Eunice Kennedy Shriver National Institute of Child Health & Human Development Division of Intramural Research

Constantine A. Stratakis, MD, D(med)Sci Scientific Director

> Open Session Report September 14, 2017, 10:05 am



Eunice Kennedy Shriver National Institute of Child Health and Human Development

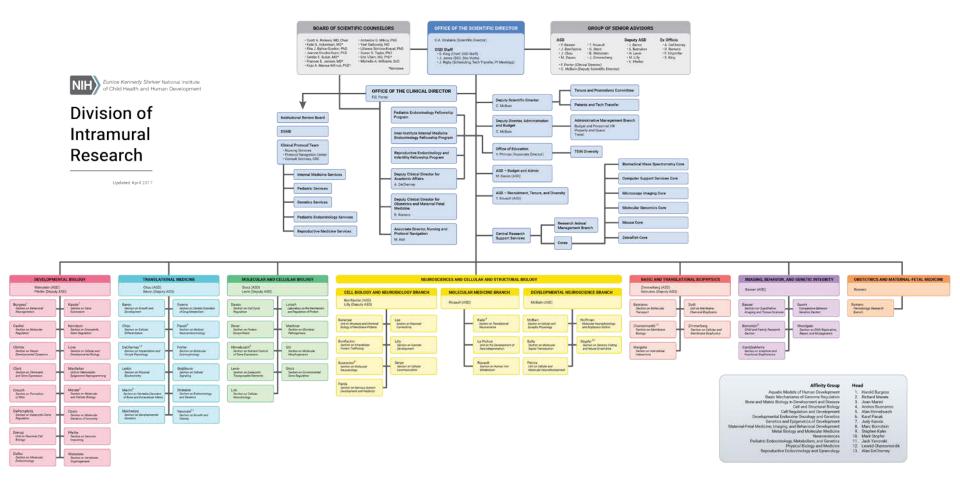


NICHD DIR by the Numbers

- Total personnel of more than 950 employees
 - 62 Pls (56 Tenured; 6 Tenure-Track)
 - 2 tenure-track recruits in FY16, 1 more in FY17
 - 2 retirements in FY17
- >80 Clinical Protocols
 - 2/3 at the NIH Clinical Center
 - 1/3 at Wayne State and elsewhere
- Accredited medical training programs:
 - o Pediatric Endocrinology
 - o Adult Endocrinology
 - Reproductive Endocrinology and Infertility
 - Medical Genetics (with NHGRI)
 - Perinatal Research and Obstetrics (Wayne State Univ/PPRO)







Affinity Group Membership

Aquatic Models of Human Development		
Burgess, Harold [Head]		
Chitnis, Ajay		
Drerup, Katie		
Feldman, Ben (Core)		
Weinstein, Brant		

Basic Mechanisms of Genome Regulation

Maraia, Richard [Head]

Cashel, Mike

Clark, David

Crouch, Bob

Depamphilis, Melvin

Woodgate, Roger

Bone & Matrix Biology in Development	
& Disease	
Marini, Joan [Head]	
Leikin, Sergey	

Cell Regulation & Development Hinnebusch, Alan [Head] Dasso, Mary Dever, Tom Dufau, Maria Lee, Chi-Hon Levin, Henry Lorsch, Jon (NIGMS) Owens, Ida Serpe, Mihaela

Shi, Yun-Bo

Cell & Structural Biology Group		
Buonanno, Andres [Head]		
Banerjee, Anirban		
Bonifacino, Juan		
Lilly, Mary		
Machner, Matthias		
Storz, Gisela		

Developmental Endocrine Oncology & Genetics Pacak, Karel [Head] Backlund, Peter (Core) Lodish, Maya (Training Program) Stojilkovic, Stanko Stratakis, Constantine

Genetics & Epigenetics of Development Kassis, Judy [Head] Kennison, Jim Love, Paul Macfarlan, Todd Ozato, Keiko Pfeifer, Karl

Physical Biology & Medicine Chernomordik, Leonid [Head] Bezrukov, Sergey Sodt, Alexander Zimmerberg, Joshua

Reproductive Endocrinology & Gynecology Decherney, Alan [Head]

Maternal-Fetal Medicine, Imaging, &	
Behavioral Development	
Bornstein, Marc [Head]	
Basser, Peter	
Gandjbakhche, Amir	
Margolis, Leonid	
Romero, Roberto	
Suomi, Stephen	

Metal Biology & Molecular Medicine Kaler, Stephen [Head] Le Pichon, Claire Rouault, Tracey

Neurosciences
Stopfer, Mark [Head]
Balla, Tamas
Fields, Douglas
Hoffman, Dax
Loh, Y Peng
McBain, Chris
Petros, Tim

Pediatric Endocrinology, Metabolism, &
Genetics
Yanovski, Jack [Head]
Baron, Jeff
Chou, Janice
Delaney, Angela
Merke, Deborah (CC)
Mukherjee, Anil
Porter, Forbes



NICHD Board of Scientific Counselors

Scott Rivkees, MD (Chair)	U of Florida	Pediatric Endocrinology
Kate Ackerman, MD	U of Rochester	Dev Bio/Genetics/Pediatrics
Rita Balice-Gordon, PhD	Sanofi	Neuroscience
Jeanne Brooks-Gunn, PhD	Columbia	Dev Psych/Behavioral Sci
Serdar Bulun, MD	Northwestern	Obstetrics/Gynecology
Frances Jensen, MD	U of Pennsylvania	Neuroscience
Kojo Mensa-Wilmot, PhD	U of Georgia	Cell Biology
Antonios Mikos, PhD	Rice University	Bioengineering/Biophysics
Yoel Sadovsky, MD	Magee-Womens/Pitt	Rep Bio/Obstetrics/Gynecology
Lilianna Solnica-Krezel, PhD	WUSTL	Developmental Biology
Susan Taylor, PhD	UCSD	Struc Bio/Mol Biochem
Eric Vilain, MD, PhD	UCLA	Mol & Human Genetics
Michelle Williams, ScD	Harvard	Epidemiology



Vanessa Auld, PhD

Expertise: Developmental Biology

Professor,

Department of Zoology, University of British Columbia

Elizabeth Bonney, MD, MPH

Expertise: Molecular Biology, DNA/RNA Professor, Director of Research Division Dept of Ob/Gyn & Reproductive Sciences

University of Vermont





NICHD Board of Scientific Counselors

Deborah Johnson, PhD

Expertise: Molecular & Cellular Biology

Professor,

Department of Molecular & Cellular Biology, Baylor College of Medicine



Martha Werler, DSc

Expertise: Epidemiology

Professor & Chair, Department of Epidemiology, Boston University School of Public Health

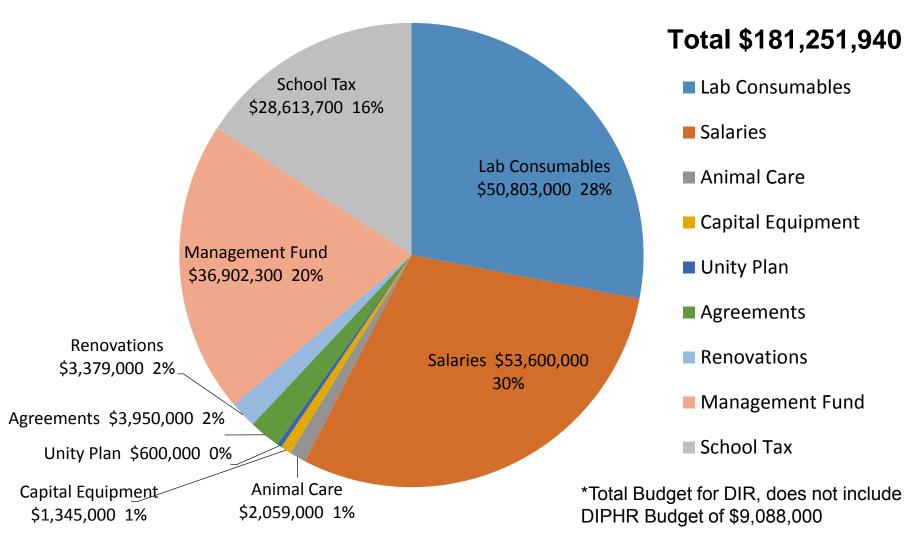




Budget & Personnel

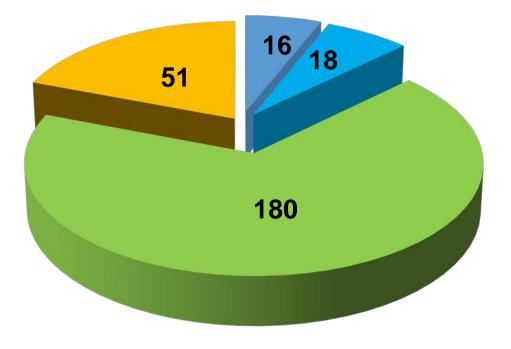


NICHD DIR Budget: FY2017 Estimates





NICHD Intramural Trainee Numbers As of April 2017, 265 total



- Clinical Fellows (16)
- Graduate Students (18)
- Postdocs (180)
- Postbacs (51)

Retirements

Igor Dawid, PhD Retired December 2016





Thomas Sargent, PhD

Retired January 2017

Recruitments - DIR

Catherine Drerup, PhD

Developmental Biology Started October 3, 2016



Claire Le Pichon, PhD

Neurosciences & Cellular & Structural Biology Started September 18, 2016

Timothy Petros, PhD

Neurosciences Started January 17, 2017





NIH Eunice Kennedy Shriver National Institute of Child Health and Human Development:

Tenure-Track or Tenure-Eligible Research Positions in Cellular & Developmental Neurobiology and Translational Research Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) National Institutes of Health Department of Health and Human Services

We are recruiting for two outstanding tenure-track investigators or tenure-eligible researchers to create innovative and vibrant research programs. The applicants will join our interactive faculty who use a variety of models to study basic mechanisms of development, pediatric disease processes, and their translation into clinical treatments (see http://www.nichd.nih.gov/about/org/dir/Pages/index.aspx).

The positions are fully supported by the intramural program of NICHD and include a start-up allowance as well as an ongoing commitment of research space, laboratory resources, and positions for staff and trainees. The successful applicants will join a faculty of 65 principal investigators whose work covers a broad range of basic, clinical, and translational research areas.

Cellular & Developmental Neurobiology (Tenure-track or Tenure-eligible)

The successful applicant must have a Ph.D., M.D., M.D./Ph.D. or equivalent doctoral degree, and will work on cellular/molecular mechanisms regulating neuronal development and differentiation at the Porter Neuroscience Research Center (PNRC) on the NIH main campus in Bethesda, Maryland, a multi-institute center dedicated to basic and clinical brain research (see https://www.nih.gov/PNRC). Applicants will have access to state-of-the art imaging equipment in their lab and at the PNRC imaging facility, NICHD core mouse and zebrafish facilities for the study of model organisms, and biomedical imaging and metabolic assessment facilities (see http://clinicalcenter.nih.gov/).

Translational Research (Tenure-track)

The successful applicant(s) must have an M.D. or M.D./Ph.D degree, or equivalent, and will join a Physician Scientist Development Program, which promotes the development of an independent research career. The investigator will combine laboratory research with a focus on human disease pathophysiology and treatment, with an emphasis on pediatric and women's health issues, although individuals from all subspecialty backgrounds that are aligned with our intramural research mission will receive equal consideration. The facilities of the NIH Clinical Center provide state-of-the-art diagnostic, treatment, and research support, in our 200-bed hospital and 93 day-patient stations devoted exclusively to the care of patients on 1600 clinical research protocols.

Honors & Awards

Gigi Storz, PhD

Biomedical Research Exemplar, WUSTL Research Exemplar Project For individuals doing high-quality, high-impact, federally-funded research who also maintain an impressive reputation for professionalism and integrity in their work

2017 Anita B. Roberts lecturer For Distinguished Women Scientists at NIH

2017 Ruth L. Kirschstein Mentoring Award





Competitive Awards

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NIH Bench-to-Bedside Awards

- Bench-To-Bedside Award Program (1999)
 - \$135,000 direct costs for two years
 - Increased to \$150,000 for 2017
 - Competitive awards
- Criteria
 - Utilize the NIH Clinical Center
 - Basic/clinical collaboration
 - Intramural/extramural collaboration (2006)





Opportunities for Collaborations at the NIH Clinical Center (U-01)



	CYCLE 1	CYCLE 2
LOI's /X02's	Total	Total
Applications	51	33
Awards	10	5 (to-date)
Success Rate	20%	18%

- Goal: to support collaborative research projects aligned with NIH efforts to enhance the translation of basic biological discoveries into clinical applications that improve health
- Up to \$500K/year x 3 years, renewable
- Teams *must* have one extramural and one intramural co-PI
- Some of the work *must* be done at the CC
- NICHD: 2 awards in Cycle 1
 - 2 awards in Cycle 2
 - **3 applications in Cycle 3**
 - 2 applications in Cycle 4



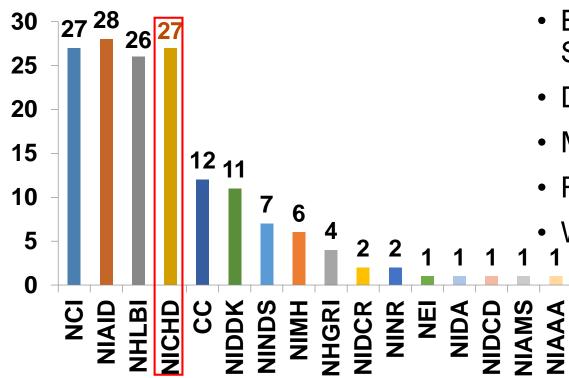
NICHD Bench-to-Bedside Awards, FY17-18

NICHD PI	Extramural Collaborators	Project Title	Total Funding
Yanovski, Jack	Kristen O'Connell (U of Tenn)	Melanocortin agonist to bypass leptin resistance of Bardet-Biedl Syndrome	\$353,200
Porter, Forbes	Daniel Ory (WUSTL)	Investigations of Juvenile Neuronal Ceroid Lipofuscinosis (CLN3)	\$285,000
Stratakis, Constantine	Tomlins Rainey (UMich)	Therapeutic Targets in African Americans with Primary Aldosteronism	\$285,000



NIH Bench-to-Bedside Awards

Bench-to-Bedside Awards by Lead Institute 2006 – 2015



- AIDS
- Behavioral and Social Sciences
- Dietary Supplements
- Minority Health
- Rare Diseases
- Women's Health Projects

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DDIR Innovation Awards

- The goals of the program are to provide seed money for innovative and high-impact research, and to stimulate interactions among investigators.
- Follows recommendation from the IRP Review
- Four types of awards:
 - Building Scientific Communities program project awards
 - Building Research Capacity centers & facilities
 - Building Bridges collaborations with extramural investigators & industry
 - Building a Strong Workforce



DDIR Innovation Awards

PIs	Project Title
Chitnis, Ajay Tanner, Kandice (NCI) Liu, Jian (NHLBI)	Investigating Collective migration in the Zebrafish Lateral Line primordium (\$300K)
Zimmerberg, Joshua Duffy, Patrick (NIAID)	Advancing NIH Super-resolution Optical Microscopy Capabilities <mark>(\$500K)</mark>
Hoon, Mark (NIDCR) Chesler, Alexander (NCCIH) Le Pichon, Claire Ryba, Nicholas (NIDCR)	Mechanisms Linking Neural Stress and Injury to Persistent Pain (\$300K)
Hu, Guang (NIEHS) Macfarlan, Todd Ge, Kai (NIDDK) Wade, Paul (NIEHS)	Systematic Dissection of the Mechanisms Governing Retrotransposon Silencing (\$250K)

NICHD DIR Competitive Awards

NICHD DIR Director's Awards, FY16

- Two years of funding
 - FY14-15, \$3M
 - FY16-17, \$2M, 8 of 25 applications funded
- Established pursuant to a recommendation in the July 2013 Blue Ribbon Panel Report to create competitive research opportunities with a collaborative focus among investigators, and to support new research ideas.
- Uses a modified application based on an R-21, and an expedited administrative review process with the support of NICHD's Division of Extramural Research and a panel of NIH extramural reviewers.



NICHD DIR Director's Awards, FY16-17

PI	Collaborators	Title
	Roche, Katherine W (NINDS) McBain, Chris Delaney, Angela	The role of DLG2/PSD-93 in the initiation of puberty and the impact of mutations on NMDA receptor signaling and pubertal disorders
Chernomordik, Leonid Margolis, Leonid	Love, Paul	Syncytin-carrying exosomes in placental induction of regulatory T cells: a mechanism of maternal/fetal tolerance
Dasso, Mary	Serpe, Mihaela Oliver, Brian (NIDDK)	Mechanistic analysis of IRBIT protein function in tissue homeostasis
Kassis, Judy	Pfeifer, Karl	The role of the Wapal, a regulator of cohesin removal, in development and disease
	Pfeifer, Karl Fan, Ruzong (DIPHR) Dave, Uptal (Vanderbilt) Ehrlich, Lauren (UT Austin)	Investigating the role of Ldb1 transcription complexes in T cell Acute Lymphoblastic Leukemia
Marini, Joan	Yanovski, Jack	PEDF-PPARā-CyclophilinA/B pathway modulates osteogenesis and adipogenesis
Stopfer, Mark Lee, Chi-Hon		Neural Mechanisms of Gustatory Coding
Weinstein, Brant	Bonifacino, Juan	Regulation of endocytic trafficking by the microtubule motor dynein

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NICHD DIR Competitive Awards

NIH Intramural-to-Russia: US-Russia Collaborative Research Partnerships in HIV/AIDS and Comorbidities Awards

Funding through the NIH Office of AIDS Research

- 3 years of funding, FY17-FY19
- Reviewed in parallel by NIH's Center for Scientific Review and the Russian Foundation for Basic Research
- The program seeks to further develop crucial research infrastructure and capacity building between research communities in the U.S. and Russia.



NIH Intramural-to-Russia Awards, FY17-19

PI	Project Title
Zimmerberg, Joshua	GAG protein of HIV: from mechanisms of self-assembly towards novel vaccine (\$19K in FY17; \$57K over 3 years)
Margolis, Leonid	Multi-targeted antivirals for prevention and treatment of HIV infection (\$93K in FY17; \$263K over 3 years)



Office of AIDS Research Strategic Funding, FY17

PI(s)	Project Title
Zimmerberg, Joshua	Determining the cellular mechanisms of HIV viral assembly and latency (\$321K)
Chernomordik, Leonid Margolis, Leonid	New potential target for HIV antiviral: Phosphatidylserine dependence of HIV cell entry (\$380K)



Postdoctoral Research Associate (PRAT) Fellowships

- Administered by NIGMS, the PRAT program supports ~20 postdoctoral fellows in intramural NIH labs, providing 3 years stipend support
- 2017 Awards:
 - Sara Young (Dever Lab)
 - Abhi Subedi (Burgess Lab)
 - Jim D'Amour (McBain Lab)
- Fellows currently in the program:
 - Joo Yun Jun (Yanovski Lab)
 - Jonathan Murphy (Hoffman Lab)
 - Ashley Swanson (Woodgate Lab)

NICHD now hosts six PRAT fellows out of 20 slots

Intramural Research Fellowships

- For Postdoctoral and Clinical Fellows in their second or third training year
- Training on how to write various components of an NIH grant
- Application prepared by fellow under guidance of their mentor
 - 10 pages max. (NIH Biosketch, Specific Aims, Significance and Approach, Budget Justification, and Mentor's Statement of Support)
- Award of \$30K for 1 year, toward the fellow's stipend; up to 3 awards per year

Review by BSC members. Fellows' applications and reviewing instructions will be sent on August 15th. Scores with comments are due by October 1st.

Diversity Initiatives

committed to train, support, and sustain a diverse cohort of individuals traditionally underrepresented in science

• NICHD Developing Talent Scholars Program

Postbacs/Grad Students: 2 new recruits for '17-18

- LaTaijah Crawford (Shi Lab)
- Carlos Echeverria (Dasso Lab)
- Two alumni (Rim Mehari and Nicolas Johnson)
 recently accepted into medical school
- Summer Trainees
 - Centrally-funded slots for students from groups traditionally underrepresented in science or from disadvantaged backgrounds
 - 15 filled for 2017
- Fellows Recruitment Incentive Award
 Postdocs: 1 recruit every fiscal year
 - Alejandra Garcia (Stopfer Lab, started in Nov. 2017)





Three-minute Talks (TmT): Science Communication Training and Awards Program

- Professional training in speech development and presentation delivery
- One-on-one coaching and feedback in a supportive environment
- For finalists: A professionally produced video of your talk for NICHD promotional efforts and your own use, plus travel/training awards
- Final competition with NHGRI & NIDCR June 27th



2017 NICHD Finalists Afrouz Anderson Miranda Broadney Neda Sadeghi Arup Chakraborty Hadis Dashtestani

TmT Finalist



Miranda Broadney, M.D., M.P.H.

Senior Fellow, Section on Growth & Obesity Thrasher Research Fund Scholar

MD, MPH from Ohio State University

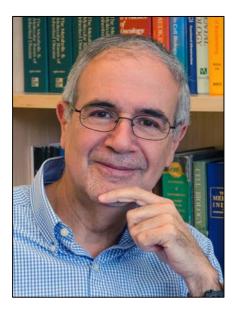
Graduate of the NIH Pediatric Endocrinology Fellowship Training Program



Thank you!



Coatopathies: Genetic Disorders of Protein Coats



Juan S. Bonifacino, Ph.D.

Associate Scientific Director, Cell Biology & Neurobiology Branch

Senior Investigator and Head, Intracellular Protein Trafficking Section