

Intellectual and Developmental Disabilities Research Center (IDDRC)



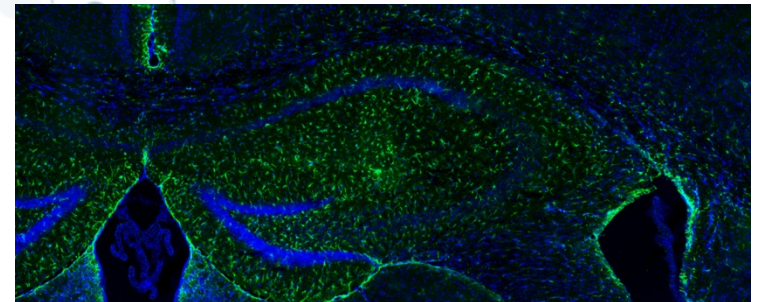
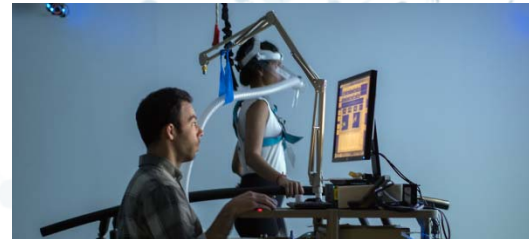
E. Mark Mahone, Ph.D., ABPP, Director
S. Ali Fatemi, M.D., M.B.A., Co-Director
Erin Jones, B.A., Project Manager

U54 HD079123

Intellectual and Developmental Disabilities Research Center (IDDRC)

- Provides research services to faculty engaged in research related to the field of IDD at little or no cost
- Funded by NICHD
- IDDRC services include:
 - Consultation and protocol development
 - Registries and recruitment
 - Access to lab and imaging facilities
 - Testing facilities
 - Statistical analyses
 - And more!
- For more info or to request IDDRC services:

www.KennedyKrieger.org/IDDRC



IDDRC at Kennedy Krieger

- Joint venture between KKI and JHU
- One of 14 national centers
- Current annual budget ~ \$800,000 direct
- Currently: 5 cores and 1 research project
- > 50 faculty/staff from KKI & JHU
- Supports ~100 studies annually
 - Produces ~100 publications annually
- National representation

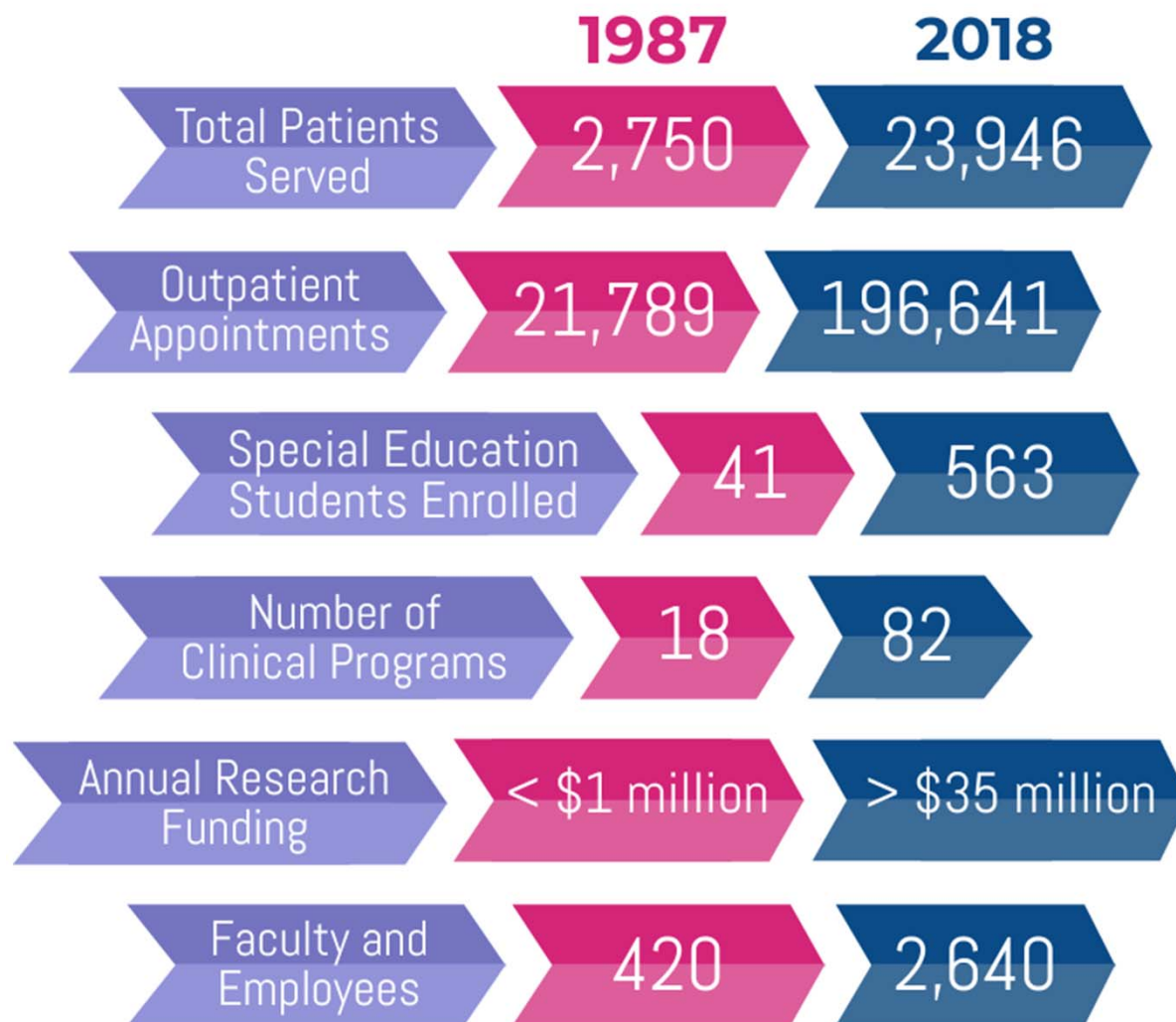
IDDRC Affiliated Programs

- Hugo Moser Research Institute
- Kennedy Krieger Clinics
- F.M. Kirby Research Center for Functional Brain Imaging
- Maryland Center for Developmental Disabilities
- IDDRC National Network
- Mid-Atlantic Consortium of IDDRCs
- The Johns Hopkins Institute for Clinical and Translational Research
- Johns Hopkins Genetic Resources Core Facility
- Eunice Kennedy Shriver National Institute on Child Health and Human Development
- Kennedy Krieger Clinical Trials Unit
- Kennedy Krieger Center for Autism and Related Disorders
- Wendy Klag Center for Autism and Developmental Disabilities
- Leadership in Neurodevelopmental and Other Related Disabilities (LEND) Program at the Kennedy Krieger Institute
- Kennedy Krieger Neuropsychology Research Lab
- Interactive Autism Network
- Association of University Centers on Disabilities
- Center for Innovation and Leadership in Special Education
- Johns Hopkins McKusick-Nathans Institute of Genetic Medicine

KKI IDDRC History

- A Research Institute was established within KKI in 1987
 - An Intellectual and Developmental Disabilities Research Center (called MRRC and MRDDRC at the time) was included as central to the Research Institute.
- Center leadership has changed approximately every 8-9 years, with former directors overlapping with new ones, thus ensuring stability and orderly succession.
 - Hugo M. Moser, M.D. (1987-1996)
 - Martha B. Denckla, M.D. (1996-2005)
 - Michael Cataldo, Ph.D. (2005-2014)
 - Wayne Silverman, Ph.D. (2014-2017)
 - Mark Mahone, Ph.D. (2017-present)

IDDRC Era: 30 Years of Progress



KKI Specialized Clinics

- Ataxia Telangiectasia Clinic
- Barth Syndrome Clinic
- Bone Disorders Program
- Center for Autism and Related Disorders
- Center for Brain Injury Recovery
- Center for Genetic Muscle Disorders
- Philip A. Keelty Center for Spina Bifida and Related Conditions
- Deafness-Related Evaluations and More (DREAM) Clinic
- Down Syndrome Clinic and Research Center
- Fetal Alcohol Spectrum Disorders Clinic
- Fragile X Clinic
- Genetic Counseling (Neurogenetics)
- Holoprosencephaly and Related Malformations Center
- Hunter Nelson Sturge-Weber Center
- Infant Neurodevelopmental Center
- Infants, Toddlers, and pre-School Years (ITSY) Clinic
- Kabuki Syndrome Clinic
- Metabolism Clinic
- Moser Center for Leukodystrophies
- NICU Clinic
- Infant Hypothermia Disorder (CHD)
- Neuropsychiatry in Epilepsy Program
- Osteogenesis Imperfecta Clinic
- Phelps Center for Cerebral Palsy and Neurodevelopmental Medicine
- Sickle Cell Neurodevelopmental Clinic
- Sleep Clinic (Neurology)
- Rett Syndrome Clinic
- Tuberous Sclerosis Clinic

2014 – Present

- The current Center has supported:

182

**Unique
Research
Projects**

129

**Principal
Investigators**

387

**Peer-
Reviewed
Publications**

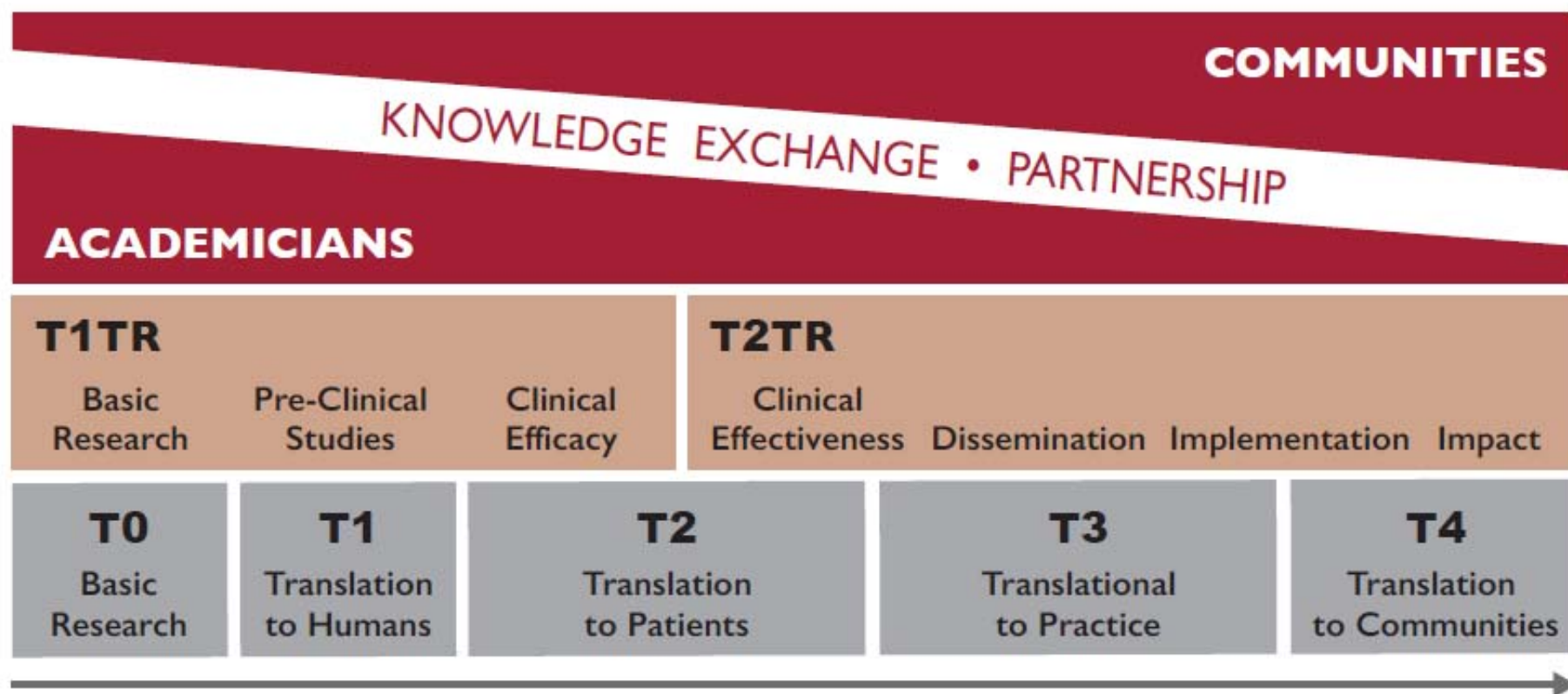
Labs-to-Clinics-to-Community

- Center is central to a scientific enterprise involving investigators working to understand and address IDD
- Nucleus of a larger program of research supported through the Hugo Moser Research Institute
 - Leveraging internationally recognized expertise and infrastructure, Center's goal is moving knowledge along the continuum from "***labs-to-clinics-to-community***"

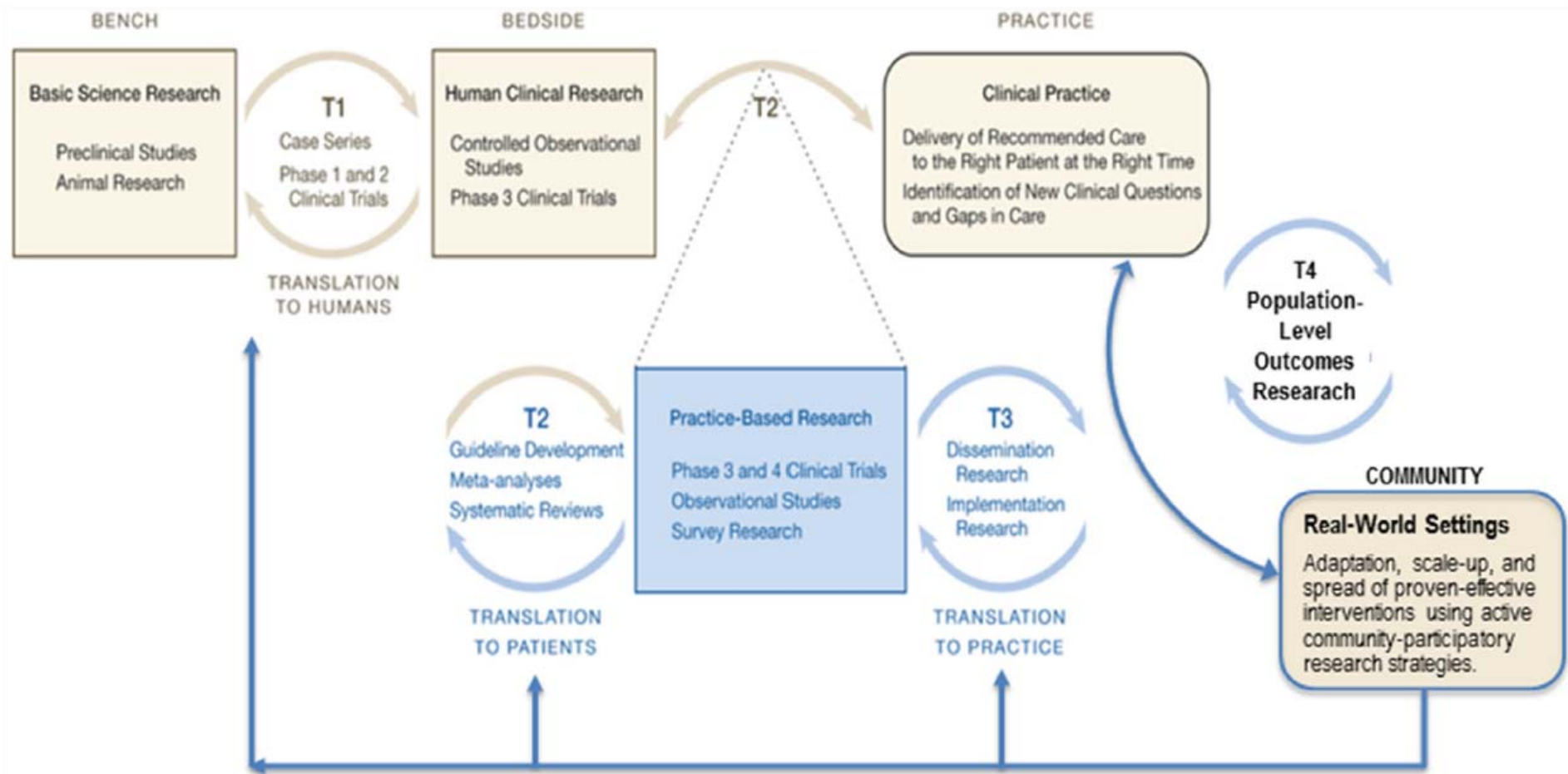
Translational Science

- Involves translation of basic scientific findings from the laboratory into potential treatments and meaningful health outcomes
- For individuals with IDD, interventions may not result in cure
 - Behavioral and cognitive challenges remain
 - Ongoing treatment settings involves schools
 - Opportunity to translate basic and clinical scientific findings in IDD to community practice (e.g., schools) and policies (local/state/national)

What is the T0 to T4 Translational Research Continuum?



Communities: The T4 Translation



Westfall, JM, & Menash, GA (2018). T4 translational moonshot: Making cardiovascular discoveries work for everyone. *Circulation Research*. DOI: 10.1161/CIRCRESAHA.117.312273

Clinical Translational Core

Co-Directors: S. Ali Fatemi, MD, MBA, Robert Findling, MD, & Sujatha Kannan, MBBS, MD

Genomics Core

Co-Directors: Jonathan Pevsner, PhD & Kimberly Doheny, PhD

Behavioral Phenotyping Core

Co-Directors: Louis Hagopian, PhD & E. Mark Mahone, PhD

Administrative Core

Co-Directors: E. Mark Mahone, PhD & S. Ali Fatemi, MD, MBA

Research Project

Co-Directors: Mary Ann Wilson, PhD & Sujatha Kannan, MBBS, MD

Neuroimaging Core

Director: Peter van Zijl, PhD



JOHNS HOPKINS
SCHOOL of MEDICINE

U54 HD079123

Intellectual & Developmental Disabilities Research Center

at Kennedy Krieger Institute and
the Johns Hopkins University School of Medicine
Director: E. Mark Mahone, PhD | Co-Director: S. Ali Fatemi, MD, MBA

KennedyKrieger.org/IDDRC

Clinical Translational Core

- Navigation to move ideas from inception to translation
 - Coordination with other IDDRC, KKI, and JHU resources
 - Registries and recruitment of research participants
- Development/implementation of clinical trials (CTU)
 - Regulatory approvals by IRB and FDA
- Consultation on biostatistics and clinical trial design
- Neuroscience facilities, including JHU Microscopy Core, brain histology and animal surgery areas
- Access to JHU Metabolomics Core
- Consultation regarding iPSC development
- Consultation with Carey School of Business faculty

Clinical Trials Unit - Examples

ASD

- **Investigational Product Mechanism:** BALOVAPTAN
- **Study Objective:** To evaluate the efficacy of 24-week treatment with balovaptan (RO5285119) compared to placebo
- **Basic Eligibility:**
 - Age 5-17 years old
 - DSM-5 criteria for ASD or ICD10 criteria for Autism diagnosis confirmed by ADOS-2 criteria
 - IQ \geq 70
- **Compensation:** \$50.00 for each completed study visit.
- **Participation** will last approximately 30 weeks and will include approximately 7 visits to KKI.

Clinical Trials Unit - Examples

ADHD

- **Investigational Device:** Device/iPAD game
- **Study Objectives:** Determine the effects of combining AKL-T01 (with AKL-X01 symptom tracking) as adjunctive treatment to stimulant medication
- **Basic Eligibility:**
 - Children ages 8-14 years inclusive at the time of consent
 - Diagnosis of ADHD according to the Diagnostic and Statistical Manual of Mental Disorders-V (DSM-V), confirmed with the Mini International Neuropsychiatric Interview for Children and Adolescents (MINI-KID)
 - Impairment Rating Scale (Parent Report) score of ≥ 3
- **Compensation:** \$125.00 for each completed study visit.
- **Participation** will last approximately 84 days and will include approximately 4 visits to KKI

Genomics Core

- Partnership with Johns Hopkins Genomics and the Institute of Genetic Medicine
 - CLIA compliant laboratory facilities
 - Clinical exome testing, whole exome sequencing (WES) and whole genome sequencing (WGS) services
 - Molecular cytogenetics and microarray
- Tissue culture and cell repository
- Bioinformatics and software development

Neuroimaging Core

- Access to resources of FM Kirby Center for Functional Brain Imaging and faculty
 - Education (courses) and technical support
 - Two 3.0T and one 7.0T scanners for humans, and 11.7T and 17.6T high resolution small animal scanners
 - Anatomic imaging (volume, shape, morphology)
 - Functional MRI (task- and non-task based)
 - Diffusion tensor imaging (DTI)
 - MR spectroscopy
 - Transcranial magnetic stimulation (TMS)

Behavioral Phenotyping Core

- Development of outcome measures for clinical trials
- Full range of deep behavioral phenotyping methods
- Examination of relationships between behavioral phenotypes and other biomarkers (genetic, epigenetic, physiological, neuroimaging)
- Quantitative study of movement and motor learning
- Development of methods for electrophysiology and ERP studies
- Comprehensive neuropsychological testing facilities
- Assistance with preparation for procedures
- Clinical registry of phenotyped patients

Research Project

- Focused on reducing brain damage in infants exposed to perinatal hypoxia-ischemia (HI), employing a mouse model to examine post-injury impacts of intervention
 - Evaluate the cellular distribution of D-NAC within microglia, astrocytes and neurons, when administered immediately after HI, at the onset of rewarming, or 24h after HI, with or without therapeutic hypothermia
 - Compare the neuroprotective efficacy achieved by hypothermia alone, D-NAC alone, and hypothermia in combination with D-NAC, in P7 mice subjected to HI

Administrative Core

- Management and coordination of Center activities
 - Website, newsletters, lectures
- Setting policies for ensuring that Core services reflect the highest standards of quality and efficiency
- Developing interactions with other Centers
- Coordinating efforts among LEND, MCDD, and AUCD
- Contributing to the training of students and early career professionals (e.g., MCDD trainings)
- Outreach, communication, and advocacy via AUCD



AUCD

ASSOCIATION OF UNIVERSITY CENTERS ON DISABILITIES

RESEARCH, EDUCATION, SERVICE

- Network of interdisciplinary centers advancing policy and practice for and with individuals with developmental and other disabilities, their families, and communities
 - UCEDD (67)
 - LEND (52)
 - IDDRC (14)
- Federal partners : MCHB, NICHD, CDC, ADD



AU C D

ASSOCIATION OF UNIVERSITY CENTERS ON DISABILITIES

R E S E A R C H , E D U C A T I O N , S E R V I C E

- Leadership on major social problems affecting all people with developmental or other disabilities or special health needs
- Advocacy with Congress and executive branch agencies that fund and regulate programs used by people with disabilities
- Networking and partnering with other national organizations to advance the network's national agendas
- Promoting communication within the network and with other groups by collecting, organizing, and disseminating data on network activities and accomplishments
- Technical assistance provision on a broad range of topics

Linking IDDRC Discoveries to Advocacy via MCDD

- U.S. Department of Labor, Office of Disability Employment Policy, Community of Practice on youth receiving SSI
- Council of Parent Attorneys and Advocates
- Maryland Community of Practice for Supporting Families
- The Maryland Disability and Health Inclusion Program
- Maryland Department of Health, DDA, Quality Advisory Council
- Maryland Developmental Disabilities Council,
- Coalition to Reform School Discipline in Maryland
- Education Advocacy Coalition for Students with Disabilities
- First Maryland Disability Trust, Inc.
- Collaborative Law Project
- The Arc of Baltimore
- Maryland Commission on Caregiving
- Maryland Respite Care Coalition

CILSE (est. 2012)

- Translate scientific discoveries in IDD into educational practice and policy
- Train future educational leaders, leveraging IDDRC, LEND, and UCEDD resources at KKI
 - Training for mid-career educators
 - Didactics
 - Interdisciplinary experiences
 - Immersion in neuroscience and behavioral research
 - Full participation in LEND activities
- Outreach, advocacy, teaching, training

Center for Innovation & Leadership in Special Education

Fellowship Program Core Content Areas



Project UNITE

(Uniting Neuroscience, Innovative Teaching & Education)

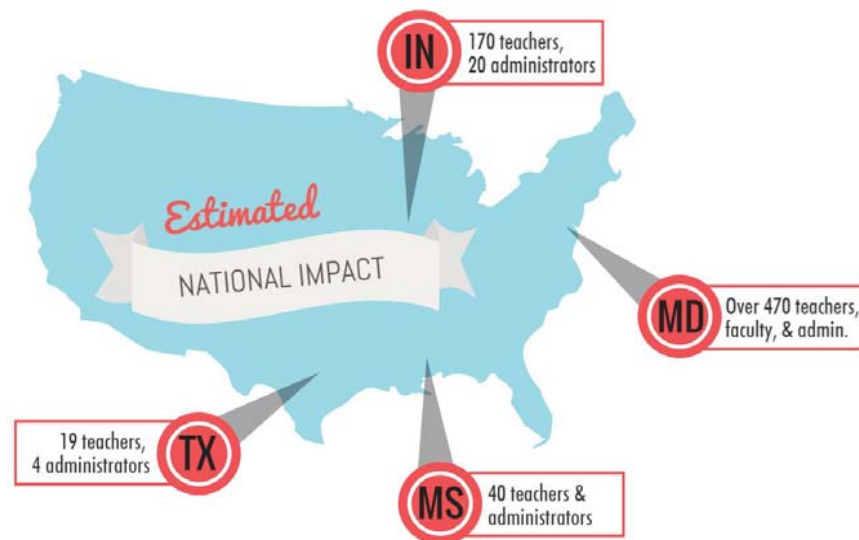


Project UNITE

(Uniting Neuroscience, Innovative Teaching & Education)

Center for Innovation & Leadership in Special Education

Professional Development and Instructional Coaching Services



- 19 graduates since 2012
- Transition to blended learning grad certificate

Teachers



83

1:1
Consultations



155

Students

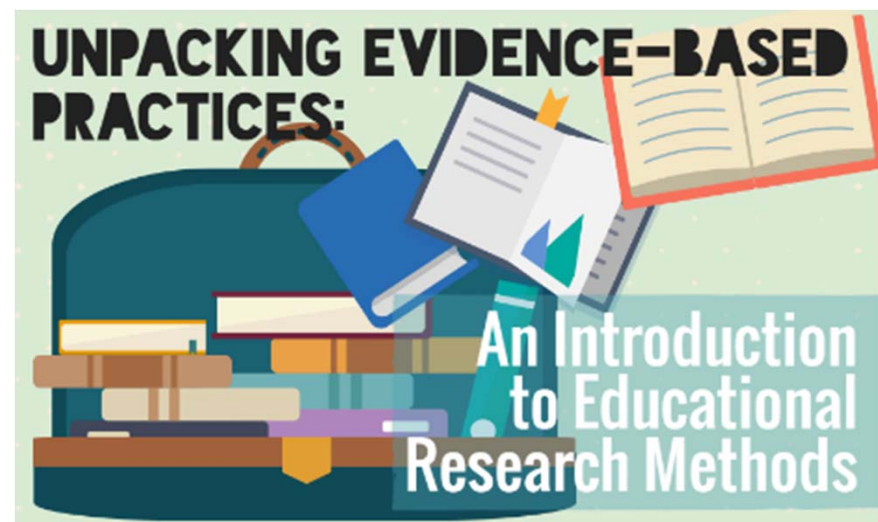
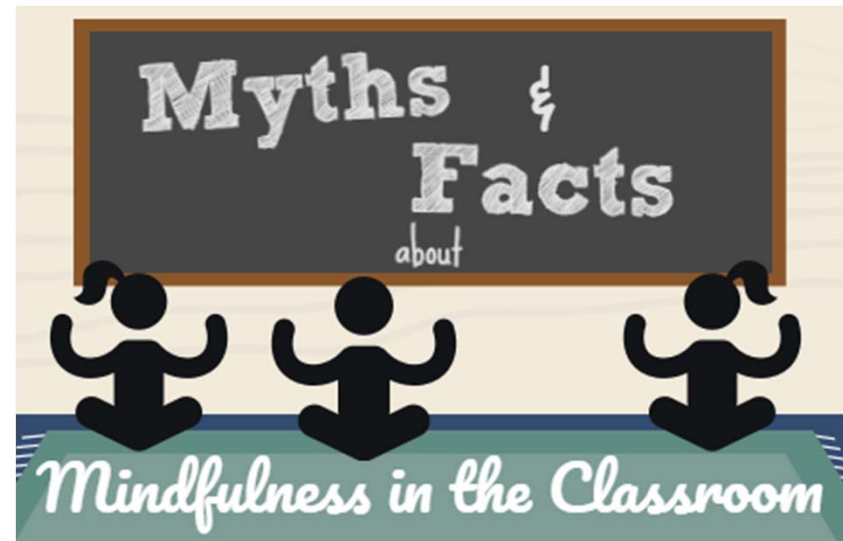


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- Teach for America
- Urban Teachers
- Towson University
- Center for Applied Special Technology (CAST)
- MD State Dept. Education

Weekly Blog

“Linking Research to Classrooms”



2019 Center News

- Competitive renewal 2020
 - Submission likely January 2020
- Bridge funding
 - June 1, 2019-May 31, 2020
- Recruitment for research project for new submission
 - Details forthcoming in April 2019
 - Embedded project 5 years
 - Leverages IDDRC Cores
 - Annual budget ~\$150-175 direct costs

IDDRC Supported Discoveries

- Diagnosis/treatment of adrenoleukodystrophy
- Discovery of the gene causing Sturge-Weber syndrome
- Novel therapeutic trials for girls with Rett syndrome
- Clarifying brain pathology in CP using DTI
- Understanding the neural basis of ASD
- Improving treatment outcomes for severe behavior disorders
- Optimizing motor learning/relearning for hemiparesis

Eligibility For IDDRC Support

- Any Kennedy Krieger Institute or Johns Hopkins University faculty member engaged in research of relevance to IDD
- To access IDDRC support, faculty must have at least one funded project that is relevant to IDD
- Graduate students and postdoctoral fellows are encouraged to access IDDRC resources through primary IDDRC investigators/mentors

What Will the IDDRC Do For You?

- Access to IDDRC Core services
- Discounted fees for Core services
- Access to news, ideas, and research related to IDD
- Access to education, funding, and advancement opportunities
- Ability to include a description of the resources, services, and expertise included in the IDDRC cores, and available to your proposed project, in grant applications
- Ability to present research and findings through IDDRC-sponsored lectures, symposia, and seminars

Investigator Resources

- NIH Biosketch
- My Bibliography
- SciENCv
- NCBI
- PubMed Central



OMB No. 0925-0001 and 0925-0002 (Rev. 10/

BIOGRAPHICAL SKETCH

Provide the following information for the Senior/key personnel and other significant contributors. Follow this format for each person. **DO NOT EXCEED FIVE PAGES.**

NAME:

eRA COMMONS USER NAME (credential, e.g., agency login):

POSITION TITLE:

EDUCATION/TRAINING *(Begin with baccalaureate or other initial professional education; include postdoctoral training and residency training if applicable. Add/delete rows as needed.)*

INSTITUTION AND LOCATION

DEGREE
(if
applicable)

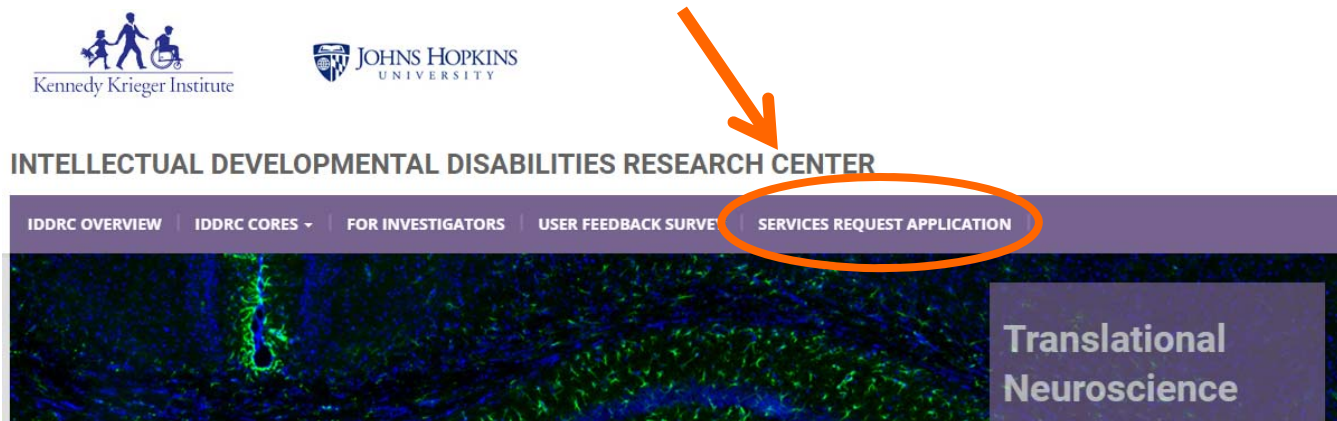
Completion
Date
MM/YYYY

Responsibilities of IDDRC Supported Investigators

- Conduct research in accordance with IDDRC mission
- Utilize proper IDDRC NIH acknowledgement (NIH U54 HD079123) for all publications and presentations benefitting from core services
- Report progress regularly on research activities, grants, and publications relating to IDDRC-supported projects
- Complete annual evaluation of IDDRC core services
- Contribute to scientific dialogue through IDDRC lectures, symposia, communications, and seminars

To Access IDDRC Services

- Contact Mark Mahone, Ali Fatemi, or Erin Jones
- Contact any of the Core Directors
- Complete a Services Request Form to request Core support
 - Available on the IDDRC website
(<https://iddrc.kennedykrieger.org>)



What We Need

- Contact us for assistance with new projects before they are submitted
- Let us know about any new funding for research (federal, foundation, donor)
- Keep us posted on new publications and discoveries
- Join the IDDRC as an affiliated faculty member