanisms. We are also gaining an understanding of the underlying biological mechanisms in autism. Evidence is converging that autism may be fundamentally a problem of the synapse, the connection between brain cells (neurons). Neuroscientists are mapping the biochemical pathways that appear to be disrupted in autism and that are interfering with how neurons in the brain communicate with each other. Some of the very best neuroscientists in the world are working hard at understanding how genetic mutations can change the way in which neurons communicate and developing therapeutic strategies that might restore synaptic function. While this work is offering real hope, the pace of discovery is simply too slow in large part because of a lack of adequate funding for such research. Barriers to translational science that can bring basic findings from the bench to the bedside are significant. The gulf between a basic discovery and the development of a new treatment is so overwhelming that it has been called "a valley of death" by some scientists. Scientists are ill-equipped to develop the technologies that are required for translational research, and funding of such research is often a lower priority relative to discovery-focused research at the NIH. Thus, translational research must be supported through multi-disciplinary collaboration and targeted funding to support technological and scientific advances that will help pave the way to treatment, prevention, and cure. This is why Autism Speaks strongly supports Senator Specter's "Cures Acceleration Network" (CAN Act) and hopes that the policies addressed in that bill will be incorporated into the final health care reform legislation.

It will be crucial for the federal government to provide the necessary resources needed to move scientific discovery from the lab to the clinic. Autism Speaks is poised to help in this process not only with scientific grants, but also with our support for the large scientific data bases, such as the Autism Genetic Resource Exchange, and collaborative networks that are capable of conducting clinical research and disseminating evidence-based methods, such as the Autism Treatment Network and the Clinical Trials Network.

Early detection and early behavioral intervention. Two areas in which we have made significant progress in the past several years are early diagnosis and early intervention. Screening methods for autism now exist for children as young as 18 months of age, and the American Academy of Pediatrics now recommends that all babies be screened for early signs of autism at 18 and 24 months of age. Autism Speaks and the NIH are funding several studies focused on detecting autism in even younger infants. Building on these important findings, researchers have developed a variety of protocols to intervene at early ages, and Autism Speaks is funding several clinical trials that are evaluating the efficacy of these intervention methods for infants as young as 12 months of age. The hope is that, by detecting autism early in life before the full syndrome has become manifest, we will be able to reduce its severity or even prevent the syndrome from developing. However, the impact of this work will not be felt unless pediatricians are using the available screening methods and parents have access to trained professionals who can deliver these interventions. That is why it is critical that we continue to study dissemination methods, invest in training professionals and caretakers, and support federally-mandated insurance coverage for behavioral interventions which are very cost effective in the long run.

Treatment across the lifespan. While behavioral interventions are effective for some individuals, there is significant individual variability in response to such interventions. Biological research is offering hope that medical interventions that address core symptoms of autism will someday be available. Today, however, many individuals with autism suffer without relief both from the autism itself and also from a range of medical conditions that are associated with autism, such as sleep disorders, gastrointestinal problems, epilepsy, anxiety, and depression. Very few clinical trials have been conducted that address these co-morbid medical conditions. Practice standards that can guide physicians on how to detect, assess, and treat these conditions do not exist. Virtually no comparative effectiveness studies have been conducted to evaluate which treatment methods are more effective for treating core autism or its associated conditions. Thus, parents are left to sort through confusing and often inaccurate information about various treatment claims and options. Without the benefit of comparative effectiveness research, clinicians are often at a loss in helping parents make evidenced-based treatment decisions. This is a gap in autism research that must be addressed.

It is imperative that enhanced effort be directed toward developing effective treatments across the entire lifespan. Very little research has been conducted to address the issues that adults with autism face, despite the fact that adult care accounts for the bulk of the \$35 billion that is spent annually on caring for individuals with autism in the US. Unlike other health conditions, we have very limited information about autism health care utilization, barriers to access, health care disparities in the US, or cost-effectiveness models.

A major barrier to appropriate treatment for individuals with autism is lack of access to well-trained providers. Research must identify the most effective, scalable, and feasible models for community-based delivery of services. This will require identifying factors that impede or promote the adoption of optimal practices. Training and capacity-building are needed to increase the number of well-trained professionals, including physicians and allied health professionals, such as nurses, psychologists, and educators, as well as physical, occupational, and intervention therapists.

In summary, it is clear that the insights we have gained have resulted directly from investments in scientific research on autism. However, without further and increased investment in science, our progress will be very slow. The IACC Strategic Plan for Autism Research has benefited greatly from input from the autism community, including private funding agencies. The IACC conducted a portfolio analysis of public and private autism research to enable future iterations of the Strategic Plan to build on existing research. We have seen how important this coordination and collaboration has been, and we must, as a community, continue to insist on it. The Strategic Plan was completed in January 2009, so it remains to be seen how closely it will be followed by the NIH with respect to research support. But I am hopeful that the collaboration between public and private funders of autism research will result in significant and innovative research that advances our understanding of the causes, prevention methods, treatments, and a cure for autism.

Next Steps

Mr. Chairman, the challenge of autism and its impact on our children, our families, and our nation are daunting, and the plans of Autism Speaks are equal to the task.

In the short term, we are focused on ensuring that Congress matches in its appropriations bills the authorized levels for HHS's autism research, surveillance, education and treatment set by the Combating Autism Act over the next two fiscal years. We are also planning for the reauthorization of the Combating Autism Act which is set to expire on September 30, 2011.

We have also been working on the next phase of the autism agenda—the provision of enhanced treatment, support, services, and research for individuals with ASD. That effort, which began with preliminary discussions with then Senator Barack Obama, has produced the Autism Treatment Acceleration Act of 2009, introduced in the Senate by Senator Richard Durbin (S. 819) and in the House by Congressman Mike Doyle (H.R. 2413). The ATAA includes a number of key authorizations, including Autism Care Centers and an Adult Services Demonstration, a population-based autism spectrum disorders registry, a National Network for ASD Research and Services, and most important, a requirement that health insurers cover diagnostic assessment and treatments for autism, including Applied Behavioral Analysis therapy. Of course, we are making every effort to see to it that this insurance component of the ATAA becomes law as a component of health care reform.

The Continuing Challenge

At the Senate Labor-HHS Subcommittee's first hearing on autism in April 2007, Autism Speaks Co-Founder Bob Wright issued a challenge to Congress and our nation, when he stated:

“The public health crisis posed by autism requires an extraordinary response. With every new child diagnosed with autism costing an estimated \$3 million over his or her lifetime, we cannot afford to rely on standard, ‘business as usual’ practices. The autism crisis demands a focused, coordinated, and accountable response by our public health agencies, similar to the federal response to the AIDS crisis in the 1990s, with line-item appropriations for autism intervention, surveillance and research tied to a strategic plan.”

Mr. Chairman, over the past two years, you and your subcommittee have met this challenge and responded to autism not with promises, but with resources to begin a meaningful fight against this public health crisis.

But more is needed to better understand the disorder, to better diagnose it, to better treat those children who have it, and ultimately to find cures for this spectrum of disorders. We do not need only to ramp up the NIH’s investment in autism research, we also need to ensure that every dollar spent on this disorder is targeted and maximally impactful.

Thank you, Mr. Chairman, for your time, for your commitment, and for your leadership.