Neurofibromatosis Network Advocacy Program

Structure Your Congressional Meeting

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1. Introduction & Thank you
2. Explain NF
   - Personal Story
3. How is NF Research Funded
   - Explain the NF Research Program (NFRP)
   - How the NFRP and NIH complement each other
4. Impact
   - Military Benefit
   - How NF Impacts State/District
   - Accomplishments
5. Fiscal year 2021 request

What is Neurofibromatosis (NF)?

- Highly variable genetic disorder of the nervous system which can affect every organ system
- Causes tumor growth along nerves
- NF is a family of tumor disorders where a protein is lacking
- Without this protein, tumors can grow in the brain, spine and along nerves that lead to a variety of issues
- NF occurs worldwide in all races and ethnic groups and both sexes and can appear in any family
- Some tumors may be visible, and some may not
- NF affects more than 120,000 Americans; this makes NF more prevalent than Cystic Fibrosis, hereditary Muscular Dystrophy, Huntington's disease and Tach Sachs combined

NEUROFIBROMATOSIS NETWORK

Neurofibromatosis Manifestations

Tumors

Growing Along Nerves

- Including Skin
- Brain
- Spinal Cord

Malignancies

Malignant peripheral nerve sheath tumor (MPNST)

Increased risk of breast cancer

Severe Pain

Learning Deficits/ Cognitive Disorders

Learning Disabilities

ADHD

Autism

Motor Deficits

Skin Conditions

Café-au-lait spots

Dermal neurofibromas

Nervous System Disorders

Neurofibromas

Epilepsy

Headaches

Visual Impairments

Tumors of the eye

Blindness

Retinal hamartomas

Deafness

Tumors of the ear

Vascular Disease

Hypertension

Dysplasia of blood vessels

Musculoskeletal Disorders

Muscle weakness

Scoliosis

Bone Abnormalities

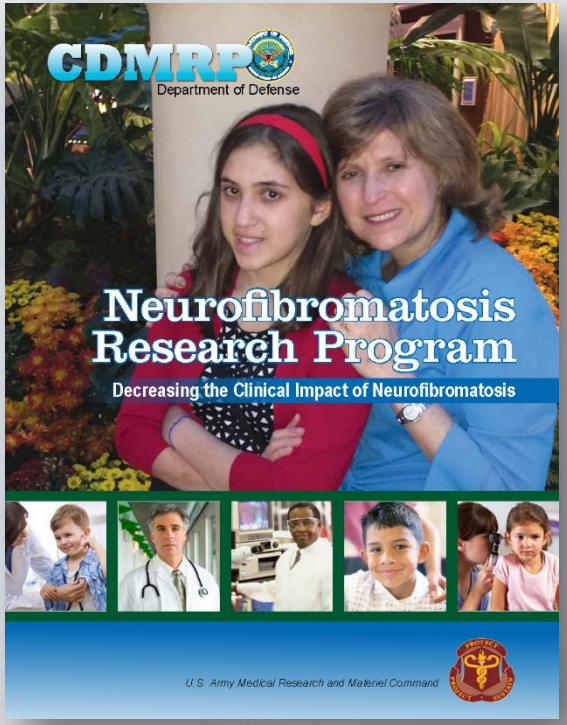
Congenital hydrocephalus

NEUROFIBROMATOSIS NETWORK


OUR INSPIRATIONS
DISABILITY
NEUROFIBROMATOSIS
NF
NETWORK

How is NF research funded?

Through the Department of Defense, Congressionally Directed Medical Research Program (CDMRP)



And several Institutes at the National Institutes of Health (NIH).

DoD Neurofibromatosis Research Program (NFRP) and National Institutes of Health (NIH)

- The NFRP has been efficiently run since 1996 through the Army Medical Research & Materiel Command that offers cutting edge awards through a competitive two tiered peer-review process to fill gaps in ongoing research, complementing initiatives sponsored by other agencies, such as the National Institutes of Health (NIH).

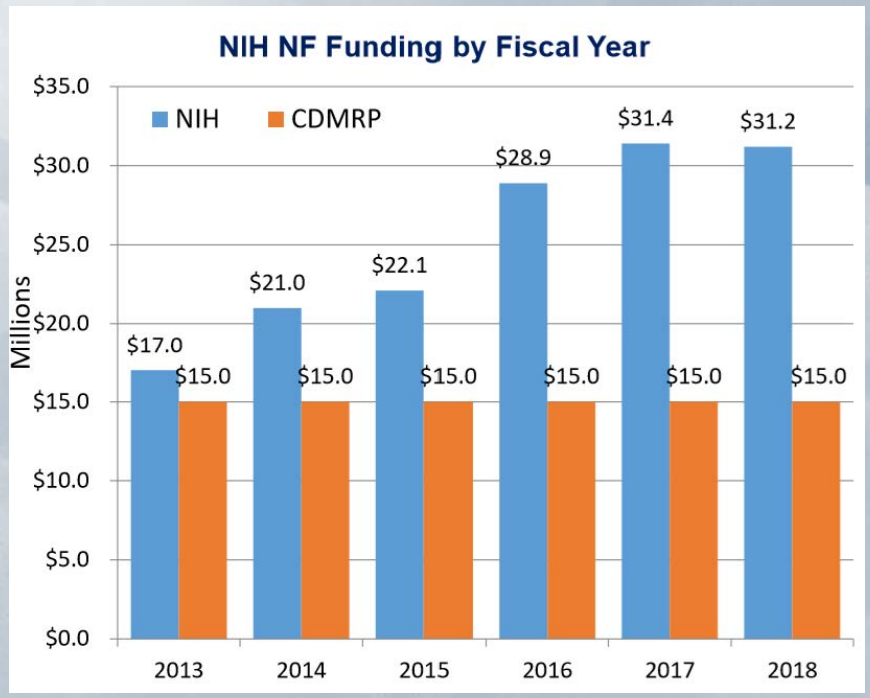
- The NFRP's successful peer-review process has participation from NF researchers, NIH and from Patient Representatives.

- The NF Clinical Trials Consortium, established with NFRP funds in 2006, significantly accelerates the clinical trial process by recruiting patients from clinical sites across the country with an operational center to analyze the data.

- NF specific report language encourages increased funding for NF research at several institutes at NIH.

NFRP and NIH research programs collaborate

- Due to the success of the NFRP, NF Research at NIH has proportionally increased since the inception of the CDMRP program.
- The NFRP funds cutting edge, higher risk research projects, these projects collect data which increases the chance for good NF science projects to be funded by NIH.
- NIH and NFRP collaborate on NF research throughout the year
- NIH holds an inter-institute meeting – NFRP is represented
- NFRP review panel meeting – NIH is represented

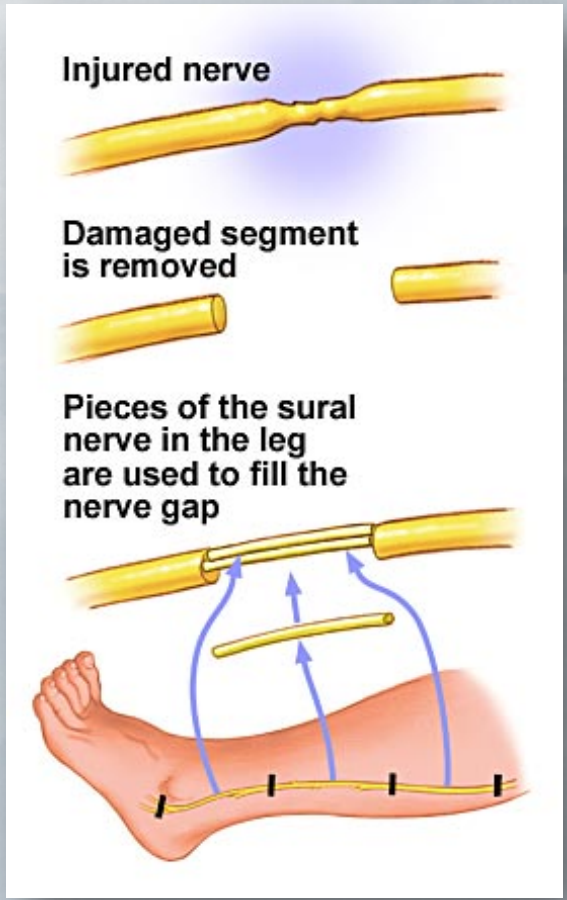


MILITARY BENEFIT

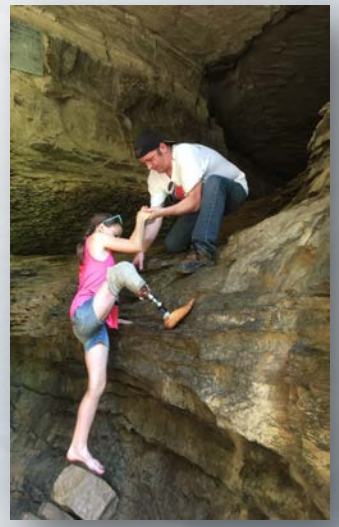
DEPARTMENT OF DEFENSE
NeurofibromatosisResearchProgram

Research on the NF population offers insight into many disease areas. The NFRP is providing critical research that is of benefit to the military and the general population.

- Mutated genes in NF are often mutated in diseased tissues in people who don't have NF.
- Proteins made by those NF genes play key roles in many normal body processes.
- Drugs that restore normal NF-associated function are being developed to treat the manifestations of NF, but may also help people without NF who are suffering from various conditions including **diseases of the nerves, brain, bones, blood vessels, and pain**.



Military Benefit



Bone Repair

Skeletal abnormalities affect up to one-third of patients with NF1. Included among these is improper fracture healing that may lead to permanent bone damage and amputation. Research involving NF1-associated bone disease and healing is likely to be more broadly applicable, including to military-associated injuries.

Pain

Pain is one of the most common symptoms for patients with NF, as it is for members of the military. Advances in the field of NF-associated pain may extend to benefit the military through better understanding of new drug targets, discovery of non-opioid therapeutics and personalized medicine.

Cancer

Both NF1 and military service are associated with increased risks of certain cancers, including breast cancer and sarcomas. Therefore, discoveries involving the biology or treatment of NF1-associated cancers are relevant and important to military service members and veterans.

Impact on State/District

US population: 313,800,000
US NF population: 128,032

How NF impacts State/District

DoD research funding to states
Fiscal Year 1996-2018

CDMRP NFRP Funding by Location

	Total
Alabama	34.72
Arizona	0.23
California	41.12
Conn.	1.83
District of Columbia	6.04
Florida	8.17
Georgia	2.27
Illinois	1.12
Indiana	14.36
Iowa	3.38
Louisiana	1.62
Kentucky	0.81
Maine	0.92
Maryland	9.93
Massachusetts	49.67
Michigan	9.15
Minnesota	2.94
Missouri	8.82
New Jersey	1.44
New York	14.53
North Carolina	3.70
Ohio	20.68
Oklahoma	0.76
Oregon	4.29
Pennsylvania	12.59
South Carolina	0.78
Tennessee	0.58
Texas	11.18
Utah	3.03
Virginia	1.84
Washington	3.25
West Virginia	0.43
Wisconsin	0.78
FINAL TOTALS	276.96

DoD Neurofibromatosis Research Program (NFRP)

Vision

Decrease the clinical impact of neurofibromatosis

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

Accomplishments of the NFRP

February 2018, AstraZeneca and Merck announced that the FDA granted OrphanDrug Designation for **selumetinib, a MEK** 1/2 inhibitor, for the treatment of NF1.

- Preclinical studies on selumetinib were funded by the NFRP
- Data on selumetinib study led to clinical trial through the NIH
- Recruitment for clinical trial supported by the NF Clinical Trial Consortium funded by the NFRP
- Over 70% of participants on this MEK clinical trial are showing reductions of 20-50% in plexiform tumor size.

Kristy participating in MEK Clinical Trial

Accomplishments of the NFRP

NF Clinical Trials Consortium (NFCTC)

- Established in 2006 to develop and perform clinical trials
- Consortium is composed of 25 clinical sites
- Allows partnership with well established NF Centers
- 13 completed and ongoing Clinical Trials, 4 more Clinical Trials to open in 2020

New Investigators Awards

FY	Awards Made	Funds Invested
99-17	68	$39M
99-13	54	$30M

67% retention rate

149 Publications
22 Research Resources
65 Awards Received
$15M Funding Obtained

Exploration Hypothesis Development Awards

Exploration of innovative, high-risk, high-gain, and groundbreaking concepts

Initial NFRP investment **$2.9 M** *(25 awards)*

Follow-on funding received **$13.2 M** Return on investment **4.5X**

27 follow-on awards and 36 publications

Fiscal Year 2021 Request

- **Defense Appropriation Request:** $20 million for the Army's NF Research Program (NFRP) in the FY 2021 Department of Defense Appropriations bill.

- **Labor HHS Appropriations Request:** Inclusion of report language on NF research at the National Institutes of Health in the FY 2021 Labor, Health and Human Services, Education Appropriations bill.

Challenges the NF Clinical Trial Consortium face due to funding issues

- As the science has rapidly advanced, we now have more potential trials and medications to evaluate than funding will support

- The increasing complexity of the regulatory requirements (e.g. ethics board and FDA compliance) have created a need to increase operation center staff to keep up with requirements

- Consortium sites have always been thinly supported. Thus, the participating institutions lose money in order to participate and provide these cutting-edge therapies to their patients

- We have continual requests to add sites to increase geographic access that we are unable to accommodate for lack of funding

- Maintaining the research database is increasingly expensive, particularly in light of new FDA compliance requirements

How you can help:

Sign onto the Dear Colleague letter that will be circulated in both the House and Senate. We will contact you when it is circulated.

- Letters sponsors include:
  - Senator Edward Markey (D-MA): Adam Axler
  - Rep. Collin C. Peterson (D-MN): Rebekah Solem
  - Rep. Glenn Grothman (R-WI): Patrick Konrath
  - Rep. Peter Welch (D-VT): Isaac Loeb
  - Rep. Pete Stauber (R-MN): Jeff Bishop

- Include these requests on your **priority/wish list** and submit the request to the Defense Appropriations Subcommittee ($20million) and the Labor, Health and Human Services Appropriations Subcommittee (report language).

Thank you for your time!