Eunice Kennedy Shriver National Institute of Child Health and Human Development and the NIH Clinical Center
NICHD and the NIH Clinical Center

Clinical Research
Clinical Care
Clinical Training
NICHD and the NIH Clinical Center

- Warren Grant Magnuson Clinical Center
- Mark O. Hatfield Clinical Research Center (2005)
  - 240 Beds (22 Pediatric)
  - 10,700 new patients
  - 5,400 inpatients
  - 100,500 outpatients
NICHD and the NIH Clinical Center

- NICHD Clinical Protocols
  - 85 Protocols
  - 36 Principal Investigators
    - 14 Senior Investigators
    - 6 Staff Clinicians
  - 122 Associate Investigators

- Endocrinology
  - Pediatric
  - Adult
  - Reproductive

- Genetics
NICHD and the NIH Clinical Center

• NICHD Patients/Subjects
NICHD and the NIH Clinical Center

- NICHD Patient/Subjects
  - Inpatient days 2449
  - Outpatient visits 5080
  - Adjusted Patient Days 4481
NICHD and the NIH Clinical Center

• Office of the Clinical Director: Research Support
  – Staff Clinicians
    • 2 Internal Medicine
    • 3 Pediatric (2 Additional Pending)
    • 2 Gynecology
  – Nurse Practitioners
    • 3 Pediatric
    • 1 Family
    • 1 Adult
  – Research Nurses/Research Coordinators (4)
NICHD and the NIH Clinical Center

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

- AIDS
- Behavioral and Social Sciences
- Dietary Supplements
- Minority Health
- Rare Diseases
- Women’s Health Projects
NICHD and the NIH Clinical Center

17 Investigators/Senior Investigators
2 Associate Clinical Investigators
3 Staff Clinicians
6 Staff Scientists
NICHD and the NIH Clinical Center

• **U01 Grants: Opportunities for Collaborative Research at the NIH Clinical Center**
  - Supports collaborative research to enhance the translation of basic biological discoveries into clinical applications that improve health
  - Extramural/Intramural/Clinical Center
  - $500,000 direct costs, 3 years (now 4 years)

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<th>LOIs/X02s</th>
<th>CYCLE 1 2013</th>
<th>CYCLE 2 2014</th>
<th>CYCLE 3 2015</th>
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<td>Resubmissions</td>
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<td>Awards</td>
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<td>Success Rate</td>
<td>20%</td>
<td>30%</td>
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### NICHD and the NIH Clinical Center

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- A Phase 1 Dose Escalation Study of Vorinostat in Niemann-Pick C1 Disease
- Birth Defects: Moebius Syndrome and Related Facial Weakness Disorders
- Antiretroviral Therapy in Aicardi Goutieres Syndrome
- Choroid Plexus-Directed Gene Therapy for Alpha-Mannosidosis
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• Weill Medical College
• Washington University
• Icahn School of Medicine
• Boston Children’s Hospital
• National Children’s Hospital
• Children’s Hospital of Philadelphia

• NHGRI
• NEI
• NINDS
• Clinical Center
NICHD and the NIH Clinical Center

• Pediatric Endocrinology Inter-Institute Training Program
  – Clinical Sites
    • NIH Clinical Center
    • Children’s National Medical Center
    • Johns Hopkins Medical Center
    • Georgetown University
  – Three fellows per year

• Inter-Institute Endocrine Training Program
  – Clinical Sites
    • NIH Clinical Center
    • Georgetown University
    • Washington Hospital Center
  – Five fellows per year (2 NICHD, 3 NIDDK)
NICHD and the NIH Clinical Center

• Reproductive Endocrinology and Infertility Training Program
  – Clinical Sites
    • NIH Clinical Center
    • Shady Grove Fertility Center
    • Walter Reed National Military Medical Center
  – Three fellows per year

• Medical Genetics Training Program
  – Program in NHGRI/Johns Hopkins
  – Faculty and Fellowship Executive Board
Section on Genetics and Endocrinology

Adrenal Tumors/Hyperplasia: “Omics Approach”
WG/ES, CGH, CNV, RNAseq, methylation

Gigantism and Acromegaly Due to Xq26 Microduplications and GPR101 Mutation

Section on Growth and Development

Exome sequencing identifies *ACAN* mutations in AD short stature

Aggreccan: Proteoglycan in cartilage matrix
- Short stature
- Advance bone age
- Joint disease

Nilsson et al. (2014) JCEM 99: E1510-18
Section on Growth and Obesity


- **RCT** of Effects of Interrupting Sedentary Behavior on Metabolic and Cognitive Outcomes in Children. JCEM 100: 3735-3743, 2015


- **RCT** to prevent depression and ameliorate insulin resistance in adolescent girls at-risk for Type 2 Diabetes. In Press, Ann Behav Med

- Niacin to suppress lipolysis in children with obesity. In press, Pediatr Obes
Section on Medical Neuroendocrinology

Pheochromocytoma and Paraganglioma

68Ga-DOTATATE PET: novel functional imaging for SDHB-related metastatic PHEO/PGLs and HNPGL

Overall detection rate 98.6%

Section on Molecular Dysmorphismology

Phase 1/2a trial of 2-hydroxypropyl-β-cyclodextrin for the treatment of Niemann-Pick Disease, type C1

- NCATS pilot project
- FDA breakthrough designation
- Multicenter, multinational Phase 2b/3 trial
Thank you
NICHD and the NIH Clinical Center