CDMRP Department of Defense

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. In 2015, an ad hoc committee of the National Academies of Sciences, Engineering, and Medicine was assembled to evaluate the CDMRP's two-tier review process and its coordination of research priorities with the National Institutes of Health (NIH) and the Department of Veterans Affairs (VA). As part of their final report,[1] the committee recommended that each CDMRP program "… develop a strategic plan that identifies and evaluates research foci, benchmarks for success, and investment opportunities for 3–5 years into the future," and that these strategic plans "should specify the mission of the program, coordination activities with other organizations, research priorities, how those priorities will be addressed by future award mechanisms, how research outcomes will be tracked, and how outcomes will inform future research initiatives."

In response to these recommendations, this document presents the current strategy for the CDMRP's Autism Research Program (ARP). The 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 to the public and other stakeholders. Funding for the ARP is Congressionally appropriated on an annual basis; therefore, there is no guarantee of future funding. The ARP Strategic Plan will be reviewed during the program's annual Vision Setting meeting and updated 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

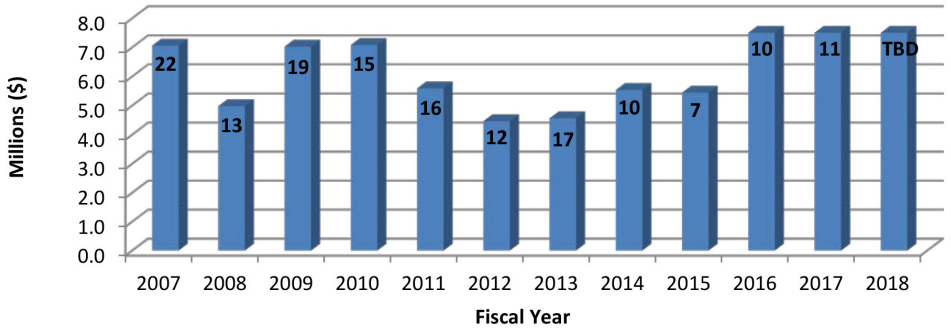
MISSION: Promote innovative research that advances the understanding of autism spectrum disorder and leads to improved outcomes for Service members, their families, and the American public

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 ASD advocates on the ARP Programmatic Panel provide unique perspectives on the needs of individuals and families with a member diagnosed with ASD that have greatly contributed to the success and impact of the ARP.

FUNDING HISTORY AND NUMBER OF AWARDS

The ARP has received $81.9 million in appropriations since the program's inception in 2007. During this time, the program has funded a total of 152 projects.

ARP Appropriations and Number* of Awarded Projects

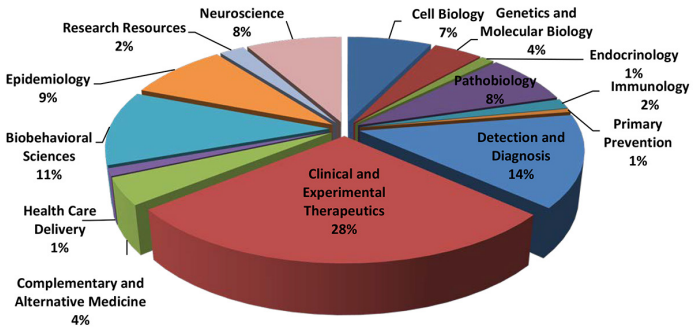


*The number of awarded projects is anticipated for fiscal year 2017 (FY17), pending final negotiations, and is to be determined for FY18.

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).

ARP FY07-FY16 Portfolio Categorized by Research Area (by Dollars)

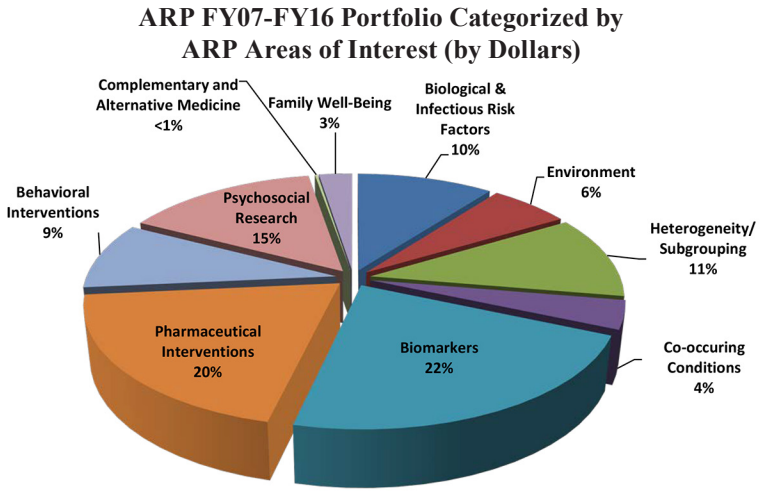


For reference, the ARP's Areas of Interest through the years of the program are summarized in the table below.

	FY07	FY08	FY09	FY10	FY11	FY12	FY13	FY14	FY15	FY16
Comorbidity or co-occurring conditions	✓	✓	✓		✓	✓	✓	✓	✓	✓
Subgrouping and mechanisms of heterogeneous clinical expression						✓	✓	✓	✓	✓
Identification and/or validation of therapeutic targets	✓	✓		✓	✓*	✓*	✓*	✓**	✓**	✓**
Biomarkers and/or treatment predictors	✓	✓	✓		✓	✓	✓	✓	✓	✓
Environment	✓	✓		✓				✓	✓	✓
Clinical resource development	✓				✓					
Pharmacological treatments/interventions				✓	✓			✓	✓	✓
Psychosocial research and/or interventions				✓		✓	✓	✓		
Complementary and alternative medicine				✓	✓					
Behavioral and/or other non-pharmacological therapies/interventions					✓			✓	✓	✓
Dissemination/implementation of behavioral interventions								✓	✓	✓
Encouraged multidisciplinary collaborations					✓					
Family/caregiver well-being							✓			
Key transitions to independence									✓	✓

*Excluding gene discovery
**In preclinical models

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.



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/autismlab/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 59[2] 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 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

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

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)

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 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

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.

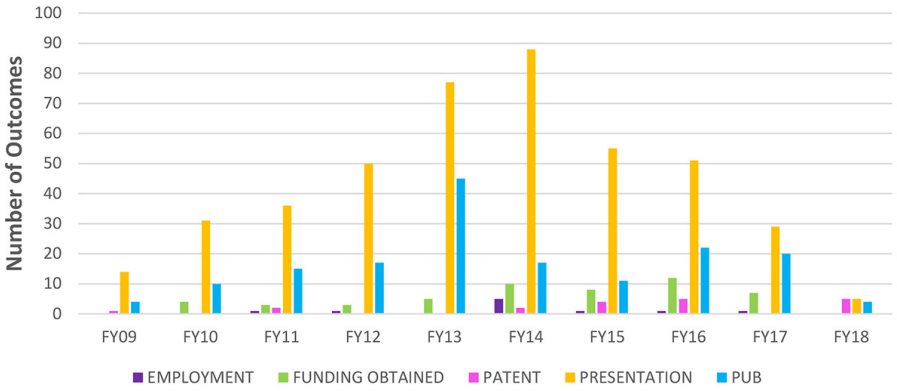
MEASURING PROGRESS

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-FY18.

Autism Research Program Outcomes Per Year*



*FY17 is anticipated, and FY18 is to be determined.

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. *Evaluation of the Congressionally Directed Medical Research Programs Review Process, a Report of the National Academies of Sciences, Engineering, and Medicine*. 2016. The National Academies Press, Washington, DC. (http://nationalacademies.org/hmd/reports/2016/cdmrp.aspx).
2. Baio J, Wiggins L, Christensen DL, et al. Prevalence of Autism Spectrum Disorder Among Children Aged 8 Years — Autism and Developmental Disabilities Monitoring Network, 11 Sites, United States, 2014. *MMWR Surveill Summ* 2018; 67(No. SS-6):1–23. (http://dx.doi.org/10.15585/mmwr.ss6706a1).