NEUROFIBROMATOSIS RESEARCH PROGRAM

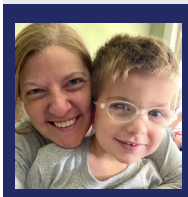
CDMRP
DEPARTMENT OF DEFENSE
CONGRESSIONALLY DIRECTED MEDICAL RESEARCH PROGRAMS

MISSION: Promote research directed toward the understanding, diagnosis and treatment of NF1, NF2 and schwannomatosis to enhance the quality of life for persons with these disorders that impact Service Members, Veterans and the general public

Congressional Appropriations

FY96-FY24:

$452.8M total



"Neurofibromatosis has had our family on a roller coaster ride for over 4 years. Our son Jay has had a variety of medical complications big and small. The one constant is an overwhelming uncertainty of what will come next. Having the opportunity to be a peer reviewer with the NFRP has helped to soothe some of the anxiety this uncertainty has given me as a mother. Seeing first-hand how hard medical professionals, researchers and scientist are working to find a cure and better treatments is truly encouraging"

Laura Haslam, Neurofibromatosis Midwest, FY21-FY23 Consumer Peer Reviewer

SCOPE OF THE PROBLEM[1]

Neurofibromatosis comprises **3** disorders caused by distinct genetic mutations

1. Neurofibromatosis 1 – Commonly diagnosed in children
2. Neurofibromatosis 2
3. Schwannomatosis

} Typically diagnosed in early adulthood

Chronic condition, currently no cure

Tumors can grow anywhere in the nervous system and cause abnormalities in skin, bones and spinal cord.[1]

~100,000
Americans diagnosed[2]

All ages, sexes and ethnicities affected[2]

RELEVANCE TO MILITARY HEALTH

NF research offers a potential avenue for **advancing treatments** of other military-relevant conditions, such as cancer, osteoarthritis and other bone-related conditions.[3]

MHS medical encounters from 2013-2022 included **1,873** outpatient encounters for Service Members and **45,065** for other DOD beneficiaries.[4]

PROGRAM PRIORITIES

- Foster basic and exploratory research
- Facilitate rapid testing of potential therapeutics
- Increase research capacity
- Encourage research in areas of critical interest to patients

[1] https://www.mayoclinic.org/diseases-conditions/neurofibromatosis/symptoms-causes/syc-20350490

[2] https://www.mdanderson.org/cancer-types/neurofibromatosis.html

[3] https://www.nfnetwork.org/nf-network-advocacy-program/military-benefit/

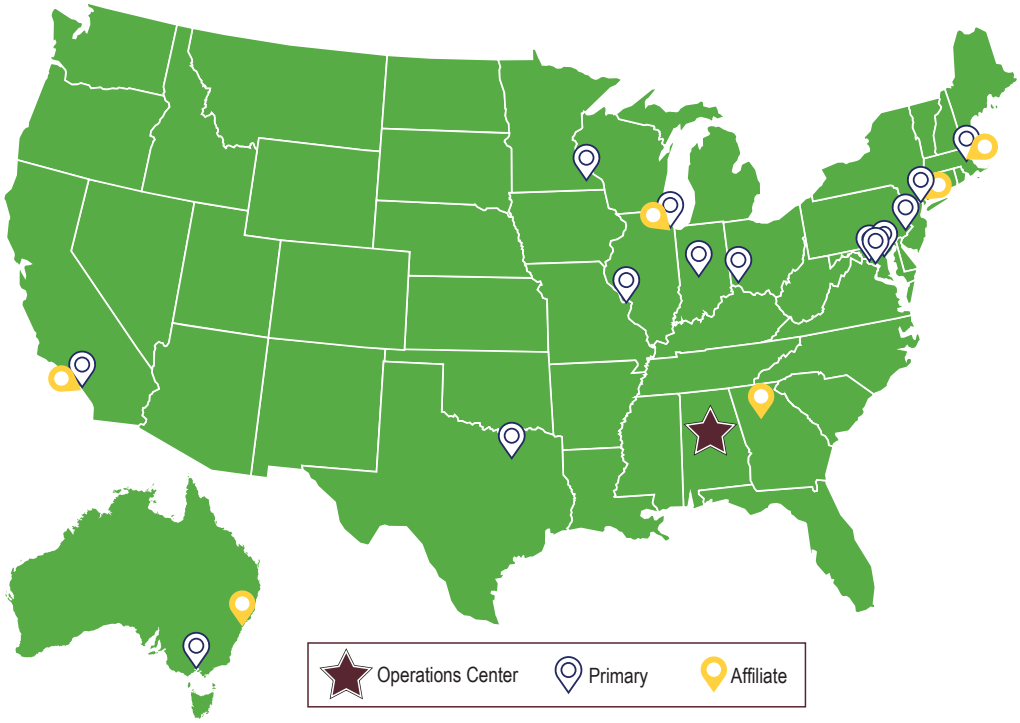
[4] Defense Medical Surveillance System, The Armed Forces Health Surveillance Division, Defense Health Agency, Silver Spring, Maryland.

For more information, visit: https://cdmrp.health.mil/nfrp

PROGRAM IMPACT AND OUTCOMES

NFRP-FUNDED NEUROFIBROMATOSIS CLINICAL TRIALS CONSORTIUM

- Established in 2006
- **15** clinical trials launched
  - **15** primary clinical sites
  - **6** affiliate sites around the U.S. and Australia
  - Operations center at the University of Alabama at Birmingham
- **$5M** leveraged from pharmaceutical companies and patient support organizations
- **NEW** efforts involve:
  - Facilitating comparison of clinical trials in the U.S. and Europe
  - Developing an imaging repository



NEUROFIBROMATOSIS CLINICAL TRIALS CONSORTIUM RESEARCH SPOTLIGHTS

Clinical Trial of Guanfacine – FDA-approved medication undergoing evaluation in children and adolescents with NF1 for the treatment of ADHD and cognitive deficits.

Clinical Trials and FDA Approval of Selumetinib – Phase 1 and phase 2 clinical trials supported regulatory approval for treatment of NF1 pediatric patients with inoperable plexiform neurofibromas.

DEDICATION TO INCREASING RESEARCH CAPACITY

69 AWARDS *and* $49.4M INVESTED to grow the NF research community and support investigators new to neurofibromatosis research.

NFRP RESEARCH RESOURCES INDEX

- Categorizes over **118 total research resources**, including animal models, datasets and molecular and cellular tools assessed in NFRP-funded research.

For more information on the resource, check here:

ONGOING HIGH-IMPACT RESEARCH

- Development of a **precision medicine platform** to evaluate combination drug treatment of malignant peripheral nerve sheath tumors using 3-D microtissue cultures
- Assessment of **nivolumab and ipilimumab** as a **potential therapy** for NF1 patients with newly diagnosed malignant or pre-malignant peripheral nerve sheath tumors
- Assessment of an **online platform "NF Guidelines to You" to increase rates of health surveillance** in children and adults with NF1 who do not have access to NF specialty clinics
- Advancement of **machine-learning based model** that computes **individualized assessments** of risk of post-radiation malignancy for NF2 patients, aiding treatment decisions

Point of Contact: CDMRP Public Affairs
dha.detrick.cdmrp.mbx.public-affairs@health.mil

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