

Autism Research Program

Strategic Plan

INTRODUCTION

The Congressionally Directed Medical Research Programs (CDMRP) represents a unique partnership among the U.S. Congress, the military, and the public to fund innovative and impactful medical research in targeted program areas. Programs managed by the CDMRP have formalized strategic plans that identify program-specific research priorities; how to best address these urgencies; short- and long-term goals; investment strategies; and ways to identify and evaluate program successes with respect to the priorities.

The Autism Research Program (ARP) Strategic Plan identifies the high-impact research goals most important to its stakeholders while providing a framework that is adaptable to changes in the medical research environment to address those goals. This plan has been formulated to provide greater clarity of the program's goals over time. Congress appropriates funding for the ARP on an annual basis; there is no guarantee of future funding. The ARP Programmatic Panel members will review the Strategic Plan during the program's annual Vision Setting meeting and update it as necessary.

ARP BACKGROUND AND OVERVIEW

The ARP was initiated in 2007 to support innovative and impactful research that addresses fundamental issues and gaps in autism research. The ARP provides support for research of exceptional scientific merit and innovation with high impact that is focused on autism spectrum disorders (ASD).

Through the recommendations of the ARP Programmatic Panel, the ARP has developed the following vision and mission:

VISION: Improve the lives of individuals with autism spectrum disorder now

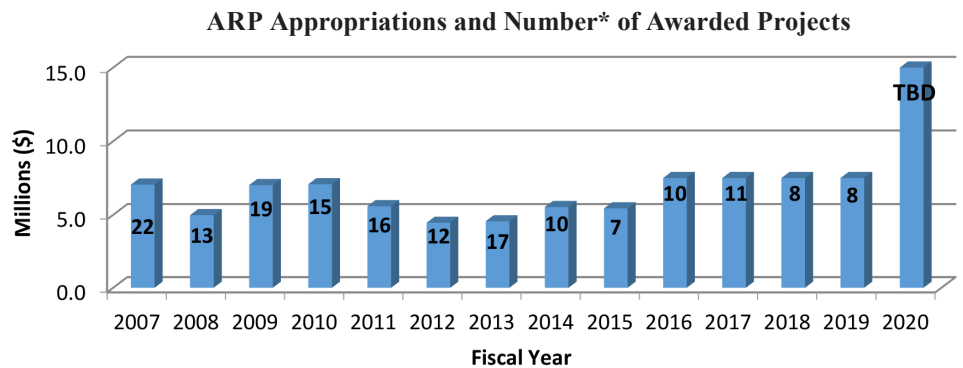
MISSION: Promote innovative research that advances understanding of autism spectrum disorder and leads to improved outcomes for Service Members, their families, and the American public

A distinct feature of the ARP is the consumer-informed approach to understanding the gaps in ASD research and recommending investments based on these gaps. The ARP Programmatic Panel includes ASD advocates who have greatly contributed to the success and impact of the ARP by providing unique perspectives on the needs of individuals and families who have a member diagnosed with ASD.



FUNDING HISTORY AND NUMBER OF AWARDS

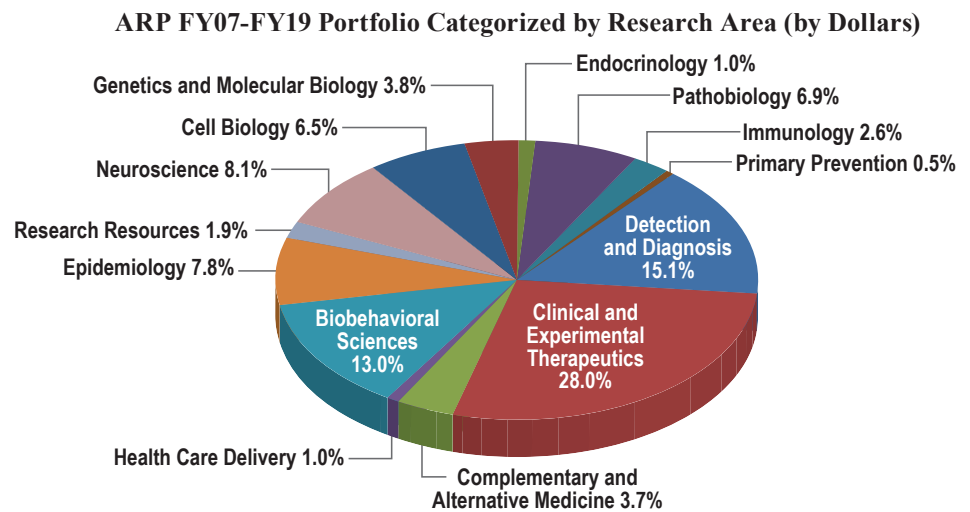
The ARP has received \$104.4 million in appropriations since the program's inception in 2007. During this time, the program has funded a total of 168 projects.



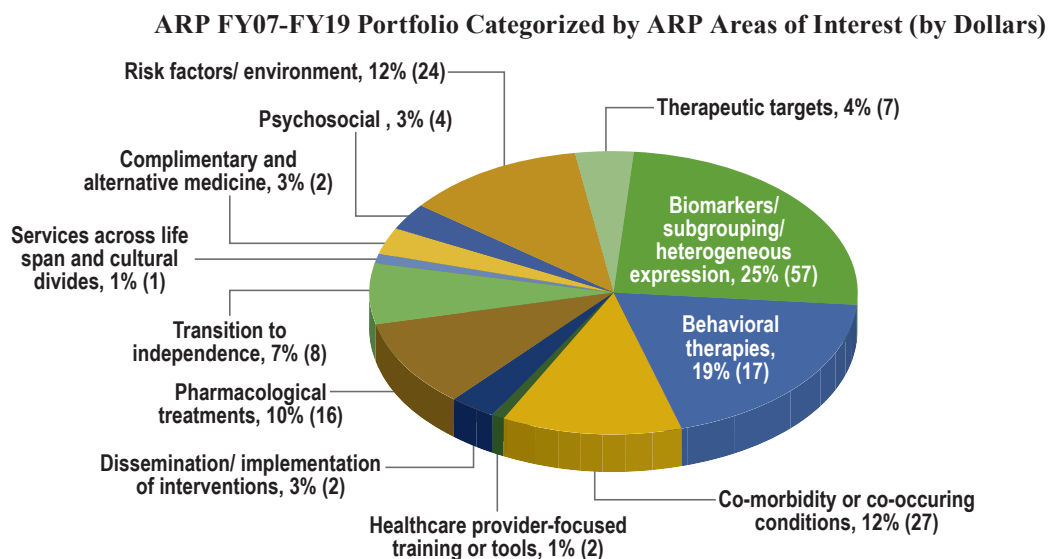
* The number of awarded projects is to be determined for fiscal year 2020 (FY20), pending final negotiations.

RESEARCH PORTFOLIO AND ACCOMPLISHMENTS

The ARP has funded projects that address the critical needs of the ASD community across a multitude of research areas, as noted in the figure to the right. Specific award information and abstracts of ARB-funded projects can be seen on the CDMRP website (<http://cdmrp.army.mil>).



With each congressional appropriation, the ARP identifies and encourages particular Areas of Interest based upon the needs of the ASD community. The figure to the right presents the ARP portfolio from fiscal years 2007-2016 (FY07-FY16), categorized into these Areas of Interest.



Autism Research Program

For reference, the ARP's Areas of Interest through the years of the program are summarized in the table below.

	FY07-09	FY10-12	FY13-16	FY17-18	FY19	FY20	FY21
Clinical resource development	✓	✓					
Co-morbidity or co-occurring conditions	✓	✓	✓	✓	✓	✓	✓
Identification and/or validation of therapeutic targets	✓	✓*	✓**	✓**	✓**	✓**	✓**
Biomarkers, subgrouping and mechanisms of heterogeneous expression and response to treatment	✓	✓	✓	✓	✓	✓	✓
Risk factors/environment	✓	✓	✓	✓	✓	✓	✓
Pharmacological treatments/interventions		✓	✓	✓	✓	✓	✓
Psychosocial research and/or interventions		✓					
Complementary and alternative medicine		✓					
Behavioral and/or other non-pharmacological therapies/interventions		✓	✓	✓	✓	✓	✓
Dissemination/implementation of interventions			✓	✓	✓	✓	✓
Key transitions to independence			✓	✓	✓	✓	✓
Healthcare provider-focused training or tools				✓	✓	✓	✓
Diagnosis and access to services across life span and cultural divides				✓	✓	✓	✓
Factors influencing quality of life during geographic relocation					✓	✓	✓
Mechanisms underlying sex differences						✓	✓
Pragmatic Trials							✓
Create tools/strategies to increase the speed with which evidence-based practices are deployed in community							✓

*Excluding gene discovery

**In preclinical models

ARP SUPPORTED CLINICAL STUDIES

- Cognitive Enhancement Therapy (CET).** CET has been proven successful in helping people with schizophrenia improve cognitive development and social functioning. An ARP-funded Clinical Trial Award evaluated the efficacy of CET on cognitive and behavioral deficits in adults with ASD, as there are a limited number of interventions aimed at adults with ASD. Following 18 months of treatment, CET was found to produce more substantial improvements in social and non-social cognition when compared to another commonly used behavior intervention. Furthermore, the adults receiving CET were more likely to be employed at the end of treatment compared to those receiving the other intervention. As this is the first empirically validated treatment to address core deficits in adults with ASD, this study will lay the groundwork for forthcoming efforts in third-party reimbursement and broad dissemination of the intervention.
- ImPact Online Program.** Improving Parents as Communication Teachers (ImPact) is an online, parent-based intervention for families of young children with ASD. This intervention, which was funded through an ARP Idea Award, is a combination of developmental and behavioral intervention strategies provided by the parent during daily routines and activities. Results showed that Internet-based instruction is a feasible method for training parents of children with ASD in evidence-based intervention strategies. This type of online training could allow greater dissemination of evidence-based practices to underserved populations. The ImPact program is currently open to the public (<http://psychology.msu.edu/autismmlab/projectimpact.html>) and has received follow-on funding from other organizations to further examine feasibility, treatment adherence, and efficacy.
- Tailored Behavioral Intervention for ASD Children with Insomnia.** An ARP-funded Pilot Award studied utilization of a tailored behavioral intervention (TAB) for ASD children with insomnia, a common co-occurring condition observed in those



with ASD. The TAB included positive routines, a calming module, and a bedtime protocol that supplemented the standard of care. Findings showed that the TAB significantly increased sleep time after only 8 weeks of training. The goal of this project is to include TAB in the Autism Treatment Network Sleep tool kit for broad dissemination to the ASD community.

RESEARCH AND FUNDING ENVIRONMENT

STATE OF THE SCIENCE

The Centers for Disease Control and Prevention (CDC) identifies the prevalence of ASD in the United States as approximately 1 in 59¹ children, and the population of adults with ASD is growing. The cause of ASD is unknown. ASD is often not diagnosed until the individual is between the ages of 4 to 5 years, which is unacceptable because years of potential early intervention therapy have been lost. Those impacted by ASD include members of the U.S. Armed Forces and their family members. The Department of Defense reports that, in 2016, the total number of active duty Service Members and their family members diagnosed with ASD was 28,206.

Many difficulties and concerns are faced by the ASD community. Individuals with ASD experience social, communication, and behavioral challenges, which can vary from mild to severe. There is lack of support and services for adults with ASD that would enable them to thrive and have successful futures. The ARP recognizes these gaps and considers any improvements and new needs for the ASD community on an annual basis.

RESEARCH FUNDING LANDSCAPE

Funding for autism research comes from a variety of sources through many programs. Autism research is funded by federal agencies, including the National Institutes of Health (NIH), CDMRP, Environmental Protection Agency, and CDC, as well as private foundations. To help coordinate the ARP investment strategy, the ARP Programmatic Panel has representation from other federal ASD funding agencies, such as the NIH and CDC, as well as from ASD advocates from private funding organizations. As the ARP develops its own research program focus and portfolio, it remains mindful of the research efforts of other funding organizations and private foundations such as Autism Speaks, the Autism Science Foundation, and the Simons Foundation. Of particular relevance is the work of the Interagency Autism Coordinating Council (<https://iacc.hhs.gov/>), a federal advisory committee charged with coordinating federal activities related to ASD. Understanding the areas of focus and investment priorities of other autism research funding sources allows the ARP to concentrate its own resources where they are most needed in order to address major knowledge gaps.

Today's medical research environment is dynamic. New research datasets are being created and made available to researchers at an ever faster rate. New technologies are emerging that will enable research in the future that is impossible today. The ARP continually monitors new technological advances that may provide a better understanding of ASD, its diagnosis and subtypes, and the most effective interventions for helping individuals with autism. Given the rapid and prolific pace of new technology introduction, it is not possible to list all pertinent technologies here, nor can we fully anticipate the technology progress that may be achieved over the 5-year planning horizon for this Strategic Plan.

In developing the ARP Strategic Plan, the Programmatic Panel members reviewed the current research and funding environment for ASD and considered the existing research portfolios and emerging technologies that offer the potential to transform understanding, diagnosis, and treatment of ASD. A core component of the ARP's strategic planning efforts is an ongoing evaluation of the external environment to identify and leverage new technology developments, as well as advances in the research field. The ARP must fit within this environment and effectively respond to its changes to maximize the value and impact of ARP-funded research.

STRATEGIC DIRECTION

Based upon the current state of ASD research, the funding landscape provided by other federal and private organizations, and the needs of the ASD community, the ARP developed its overall strategic direction, which contains four key elements:

- Fund high-impact research to address major knowledge gaps
- Invest in projects with potential for immediate implementation
- Invest in projects with potential for broad dissemination
- Focus on novel ideas that fulfill the needs of the ASD community and maintain high scientific rigor
- Support the development of an early stage diverse investigator cohort for autism research

This strategic direction of the ARP will positively impact both Service Members and the general public. Military families faced with the challenges of ASD will benefit from investments in these areas of ASD research.



NEAR- TO MEDIUM-TERM STRATEGY

Strategic Goals

The ARP has identified four near- to medium-term (5-7 years) goals to achieve the strategic direction noted above. Within these strategic goals, the panel identified Areas of Interest that, while not required, act as an indicator to the research community of the types of applications that may be of special interest to the program.

1. Understand Causes, Mechanisms, and Signs of ASD – Areas of Interest

- Environmental risk factors for ASD
- Mechanisms of heterogeneous clinical expression of ASD
- Mechanisms underlying conditions co-occurring with ASD (including, but not limited to, sleep disturbances, gastrointestinal issues, inflammation, aggression, depression, anxiety, attention deficit, seizures)
- Improved diagnosis of autism across the life span

2. Advance Effective Treatments and Interventions for Autism – Areas of Interest

- Pharmacological, genetic, and other biological treatments in ASD
- Behavioral, cognitive, and other non-pharmacological therapies
- Therapies to alleviate conditions co-occurring with ASD
- Assessment of novel therapeutics using valid preclinical models
- Dissemination/implementation of clinically validated interventions
- Cultural and socioeconomic factors in treatment efficacy, delivery, and access to services
- Tests of implementation strategies to increase use of evidence-based practices
- Understanding heterogeneity in treatment response
- Long-term treatment outcomes from previous clinical trials for ASD core systems or to alleviate co-occurring conditions

3. Address Needs of Persons with Autism into Adulthood – Areas of Interest

- Interventions promoting success in key transitions to adulthood
- Factors promoting success in key transitions to independence

4. Support Those Caring for the Autism Community – Area of Interest

- Healthcare provider-focused training or tools to improve healthcare delivery for individuals with ASD, across the life span and the continuum of care (i.e., primary care, urgent/emergent care, and disaster relief)
- Factors impacting quality of life during geographic relocation, such as military permanent change of station

INVESTMENT STRATEGY

NEAR- TO MEDIUM-TERM

Over the next 5 to 7 years, the ARP will consider projects across the research continuum, including preclinical, translational, and clinical research awards. The program will invest the congressional appropriations in the following award mechanisms in order to meet the strategic direction outlined previously:

Preclinical Research – Idea Development Award

- Support high-risk/high-reward ideas that will accelerate progress in improving outcomes for individuals with ASD.
- Support innovative ideas with the potential to yield impactful data and new avenues of investigation.

Translational Research – Clinical Translational Research Award

- Support early-phase, proof-of-principle translational studies that will examine hypothesis-based, innovative interventions that have the potential to address current clinical deficits for ASD.
- Outcomes should provide scientific rationale for subsequent development of larger clinical trials of interventions that will transform ASD clinical care.



Clinical Research – Clinical Trial Award

- Support trials with the potential to have a major impact on the treatment or management of ASD.

Career Development – Career Development Award

- Supports early-career, independent investigators and/or the transition of established investigators from other research fields to conduct innovative, high-impact ideas or early-phase, proof-of-principle clinical trials with the potential to have a major impact on ASD.

The ARP will remain open to a wide range of award mechanisms to support both its strategic goals and its interest in funding projects of strong scientific merit across the research continuum. This investment strategy will be re-evaluated and updated as necessary during the program's annual Vision Setting meeting.

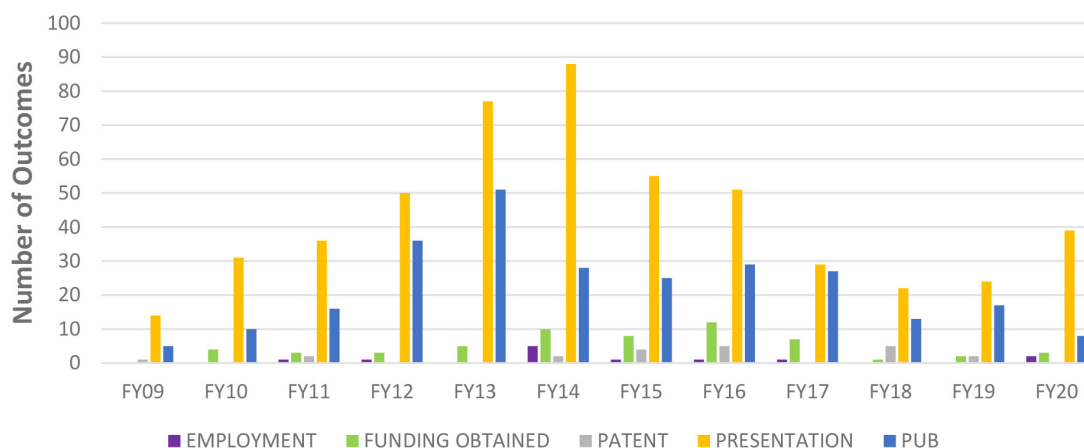
MEASURING PROGRESS

The ARP will measure its near-term success based on successful investments in the research areas that are important to fulfilling its strategic direction. Longer-term success will be evaluated based on contributions to the scientific community, follow-on research that is attributed to ARP-funded projects, and the impact of ARP-funded research on clinical treatments and interventions.

CURRENT OUTCOMES

The figure below shows the outcomes for the ARP that have been recorded from FY09-FY20.

Autism Research Program Outcomes Per Year*



SHORT-TERM OUTCOMES

- Application and funding activity
 - Quality and quantity of applications received in response to the Areas of Interest
- Contributions to the scientific community
 - Publications
 - Presentations
 - Patent applications and patents

LONG-TERM OUTCOMES

- Contributions to the scientific community
 - Publications
 - Presentations
 - Patent applications and patents
- Career advancement of scientists/researchers
- Receipt of subsequent funding by ARP-funded investigators
- Clinical impact on the ASD community

REFERENCES

1. Baio J, Wiggins L, Christensen DL, et al. 2014. Prevalence of Autism Spectrum Disorder Among Children Aged 8 Years — Autism and Developmental Disabilities Monitoring Network, 11 Sites, United States, *MMWR Surveill Summ* 2018; 67(No. SS-6):1–23. (<http://dx.doi.org/10.15585/mmwr.ss6706a1>).