

Select Interagency Autism Coordinating Committee (IACC) Strategic Plan Research Areas and Objectives

Strategic Plan Research Area and Objectives	Description of Objective
Research area 1: Diagnosis	
1. A (short-term)	Develop, with existing tools, at least one efficient diagnostic instrument (i.e., briefer, less time intensive) that is valid in diverse populations for use in large-scale studies by 2011.
1. B (short-term)	Validate and improve the sensitivity and specificity of new or existing screening and diagnostic tools, including comparative studies of general developmental screening versus autism-specific screening tools, in both high-risk and population-based samples, including those from resource-poor international settings and those that are diverse in terms of age, socio-economic status, race, ethnicity, gender, characteristics of autism, and general level of functioning by 2012.
1. C (short-term)	Conduct at least three studies to identify reasons for the health disparities in accessing early screening and diagnosis services, including identification of barriers to implementation of and access to screening, diagnosis, referral, and early intervention services among diverse populations, as defined by socioeconomic status, race, ethnicity, and gender of the child, by 2012.
1. E (short-term)	Conduct at least one study to determine the positive predictive value and clinical utility (e.g., prediction of co-occurring conditions, family planning) of chromosomal microarray genetic testing for detecting genetic diagnoses for autism in a clinical setting by 2012.
1. A (long-term)	Identify behavioral and biological markers that separately, or in combination, accurately identify, before age 2, one or more subtypes of children at risk for developing autism, and evaluate whether these risk markers or profiles can improve early identification through heightened developmental monitoring and screening by 2014.
1. B (long-term)	Develop at least five measures of behavioral and/or biological heterogeneity in children or adults with autism, beyond variation in intellectual disability, that clearly relate to etiology and risk, treatment response, and/or outcome by 2015.
1. C (long-term)	Identify and develop measures to assess at least three "continuous dimensions" (e.g., social reciprocity, communication disorders, and repetitive/restrictive behaviors) of autism symptoms and severity that can be used by practitioners and/or families to assess response to intervention for people with autism across the lifespan by 2016.
1. Other	Not specific to any objective within research area 1.
Research area 2: Biology	
2. A (short-term)	Support at least four research projects to identify mechanisms of fever, metabolic and/or immune system interactions with the central nervous system that may influence autism during prenatal-postnatal life by 2010.
2. B (short-term)	Launch three studies that specifically focus on the neurodevelopment of females with autism, spanning basic to clinical research on sex differences by 2011.
2. D (short-term)	Launch three studies that target improved understanding of the underlying biological pathways of genetic conditions related to autism (e.g., fragile X, Rett syndrome, tuberous sclerosis complex) and how these conditions inform risk assessment and individualized intervention by 2012.
2. E (short-term)	Launch three studies that target the underlying biological mechanisms of co-occurring conditions with autism, including seizures/epilepsy, sleep disorders, wandering/elopement behavior, and familial autoimmune disorders, by 2012.
2. F (short-term)	Launch two studies that focus on prospective characterization of children with reported regression to investigate potential risk factors by 2012.

2. G (short-term)	Support five studies that associate specific genotypes with functional or structural phenotypes, including behavioral and medical phenotypes (e.g., nonverbal individuals with autism and those with cognitive impairments) by 2015.
2. A (long-term)	Complete a large-scale, multidisciplinary, collaborative project that longitudinally and comprehensively examines how the biological, clinical, and developmental profiles of individuals, with a special emphasis on females, youths, and adults with autism, change over time as compared to typically developing people by 2020.
2. B (long-term)	Launch at least three studies that evaluate the applicability of autism phenotype and/or biological signature findings for performing diagnosis, risk assessment, or clinical intervention by 2015.
2. Other	Not specific to any objective within research area 2.
Research area 3: Causes	
3. A (short-term)	Coordinate and implement the inclusion of approximately 20,000 subjects for genome-wide association studies, as well as a sample of 1,200 for sequencing studies to examine more than 50 candidate genes by 2011. Studies should investigate factors contributing to phenotypic variation across individuals who share an identified genetic variant and stratify subjects according to behavioral, cognitive, and clinical features.
3. B (short-term)	Within the highest-priority categories of exposures for autism, identify and standardize at least three measures for identifying markers of environmental exposure in biospecimens by 2011.
3. C (short-term)	Initiate efforts to expand existing large case-control and other studies to enhance capabilities for targeted gene-environment research by 2011.
3. D (short-term)	Enhance existing case-control studies to enroll racially and ethnically diverse populations affected by autism by 2011.
3. E (short-term)	Support at least two studies to determine if there are subpopulations that are more susceptible to environmental exposures (e.g., immune challenges related to infections, vaccinations, or underlying autoimmune problems) by 2012.
3. F (short-term)	Initiate studies on at least 10 environmental factors identified in the recommendations from the 2007 Institute of Medicine report "Autism and the Environment: Challenges and Opportunities for Research" as potential causes of autism by 2012.
3. H (short-term)	Support at least three studies of special populations or use existing databases to inform our understanding of environmental risk factors for autism in pregnancy and the early postnatal period by 2012. Such studies could include: comparisons of populations differing in geography, gender, ethnic background, exposure history (e.g., prematurity, maternal infection, nutritional deficiencies, toxins), and migration patterns; and comparisons of phenotype (e.g., cytokine profiles), in children with and without a history of autistic regression, adverse events following immunization (such as fever and seizures), and mitochondrial impairment. These studies may also include comparisons of phenotype between children with regressive autism and their siblings. Emphasis on environmental factors that influence prenatal and early postnatal development is particularly of high priority. Epidemiological studies should pay special attention to include racially and ethnically diverse populations.
3. I (short-term)	Support at least two studies that examine potential differences in the microbiome of individuals with autism versus comparison groups by 2012.
3. J (short-term)	Support at least three studies that focus on the role of epigenetics in the etiology of autism, including studies that include assays to measure DNA methylations and histone modifications, and those exploring how exposures may act on maternal or paternal genomes via epigenetic mechanisms to alter gene expression, by 2012.
3. K (short-term)	Support two studies and a workshop that facilitate the development of vertebrate and invertebrate model systems for the exploration of environmental risks and their interaction with gender and genetic susceptibilities for autism by 2012.

3. A (long-term)	Conduct a multi-site study of the subsequent pregnancies of 1,000 women with a child with autism to assess the impact of environmental factors in a period most relevant to the progression of autism by 2014.
3. B (long-term)	Identify genetic risk factors in at least 50 percent of people with autism by 2014.
3. C (long-term)	Determine the effect of at least five environmental factors on the risk for subtypes of autism in the prenatal and early postnatal period of development by 2015.
3. D (long-term)	Support ancillary studies within one or more large-scale, population-based surveillance and epidemiological studies, including U.S. populations, to collect data on environmental factors during preconception, and during prenatal and early postnatal development, as well as genetic data, that could be pooled (as needed) to analyze targets for potential gene/environment interactions by 2015.
3. Other	Not specific to any objective within research area 3.
Research area 4: Treatments and interventions	
4. A (short-term)	Support at least three randomized controlled trials that address co-occurring medical conditions associated with autism by 2010.
4. B (short-term)	Standardize and validate at least 20 model systems (e.g., cellular and/or animal) that replicate features of autism and will allow identification of specific molecular targets or neural circuits amenable to existing or new interventions by 2012.
4. C (short-term)	Test safety and efficacy of at least five widely used interventions (e.g., nutrition, medications, assisted technologies, sensory integration, medical procedures) that have not been rigorously studied for use in autism by 2012.
4. D (short-term)	Complete two multi-site randomized controlled trials of comprehensive early intervention that address core symptoms, family functioning, and community involvement by 2013.
4. F (short-term)	Launch randomized controlled trials of interventions, including biological signatures and other measures to predict response, and monitor quality of life and functional outcomes in each of the following groups: Five trials in infants and toddlers by 2013. Three trials in school-aged children and/or adolescents by 2013. Three trials in adults by 2014.
4. G (short-term)	Support at least five studies on interventions for nonverbal individuals with autism by 2012. Such studies may include: projects examining service-provision models that enhance access to augmentative and alternative communication supports in both classroom and adult service-provision settings, such as residential service-provision and the impact of such access on quality of life, communication, and behavior; studies of novel treatment approaches that facilitate communication skills in individuals who are nonverbal, including the components of effective augmentative and alternative communication approaches for specific subpopulations of people with autism; and studies assessing access and use of augmentative and alternative communication for children and adults with autism who have limited or partially limited speech and the impact on functional outcomes and quality of life.
4. H (short-term)	Support at least two studies that focus on research on health promotion and prevention of secondary conditions in people with autism by 2012. Secondary conditions of interest include weight issues and obesity, injury, and co-occurring psychiatric and medical conditions.
4. A (long-term)	Complete at least three randomized controlled trials on medications targeting core symptoms in people with autism of all ages by 2014.
4. B (long-term)	Develop interventions for siblings of people with autism with the goal of reducing the risk of recurrence by at least 30 percent by 2014.
4. C (long-term)	Conduct at least one study to evaluate the safety and effectiveness of medications commonly used in the treatment of co-occurring conditions or specific behavioral issues in people with autism by 2015.

4. D (long-term)	Support at least five community-based studies that assess the effectiveness of interventions and services in broader community settings by 2015. Such studies may include comparative effectiveness research studies that assess the relative effectiveness of: different and/or combined medical, pharmacological, nutritional, behavioral, service-provision, and parent- or caregiver-implemented treatments; scalable early intervention programs for implementation in underserved, low-resource, and low-literacy populations; and studies of widely used community intervention models for which extensive published data are not available. Outcome measures should include assessment of potential harm as a result of autism treatments, as well as positive outcomes.
4. Other	Not specific to any objective within research area 4.

Research area 5: Services

5. A (short-term)	Support two studies that assess how variations in and access to services affect family functioning in diverse populations, including underserved populations, by 2012.
5. B (short-term)	Conduct one study to examine how self-directed community-based services and supports impact children, youth, and adults with autism across the spectrum by 2014.
5. C (short-term)	Implement and evaluate five models of policy and practice-level coordination among state and local agencies to provide integrated and comprehensive community-based supports and services that enhance access to services and supports, self-determination, economic self-sufficiency, and quality of life for people with autism across the spectrum and their families (which may include access to augmentative and alternative communication technology), with at least one project aimed at the needs of transitioning youth, and at least one study to evaluate a model of policy and practice-level coordination among state and local mental health agencies serving people with autism, by 2015.
5. A (long-term)	Test four methods to improve dissemination, implementation, and sustainability of evidence-based interventions, services, and supports in diverse community settings by 2013.
5. B (long-term)	Test the efficacy and cost-effectiveness of at least four evidence-based services and supports for people with autism across the spectrum and of all ages living in community settings by 2015.
5. C (long-term)	Evaluate new and existing pre-service and in-service training to increase skill levels in service providers, including direct support workers, parents and legal guardians, education staff, and public service workers, to benefit the spectrum of people with autism and to promote interdisciplinary practice by 2015.
5. D (long-term)	Evaluate at least two strategies or programs to increase the health and safety of people with autism that simultaneously consider principles of self-determination and personal autonomy by 2015.
5. E (long-term)	Support three studies of dental health issues for people with autism by 2015. This should include: one study on the cost-benefit of providing comprehensive dental services, including routine, non-emergency medical and surgical dental services, denture coverage, and sedation dentistry to adults with autism as compared to emergency and/or no treatment; one study focusing on the provision of accessible, person-centered, equitable, effective, safe, and efficient dental services to people with autism; one study evaluating pre-service and in-service training program to increase skill levels in oral health professionals to benefit people with autism and promote interdisciplinary practice.
5. Other	Not specific to any objective within research area 5.

Research area 6: Lifespan issues

6. A (short-term)	Launch at least two studies to assess and characterize variation in the quality of life for adults on the autism spectrum as it relates to characteristics of the service delivery system (e.g., safety, integrated employment, post-secondary educational opportunities, community inclusion, self-determination, relationships, and access to health services and community-based services), and determine best practices by 2012.
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6. B (short-term)	Evaluate at least one model, at the state and local level, in which existing programs to assist people with disabilities (e.g., Social Security Administration, Rehabilitation Services Administration) meet the needs of transitioning youth and adults with autism by 2013.
6. D (short-term)	Conduct at least one study to measure and improve the quality of lifelong supports being delivered in community settings to adults across the spectrum with autism through provision of specialized training for direct care staff, parents, and legal guardians, including assessment and development of autism-specific training, if necessary, by 2015.
6. A (long-term)	Develop at least two individualized community-based interventions that improve quality-of-life or health outcomes for the spectrum of adults with autism by 2015.
6. B (long-term)	Conduct one study that builds on carefully characterized cohorts of children and youth with autism to determine how interventions, services, and supports delivered during childhood impact adult health and quality of life outcomes by 2015.
6. C (long-term)	Conduct comparative effectiveness research that includes a cost-effectiveness component to examine community-based interventions, services, and supports to improve health outcomes and quality of life for adults on the autism spectrum over age 21 by 2018. Topics should include: community housing for people with autism; successful life transitions for people with autism, including from post-secondary education to adult services, employment, sibling relationships, and day programs; and meeting the service and support needs of older adults with autism.
6. Other	Not specific to any objective within research area 6.
Research area 7: Infrastructure and surveillance	
7. B	Conduct an annual "State of the States" assessment of existing state programs and supports for people and families living with autism by 2011.
7. C	Develop and have available to the research community a means by which to merge or link databases that allow for tracking the involvement of people in autism research by 2010.
7. D	Establish and maintain an international network of biobanks for the collection of brain tissue, fibroblasts for pluripotent stem cells, and other tissue or biological material, by acquisition sites that use standardized protocols for phenotyping, collection, and regulated distribution of limited samples by 2011. This includes support for post-processing of tissue, such as genotyping, RNA expression profiling, and MRI. Protocols should be put into place to expand the capacities of ongoing large-scale children's studies to collect and store additional biomaterials, including newborn bloodspots, promoting detection of biological signatures. Support should also be provided to develop an international web-based digital brain atlas that would provide high-resolution 3-D images and quantitative anatomical data from tissue of patients with autism and disease controls across the lifespan, which could serve as an online resource for quantitative morphological studies, by 2014.
7. E	Begin development of a web-based toolbox to assist researchers in effectively and responsibly disseminating their findings to the community, including people with autism, their families, and health practitioners, by 2011.
7. H	Create mechanisms to specifically support the contribution of data from 90 percent of newly initiated projects to the National Database for Autism Research, and link with other existing data resources by 2012.
7. I	Supplement existing Autism and Developmental Disabilities Monitoring Network sites to use population-based surveillance data to conduct at least five hypothesis-driven analyses evaluating factors that may contribute to changes in autism prevalence by 2012.
7. J	Develop the personnel and technical infrastructure to assist states, territories, and other countries that request assistance describing and investigating potential changes in the prevalence of autism and other developmental disabilities by 2013.

7. K	Encourage programs and funding mechanisms that expand the research workforce, enhance interdisciplinary research training, and recruit early-career scientists into the autism field by 2013.
7. L	Expand the number of Autism and Developmental Disabilities Monitoring sites in order to conduct autism surveillance in children and adults; conduct complementary direct screening to inform completeness of ongoing surveillance; and expand efforts to include autism subtypes by 2015.
7. N	Enhance networks of clinical research sites offering clinical care in real-world settings that can collect and coordinate standardized and comprehensive diagnostic, biological (e.g., DNA, plasma, fibroblasts, urine), medical, and treatment history data that would provide a platform for conducting comparative effectiveness research and clinical trials of novel autism treatments by 2012.
7. O	Create an information resource for autism researchers (e.g., PhenX Project) to share information to facilitate data sharing and standardization of methods across projects by 2013. This includes common protocols, instruments, designs, and other procedural documents, and should include updates on new technology and links to information on how to acquire and utilize technology in development. This can serve as a bidirectional information reference, with autism research driving the development of new resources and technologies, including new model systems, screening tools, and analytic techniques.
7. P	Provide resources to centers or facilities that develop promising vertebrate and invertebrate model systems, and make these models more easily available or expand the utility of current model systems, and support new approaches to develop high-throughput screening technologies to evaluate the validity of model systems by 2013.
7. Other	Not specific to any objective within research area 7.

Source: GAO analysis of information from the National Institute of Mental Health, Office of Autism Research Coordination. | GAO-15-583R

Notes: IACC considers the objectives in research area 7, infrastructure and surveillance, to be both short- and long-term objectives.

We previously reported that no federal funds were awarded to conduct research related to 12 strategic plan objectives from fiscal years 2008 through fiscal year 2012. These objectives are not included in the table.