



Autism Speaks 2012 Research Emphasis Areas

Autism Speaks supports global biomedical research into the diagnosis, causes, prevention, and treatment of autism or its disabling symptoms. Our mission is to improve the future for all who struggle with autism spectrum disorders. In support of that mission we provide funding along the entire research continuum -- from discovery to development to dissemination -- for innovative projects that hold considerable promise for significantly improving the lives of persons with autism.

Autism Speaks research funding will be restricted to projects that address one of the following priorities:

- Understand **environmental risk factors** and their interaction with genetic susceptibility to enable prevention and improve diagnosis and treatment
- Discover **biomarkers** that can improve risk assessment and subtype stratification that will allow for an individualized approach to treatment
- Improve quality of life through **more effective medicines, behavioral interventions, and technologies**
- Enhance diagnosis and treatment of underserved and under-studied populations, specifically,
 - **Nonverbal** persons with ASD
 - **Ethnically-diverse** and/or low resource communities
 - **Adults**
 - Those with **medical co-morbidities**
- **Disseminate and implement evidence-based clinical practices** to the broader community worldwide

Examples of types of projects that would be relevant include, but are not limited to, the following:

Risk factors

- Identification of environmental risk factors for ASD, especially those utilizing novel approaches or analytic methods.
- Elucidation of gene-environment interactions relevant to risk for ASD, including animal models with direct relevance to ASD.
- Investigations into epigenetic mechanisms of gene expression that may be influenced by environmental exposures.
- Description and/or characterization of genetic and environmental influences on biological pathways affected in ASD.
- Identification of biomarkers that index increased vulnerability to specific environmental exposures or classes of exposures.

Early Detection

- Identification of early indices of risk for ASD that can be assessed before 6 months of postnatal age
- Novel biomarkers that could aid in early detection
- Preclinical markers of early risk for autism
- Development and evaluation of new tools or instruments to be used in early identification

Adults with ASD

- Longitudinal course of adult development and aging
- Development and testing of novel interventions, supports, and treatment methods
- Elucidation of factors that relate to variation in outcome
- Medical conditions and co-morbidities associated with adulthood in ASD
- Factors related to increased mortality.

Nonverbal Persons with ASD

- Development of measures to more efficiently identify common comorbid medical and psychiatric conditions that can contribute to the distress experienced by nonverbal persons with ASD
- Development of innovative methods or devices that can assist in minimizing pain and distress experienced by nonverbal persons with ASD when undergoing medical procedures (e.g. phlebotomy, neuroimaging)
- Development of methods and devices that can facilitate the communicative abilities of nonverbal persons with ASD

Molecular pathophysiology and translational research

- Elucidation of molecular mechanisms and biochemical pathways, including pathways focused on synaptic mechanisms, inflammatory processes, metabolism, and neuropeptides that have relevance for translational research
- Discovery of diagnostic biomarkers of autism, especially ones that can index biological subtypes
- Development of model systems and bioassays, especially high throughput ones, that can be used to test novel methods for normalizing pathophysiology
- Proof of concept studies in animal model systems, including identification of small molecules, peptides, proteins, and/or antibodies that hold potential for modifying core symptoms of ASD
- Use of induced pluripotent stem cells to advance understanding of abnormalities in autism, including elucidation of biomarkers, biological mechanisms, environmental sensitivities, or response to novel compounds
- Animal model studies exploring the impact of treatments on biological endpoints and the elucidation of surrogate endpoints for treatment studies

Novel treatment approaches and technologies throughout the lifespan

- Development and testing of novel treatments aimed at preventing or modifying core symptoms of autism, or associated medical conditions throughout the lifespan
- Development and testing of novel treatments of medical conditions associated with autism, including but not limited to sleep disorders, GI conditions, seizures, metabolic conditions, and psychiatric co-morbidities
- Novel treatments for individuals with ASD who are more severely affected, regressed, and have responded slowly to behavioral/psychosocial treatment approaches, including individuals with ASD who are nonverbal
- Randomized clinical trials (RCTs) that include biomarkers of therapeutic efficacy or for tailoring therapy
- RCTs that include biological or surrogate endpoints (e.g., electrophysiological, structural)
- RCTs that combine pharmacological and behavioral interventions to improve efficacy for improving core or associated symptoms of ASD
- Development and validation of sensitive and specific outcome measures that assess change in the core features of autism and that can be used in clinical trials
- Novel technologies that provide for the efficient assessment of biomarkers and physiological indices of treatment response in clinical trials
- Novel technologies that can be used to efficiently track treatment-related changes in target symptoms across a variety of naturalistic settings
- Novel technologies that can accelerate the efficacy of other treatment modalities that impact biological, psychological, educational, occupational and/or social functioning
- Novel technologies that can improve the educational, occupational and social success of individuals with ASD

Dissemination of empirically-validated methods

- Development of novel methods that will allow wide access to diagnosis and treatment services, especially in underserved and low-resource communities
- Assessment of the effectiveness of feasible, exportable, and scalable screening, diagnosis, and early intervention programs
- Identification of factors that promote or impede the implementation of empirically-validated clinical programs in the community
- Training programs that can build capacity of the community to provide interventions