ADVOCACY ASKS

NATIONAL FRAGILE X FOUNDATION

Support Fragile X research funding for Fiscal Year 2025.

Between the NIH, the CDC, and the DOD, the Federal government invests over $50M/year in Fragile X research. This varies per year, depending on the research proposals funded following the peer review process. Additionally, the CDC's Fragile X program supports awareness among professionals, published consensus guidelines, and the FORWARD project, which collects data on individuals with Fragile X across the lifespan and makes it available to researchers. You can support this in three ways:

- **Senate: Request that Fragile X be included as an authorized research area for the DOD's Peer Reviewed Medical Research Program (PRMRP).** The Appropriations Committee authorizes the medical conditions, disorders, and diseases the program may research through report language each year. Which research is funded is decided by a peer review process.

  Senator Stabenow is leading a letter. Contact Alexandra Diggs at alexandra_diggs@stabenow.senate.gov with questions or to cosign.

- **House: Request support for Fragile X at the NIH and the CDC.** The CDC has an annual line item in their budget request for $2M for Fragile X, and the NIH funds projects as they are peer-reviewed and, additionally, funds three Fragile X research centers.

  Rep. Joe Courtney (maria.costigan@mail.house.gov) and Rep. Chris Smith (John.McDonough@mail.house.gov) are leading a letter. Contact either staff member for the latest draft and cosign.

SSI Savings Penalty Elimination Act (S.2767 /H.R.5408)

This bill aims to address the regressive, anti-savings asset limits for the Supplemental Security Income (SSI) program. Specifically, the bill would increase the asset limits that were established in 1984 from $2,000 to $10,000 for individuals and from $3,000 to $20,000 for married couples. We ask that you support this proposed legislation that will empower the most financially vulnerable Americans to take financially responsible steps to prepare for the future without worrying they will reduce or lose benefits.

Accelerating Kids' Access to Care Act (H.R.4758 /S.2372)

Helping reduce the time it currently takes children covered by Medicaid or Children's Health Insurance Program (CHIP) to access specialized care when providers in their home state cannot address their needs. We urge you to support this legislation to ensure delays are eliminated to improve better outcomes. Every child deserves the best care – regardless of who they are, where they live, or their family's income.

Join the Congressional Fragile X Caucus (House only).

Contact Maria Costigan (maria.costigan@mail.house.gov) in Rep. Joe Courtney's office or **John McDonough (John.McDonough@mail.house.gov)** in Rep. Chris Smith's office.
The Caucus does not require any financial commitment or agreeing to any policy positions.

FEDERAL INVESTMENTS IN FRAGILE X-ASSOCIATED CONDITIONS

NATIONAL FRAGILE X FOUNDATION

We are incredibly thankful for all the federal government has done to support research in Fragile X- associated conditions! Without this investment, we would not be able to make strides toward effective treatments for Fragile X. Our advocates have been able to secure over $550M in research funding over the past 20 years. These are just some of the amazing things federal investments are doing.

CENTERS FOR DISEASE CONTROL AND PREVENTION (CDC)

- The CDC funds the Fragile X Registry With Accessible Research Database (FORWARD), the largest resource of health, clinical, and social support information on people with Fragile X syndrome (FXS) in the United States.
- FORWARD includes over 10 years of longitudinal data accessible to researchers. 26 publications have been generated from FORWARD data with more to be released soon. Recommendations from many of these publications have been important for informing health management, identifying risk groups, gaps in care, and needs for intervention to improve the quality of life for individuals with FXS.

NATIONAL INSTITUTES OF HEALTH (NIH)

- The NIH has $64M in active awards specific to *FMR1*-related conditions (104 Unique Researchers).
- The NIH updated its Strategic Plan for Research on *FMR1*-Associated Conditions in 2019. This plan shared goals for specific *FMR1*-associated conditions, including cross-disciplinarity goals that encourage innovative, impactful research in Fragile X.
- NIH supports The Centers for Collaborative Research in Fragile X Program, with the common goal "of facilitating the translation of basic research findings from bench to bedside and bedside to community." This grant supports research to improve the diagnosis and treatment of Fragile X syndrome (FXS) and its related conditions, aiding premier FX Centers in increasing their impact in line with the NIH Strategic Plan for Research on *FMR1*-Associated Conditions. The current awardees are Emory University, Baylor College of Medicine, and Cincinnati Children's Hospital Medical Center.
- NIH funds multiple Career Development (K) awards, some of which have gone to up-and-coming professionals who are completing innovative projects in Fragile X. This pipeline of and for new researchers is critical to advancements for Fragile X.

DEPARTMENT OF DEFENSE (DOD)

- Congress continues to recognize Fragile X as an authorized medical research area.
- The DOD Peer Reviewed Research Program (PRMRP) has funded $32M in research projects related to Fragile X syndrome.
- $11.4 million award for the first clinical trial for people living with Fragile X-associated tremor/ataxia syndrome (FXTAS)

Final

United States Senate

WASHINGTON, DC 20510

April 11, 2023

The Honorable Jon Tester
Chairman
Defense Appropriations Subcommittee
311 Hart Senate Office Building
Washington, DC 20510

The Honorable Susan Collins
Ranking Member
Defense Appropriations Subcommittee
413 Dirksen Senate Office Building
Washington, DC 20510

Dear Chairman Tester and Ranking Member Collins:

As Senators committed to improving the health of children and adults living with intellectual disabilities in the United States, we respectfully request your continued commitment to sustaining federal investments in biomedical research focused on the treatment and cure of Fragile X syndrome and its related conditions.

Mutations of the Fragile X gene result in behavioral, developmental, cognitive, reproductive, and potentially life-ending neurodegenerative conditions across generations in families and impact affected individuals from cradle to grave. Fragile X syndrome and associated disorders result from a single-gene mutation, which is the most common, known inherited cause of intellectual disabilities and autism. In fact, research has shown that the Fragile X protein regulates nearly one half of the genes suspected of causing autism. Up to 100,000 Americans have Fragile X syndrome, and up to 1,500,000 Americans have a variation of the Fragile X mutation and as a result either have, or are at risk for developing, one of the conditions associated with Fragile X and passing the gene mutation to their children. The known premutation issues are Fragile X-associated tremor/ataxia syndrome, a condition similar to Parkinson's, and Fragile X-associated primary ovarian insufficiency, which causes infertility and early menopause.

The Committee's previous support of Fragile X as one of the research areas authorized for the DoD's Peer Reviewed Medical Research Program funded some important research and has the potential to ease the burden of Fragile X and other intellectual and developmental disabilities on our military families. Military families are affected substantially by the financial and emotional costs of raising a child with intellectual and developmental disabilities, including Fragile X syndrome. This impact extends to the performance and readiness of service members and their units. Strides are being made towards effective treatments for Fragile X syndrome and other associated disorders while moving towards a cure. These treatments will help ease the burden on military families.

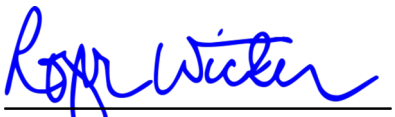
We are requesting that Fragile X be included as an authorized research area for the DoD's Peer Reviewed Medical Research Program for Fiscal Year 2024. While we understand the challenges

the Committee faces in prioritizing requests, Fragile X has a significant impact on military families across generations in every state and district. The potential for effective treatments is within reach. We believe continued support for Fragile X research is imperative. The DoD's research has been a significant contributor over the past decade, and we hope it will continue be in the future.

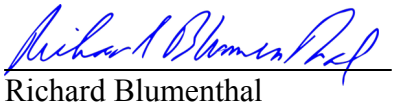
We look forward to working with the Subcommittee on this important issue. Thank you for your consideration.

Sincerely,

Debbie Stabenow
United States Senator



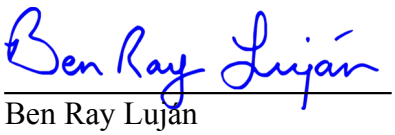
Roger F. Wicker
United States Senator



Richard Blumenthal
United States Senator

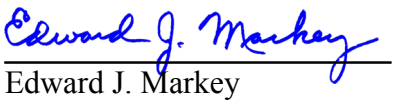
Kirsten Gillibrand
United States Senator

Gary C. Peters
United States Senator



Ben Ray Luján
United States Senator

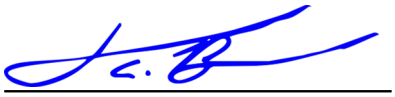
Sherrod Brown
United States Senator



Edward J. Markey
United States Senator

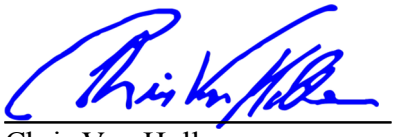
Jack Reed
United States Senator

Kyrsten Sinema
United States Senator



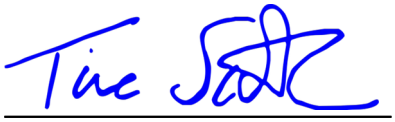
Cory A. Booker
United States Senator

Elizabeth Warren
United States Senator

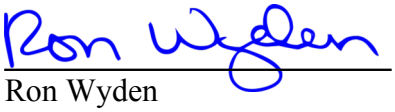


Chris Van Hollen
United States Senator

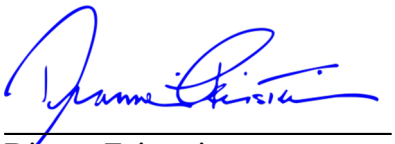
Alex Padilla
United States Senator



Tina Smith
United States Senator



Ron Wyden
United States Senator



Dianne Feinstein
United States Senator

Congress of the United States
Washington, DC 20515

Final

March 23, 2023

The Honorable Robert Aderholt
Chairman
Subcommittee on Labor,
Health and Human Services, Education
Committee on Appropriations
2358-B Rayburn House Office Building
Washington, DC 20515

The Honorable Rosa L. DeLauro
Ranking Member
Subcommittee on Labor,
Health and Human Services, Education
Committee on Appropriations
1036 Longworth House Office Building
Washington, DC 20515

Dear Chairman Aderholt and Ranking Member DeLauro,

As Members of Congress committed to improving the health of children and adults living with intellectual disabilities in the US, we respectfully request your continued commitment to sustaining federal investments in biomedical research and public health initiatives focused on the treatment and cure of Fragile X and its related conditions.

Mutations of the Fragile X (FX) gene result in behavioral, developmental, cognitive, reproductive, and potentially life-ending neurodegenerative conditions across generations and impact affected individuals from cradle to grave. Fragile X syndrome and associated conditions result from a single-gene mutation, which is the most common, known inherited cause of intellectual disabilities and autism. In fact, research has shown that the Fragile X protein regulates nearly one half of the genes suspected of causing autism. Up to 100,000 Americans have Fragile X syndrome, and up to 1,500,000 Americans have a variation of the Fragile X mutation and as a result either have, or are at risk for, developing one of the conditions associated with Fragile X.

The Committee's previous support for the important work underway at the National Institutes of Health (NIH) and Centers for Disease Control & Prevention (CDC) is advancing research towards more effective treatments and a cure for Fragile X. For instance, the CDC funds the FORWARD project, a natural history study. FORWARD is a longitudinal database that now includes 10.5 years of historical data of individuals with Fragile X and additional data on premutation carriers and other family members. This data gives researchers a view of Fragile X over the lifetime and across generations. These are resources that are not available anywhere else and are invaluable tools for educators and clinicians.

The NIH supports Fragile X research across multiple Institutes and Centers, with the primary one being the NICHD. This includes funding three national Fragile X research centers that focus on stimulating multi-disciplinary, multi-institutional research with a goal of translating basic research into treatments. We are seeing promising treatments for many of the behaviors associated with Fragile X syndrome – including several currently in various phases of clinical trials – and research that will also inform treatments and better understanding of autism. Fragile X is the most common, known, single gene cause of autism.

To ensure the rapid translation of ongoing research into near term targeted treatments, we must continue these federal investments in the Fiscal Year 2024 Labor, Health and

Human Services, and Education Appropriations bill. Specifically, we respectfully request your support for directives to:

- Expand the base of researchers and clinicians who are familiar with and trained in the Fragile X-associated disorders and promoting collaboration between basic scientists and clinicians to enable researchers to better understand phenotypes, document variations in how the disorder presents itself, identify potential biomarkers and outcome measures, and develop new interventions.

- Maintain dedicated support for CDC's national Fragile X public health program.

To this end, we respectfully request the inclusion of the following report language regarding Fragile X Syndrome:

CDC

Fragile X and Fragile X-Associated Disorders. -- The Committee commends CDC's efforts to identify and define the population impacted by Fragile X (FX) and all conditions associated with the gene mutation with the goal of understanding the public health impact of these conditions. To help this effort, the Committee urges the National Center on Birth Defects and Developmental Disabilities (NCBDDD) to support additional strategies to promote earlier identification of children with FX, such as voluntary newborn screening. The Committee also recommends the NCBDDD work to ensure underserved populations with FX conditions are being properly diagnosed and are aware of medical services available. Finally, the Committee recommends the NCBDDD support research across the lifespan of individuals living with Fragile X and the associated conditions and disorders.

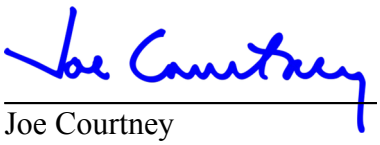
NIH

Regarding fragile X, the Committee notes the importance of expanding the base of researchers and clinicians who are familiar with and trained in the Fragile X-associated disorders and promoting collaboration between basic scientists and clinicians to enable researchers to better understand phenotypes, document variations in how the disorder presents itself, identify potential biomarkers and outcome measures, and develop new interventions. The Committee commends the NIH for recognizing the ethical, legal, and social issues in premutation screening and testing and encourages to NIH to look at existing pilot studies that are looking at innovative ways to screen newborns, study Fragile X across the lifespan, and to coordinate efforts and research with the CDC as they look at screening solutions for FMR1-related conditions.

While we understand the challenges the Committee faces in prioritizing requests, Fragile X has a significant impact on families across generations, on individuals throughout their lives, and on communities in every state and district. The potential for effective treatments is within reach, and continued support for Fragile X research and public health activities is imperative. We look forward to working with the Subcommittee on this important issue. Thank you for your time and consideration.

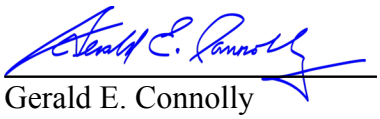
Sincerely,

Christopher H. Smith
Member of Congress



Joe Courtney
Member of Congress

Danny K. Davis
Member of Congress



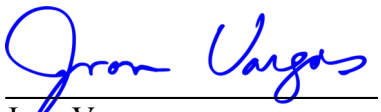
Gerald E. Connolly
Member of Congress

James P. McGovern
Member of Congress

Mark DeSaulnier
Member of Congress

Stephen F. Lynch
Member of Congress

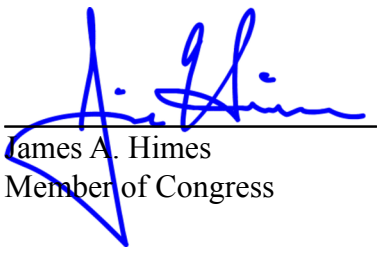
Bill Posey
Member of Congress



Juan Vargas
Member of Congress

Marcus J. Molinaro
Member of Congress

Darin LaHood
Member of Congress



James A. Himes
Member of Congress