



Department of Defense

*Neurofibromatosis
Research Program*

2000 Awards Book

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Headquarters, U.S. Army Medical Research and Materiel Command
MCMR-PLF, 1077 Patchel Street
Fort Detrick, Maryland 21702-5024



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INTRODUCTION

The U.S. Army Medical Research and Materiel Command is pleased to present the award list of funded projects for the fiscal year 2000 (FY00) Neurofibromatosis Research Program. Award negotiations were completed on September 30, 2001. The awards listed in this document were selected by a competitive two-tiered review process. Funding decisions were based upon scientific excellence evaluated in the first tier of review, followed by programmatic relevance judged in the second tier. These projects represent a diverse portfolio of scientific research directed toward the program's overall goal of promoting studies toward the understanding, diagnosis, and treatment of neurofibromatosis, as well as the enhancement of the quality of life for persons with the disease.

Congressional direction for FY00 specified \$15 million for neurofibromatosis research. Following the receipt of funds, a programmatic strategy was developed, proposals were solicited and evaluated, award recommendations were made, and contract negotiations were completed. The FY00 programmatic strategy called for Idea Awards, New Investigator Awards, Investigator-Initiated Research Awards (with or without Nested Postdoctoral Traineeships), and Clinical Trial Awards. The Idea Award is intended to encourage innovative approaches to neurofibromatosis research. The intent of the New Investigator Award is to prepare new, independent investigators for careers in neurofibromatosis and to attract established investigators new to the neurofibromatosis field. Idea Awards and New Investigator Awards do not require preliminary or pilot data. The intent of the Investigator-Initiated Research Award is to sponsor basic research leading to clinical trials relevant to neurofibromatosis or drugs that can be introduced into clinical trials. Nested Postdoctoral Traineeships, offered as an optional component of Investigator-Initiated Research Awards, are intended to enable doctoral degree graduates to either extend ongoing research related to neurofibromatosis or broaden the scope of their research to include work relevant to neurofibromatosis. The Clinical Trial Award is intended to sponsor clinical pharmacologic or gene therapy studies that look at toxicities (Phase 1) or investigate the efficacy (Phase 2) of any novel therapeutic approach for neurofibromatosis type 1 or type 2. A total of 20 studies were funded.

As the funded scientists embark on these projects, the Department of Defense and the U.S. Army gratefully acknowledge the participation of their scientific advisors, people living with neurofibromatosis, and the neurofibromatosis advocacy community. The expertise, vision, and diversity of perspectives of all individuals who contributed to this program were vital to developing a sound investment strategy on behalf of all persons living with neurofibromatosis. It is with great anticipation and excitement that we await the outcomes of this research.

FY00 Neurofibromatosis Research Program

FUNDED AWARDS

FY00 Neurofibromatosis Research Program

Idea Awards

Log Number	Principal Investigator		Institution	Proposal Title	Award Amount
	Last Name	First Name			
NF000018	Foster	Rosemary	Massachusetts General Hospital	High Sensitivity SELDI Analysis of NF1 Interactors in Mammals, Drosophila, and Yeast	\$346,000
NF000026	Hock	Janet	Indiana University, Indianapolis	Regulation of Bone Homeostasis in a Mouse Model of Neurofibromatosis Type 1 (NF1)	\$296,451
NF000010	Pulst	Stefan	Cedars-Sinai Medical Center	Expression Profiling of Cell Lines Expressing Regulated NF2 Transcripts	\$301,385
NF000022	Ratner	Nancy	Cincinnati, University of	Neurofibroma Schwann Cell Gene Expression Analysis	\$306,000
NF000014	Stephens	Karen	Washington, University of	QuEST: A New Approach to Molecular Staging of Tumors	\$297,888
NF000012	Van Aelst	Linda	Cold Spring Harbor Laboratory	Isolation and Characterization of NF1 Target Genes	\$334,000

FY00 Neurofibromatosis Research Program

New Investigator Awards

Log Number	Principal Investigator		Institution	Proposal Title	Award Amount
	Last Name	First Name			
NF000004	Hornyak	Thomas	Henry Ford Health System	Neurofibromin and Melanocyte Development and Differentiation	\$464,751
NF000002	Lajeunesse	Dennis	North Carolina, University of, Greensboro	Genetic and Molecular Characterization of Drosophila Brakeless: A Novel Modifier of Merlin Phenotypes	\$371,050
NF000013	Qin	Jun	Baylor College of Medicine	NF1-Associated Protein and Its Role in Human Genome Maintenance	\$447,998
NF000023	Rizvi	Tilat	Cincinnati, University of	Role of Neurofibromin in Gliogenesis and Astroglisis	\$457,600
NF000020	Stemmer Rachamimov	Anat	Massachusetts General Hospital	Growth Mechanisms of Schwann Cell Tumors in NF2	\$518,970
NF000029	Zhang	Lurong	Georgetown University	Therapeutic Effect of Targeted Hyaluronan Binding Peptide on Neurofibromatosis	\$465,531

FY00 Neurofibromatosis Research Program
Investigator-Initiated Research Awards

Log Number	Principal Investigator		Institution	Proposal Title	Award Amount
	Last Name	First Name			
NF000024	Barker	Peter	Johns Hopkins University, East Baltimore Campus	Proton MR Spectroscopic Imaging in NF-1	\$596,935
NF000017*	Bernards	Andre	Massachusetts General Hospital	Role of NF1 cAMP Pathways	\$843,960
NF000035*	Clapp	David	Indiana University, Indianapolis	Biochemical and Phenotypic Effects of Heterozygous Inactivation of N(f)1 in Mast Cells	\$641,885
NF000025*	Derewenda	Zygmunt	Virginia, University of	Structure-Function Relationships in Merlin, the Product of the NF2 Causal Gene	\$815,791
NF000032	Field	Jeffrey	Pennsylvania, University of	Novel Signaling Pathways for the NF1 Gene	\$884,370
NF000015	Slattery III	William	House Ear Institute	Neurofibromatosis Type 2 Natural History Consortium	\$2,990,234
NF000016	Wallace	Margaret	Florida, University of	Steroid Hormones in NF1 Tumorigenesis	\$711,454

*Provides support for a Nested Postdoctoral Traineeship

FY00 Neurofibromatosis Research Program

Clinical Trial Award

Log Number	Principal Investigator		Institution	Proposal Title	Award Amount
	Last Name	First Name			
NF000027	Widemann	Brigitte	National Cancer Institute	A Phase II Trial of the Farnesyltransferase Inhibitor R115777 in Pediatric Patients with Neurofibromatosis Type 1	\$365,683

***FY00 Neurofibromatosis Research Program
Participants***

FY00 Neurofibromatosis Research Program Peer Reviewers

IP Member	Degree	Institution/Affiliation
Ahn, Chul	Ph.D.	Clinical Epidemiology/Internal Medicine, University of Texas Medical School at Houston
Barald, Katharine F.	Ph.D.	Department of Developmental and Cell Biology, University of Michigan Medical School
Brannan, Camilynn I.	Ph.D.	Department of Molecular Genetics and Microbiology, University of Florida College of Medicine
Brown, Truman R.	Ph.D.	Department of Nuclear Magnetic Resonance, Fox Chase Cancer Center
Carroll, William L.	M.D.	Department of Pediatrics, University of Utah, School of Medicine
Castleberry, Robert P.	M.D.	Department of Pediatrics, University of Alabama at Birmingham
Cichowski, Karen	Ph.D.	Center for Cancer Research, Massachusetts Institute of Technology
Clapp, D. Wade	M.D.	Department of Pediatrics, Microbiology and Immunology, Indiana University School of Medicine
Curtis, Mary Allene	M.D.	Department of Pediatrics, University of Arkansas for Medical Sciences
Fehon, Richard G.	Ph.D.	Department of Developmental, Cell, and Molecular Biology, Duke University
Feldman, Doreen S.		Neurofibromatosis, Inc., Mid-Atlantic Chapter
Fernandez-Valle, Cristina	Ph.D.	Department of Molecular Biology and Microbiology, University of Central Florida
Fisch, Gene	Ph.D.	Department of Biostatistics, Epidemiology, and Public Health, Yale University
Friedman, Jan Marshall	M.D., Ph.D.	Department of Birth Defects and Pediatric Genetics Branch, Centers for Disease Control
Gobel, Stephen	D.D.S.	Executive Secretary
Haber, Roberta	Ph.D.	Executive Secretary
Katz, Michael I.		National Neurofibromatosis Foundation, Inc.
MacCollin, Mia	M.D.	Neuroscience Center, Massachusetts General Hospital, East
McClatchey, Andrea	Ph.D.	Department of Pathology, Massachusetts General Hospital Center
Moore, Bartlett D.	Ph.D.	Division of Pediatrics, University of Texas M.D. Anderson Cancer Center
O'Day, Michie Stovall		The National Neurofibromatosis Foundation, Inc.
Price, Kathryn Lynn		The Texas Neurofibromatosis Foundation
Ratner, Nancy	Ph.D.	Department of Cell Biology, Neurobiology and Anatomy, The Vontz Center for Molecular Studies
Robison, Leslie L.	Ph.D.	Department of Pediatrics, University of Minnesota

IP Member	Degree	Institution/Affiliation
Rutkowski, Lynn	Ph.D.	Department of Neurology, Children's Hospital of Philadelphia
Scoles, Daniel R.	Ph.D.	Neurogenetics Laboratory, Cedars-Sinai Medical Center, University of California Los Angeles School of Medicine
Shannon, Kevin M.	M.D.	University of California School of Medicine
Sherman, Lawrence S.	Ph.D.	Vontz Center for Molecular Studies, University of Cincinnati College of Medicine
Viskochil, David	M.D., Ph.D.	Department of Pediatrics, University of Utah
Welling, Duane Bradley	M.D.	Department of Otolaryngology, The Ohio State University

FY00 Neurofibromatosis Research Program Integration Panel (IP) Members

IP Member	Degree	Institution/Affiliation
Adamson, Peter	M.D.	Division of Clinical Pharmacology and Therapeutics, Children's Hospital of Philadelphia
Bellermann, Peter	M.P.A.	The National Neurofibromatosis Foundation
Copeland, Neal	Ph.D.	Mammalian Genetics Laboratory, National Cancer Institute, Frederick Cancer Research Center
Fischbeck, Kurt	M.D.	Neurogenetics Branch, National Institutes of Health, National Institute of Neurological Disorders and Strokes
Gusella, James (Chair Emeritus)	Ph.D.	Molecular Neurogenetics Unit, Massachusetts General Hospital East
Hall, Zach	Ph.D.	University of California, San Francisco
Murray, Robert, Jr.	M.D.	Division of Medical Genetics, Department of Pediatrics and Child Health, Howard University College of Medicine
Pleasure, David (Chair)	M.D.	Division of Neurology, Department of Pediatrics, The Children's Hospital of Philadelphia
Rubenstein, Allan (Chair-Elect)	M.D.	Department of Neurology, Mount Sinai School of Medicine
Vézina, Louis-Gilbert	M.D.	Department of Diagnostic Imaging and Radiology, Children's National Medical Center
Wilson, Mary Ann	B.A.	Neurofibromatosis, Inc.

FY00 Neurofibromatosis Research Program Ad Hoc Programmatic Reviewers

IP Member	Degree	Institution/Affiliation
Duffy, Brenda	M.A.	Neurofibromatosis, Inc.
Mulvihill, John	M.D.	Department of Pediatrics, University of Oklahoma

GLOSSARY OF TERMS

Glossary of Terms

Clinical Trial Award: The intent of this award mechanism is to sponsor clinical pharmacologic or gene therapy studies that look at toxicities (Phase 1) or investigate the efficacy (Phase 2) of any novel therapeutic approach for NF1 or NF2. Applicants must include preliminary data to support the feasibility of their hypotheses and approaches, along with a detailed plan to conduct a Phase 1 or 2 clinical trial during the course of the award. Ultimately, the goal of this award mechanism is to sponsor novel research that will substantially improve today's approach to the treatment of neurofibromatosis (NF).

Idea Award: The intent of this award mechanism is to encourage innovative ideas and technology to NF research. These proposals may represent a new paradigm in the study of NF, challenge existing paradigms, or look at an existing problem from a new perspective. The proposed studies may be untested, but present a high probability of revealing new avenues of investigation. Although this research is inherently risky in nature, the research plan must demonstrate solid scientific judgment and rationale. Preliminary or pilot data is not required for this award mechanism.

Investigator-Initiated Research Award: The intent of this award mechanism is to sponsor basic research leading to clinical trials relevant to NF or drugs that can be introduced into clinical trials. These grants are intended to fund independent investigators across a broad spectrum of disciplines. This award mechanism also supports the establishment of synergistic, goal-focused, and non-exclusionary consortia. Preliminary data relevant to NF research is required for these awards. Nested Postdoctoral Traineeships are being offered as an optional part of Investigator-Initiated Research Award proposals. The intent of the Nested Postdoctoral Traineeship is to enable doctoral degree graduates to either extend ongoing research related to NF or broaden the scope of their research to include work relevant to NF under the guidance of a designated mentor who is participating in the proposal.

New Investigator Award: The intent of this award mechanism is to promote and reward innovative ideas and technology from investigators in the early phases of their careers as well as those investigators new to NF research who have little or no preliminary data in NF. This research may represent a new paradigm, challenge existing paradigms, or look at an existing problem from a new perspective. A New Investigator is defined as an independent investigator below the level of Associate Professor with access to appropriate research facilities. Preliminary or pilot data is not required for this award mechanism.