

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

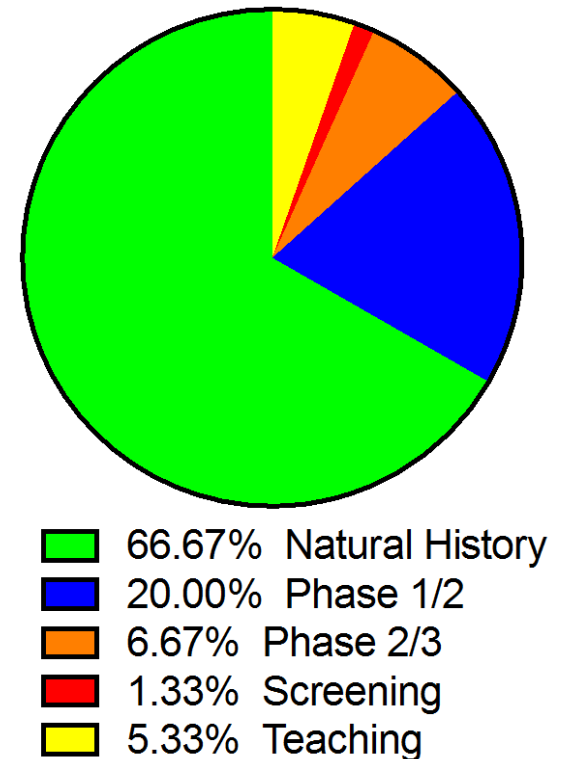
NICHD and the NIH Clinical Center

- 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

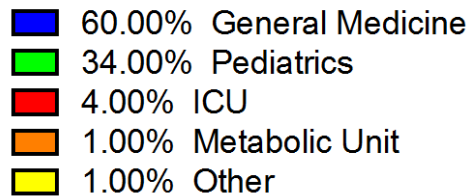
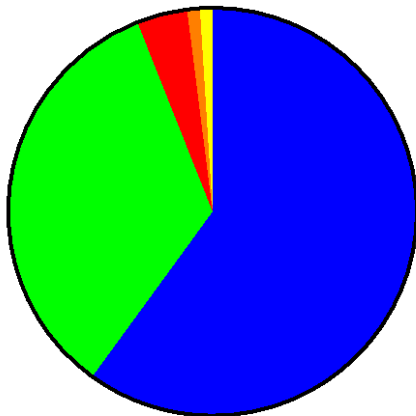
- NICHD Clinical Protocols
 - 85 Protocols
 - 36 Principal Investigators
 - 14 Senior Investigators
 - 6 Staff Clinicians
 - 122 Associate Investigators
- Endocrinology
 - Pediatric
 - Adult
 - Reproductive
- Genetics



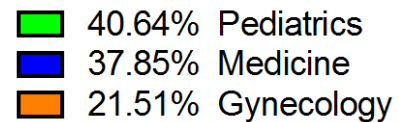
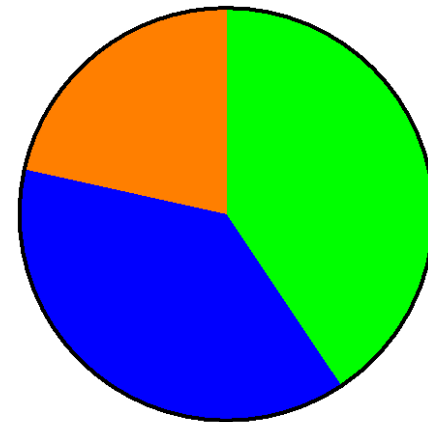
NICHD and the NIH Clinical Center

- NICHD Patients/Subjects

Inpatient



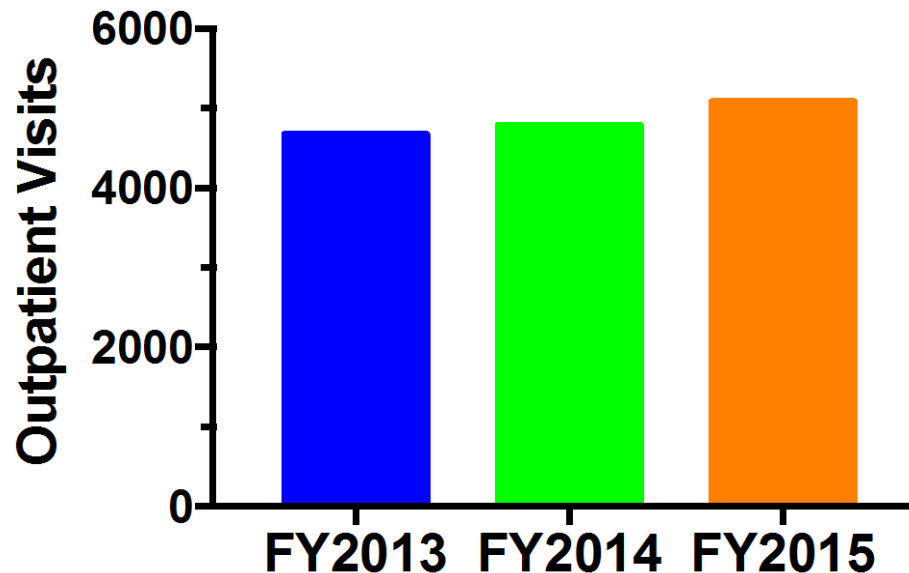
Outpatient



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)

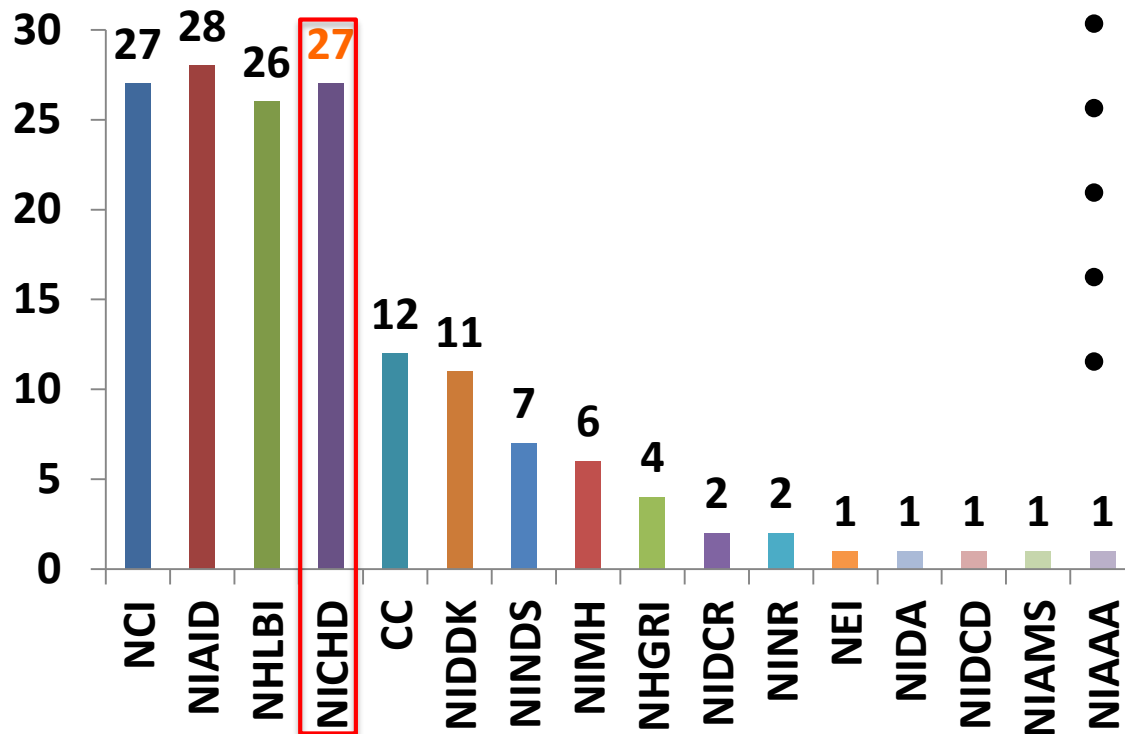
NICHD and the NIH Clinical Center

- 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)



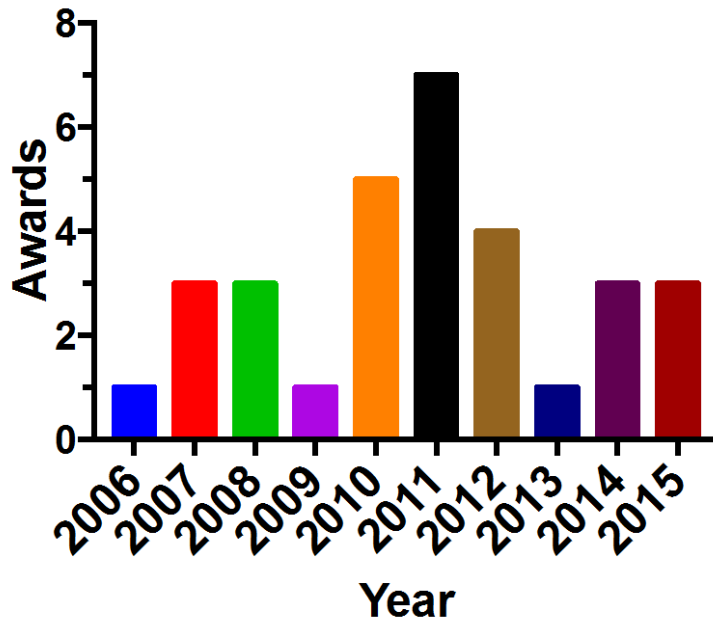
NICHD and the NIH Clinical Center

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)

LOIs/X02s	CYCLE 1 2013	CYCLE 2 2014	CYCLE 3 2015	CYCLE 4 2016
Submissions	74	44	47	54
Applications	51	33	36	40
Resubmissions	N/A	14	6	10
Awards	10	10	3*	--
Success Rate	20%	30%	8%	--

NICHD and the NIH Clinical Center

Funding IC	Cycle 1	Cycle 2	Cycle 3
NCI	3	1	1
NHLBI	1	2	1
NIAID	4	3	1
NICHD	2	2	---
NHGRI	---	1	---
NIAAA	---	1	---

- **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**

NICHD and the NIH Clinical Center

- 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

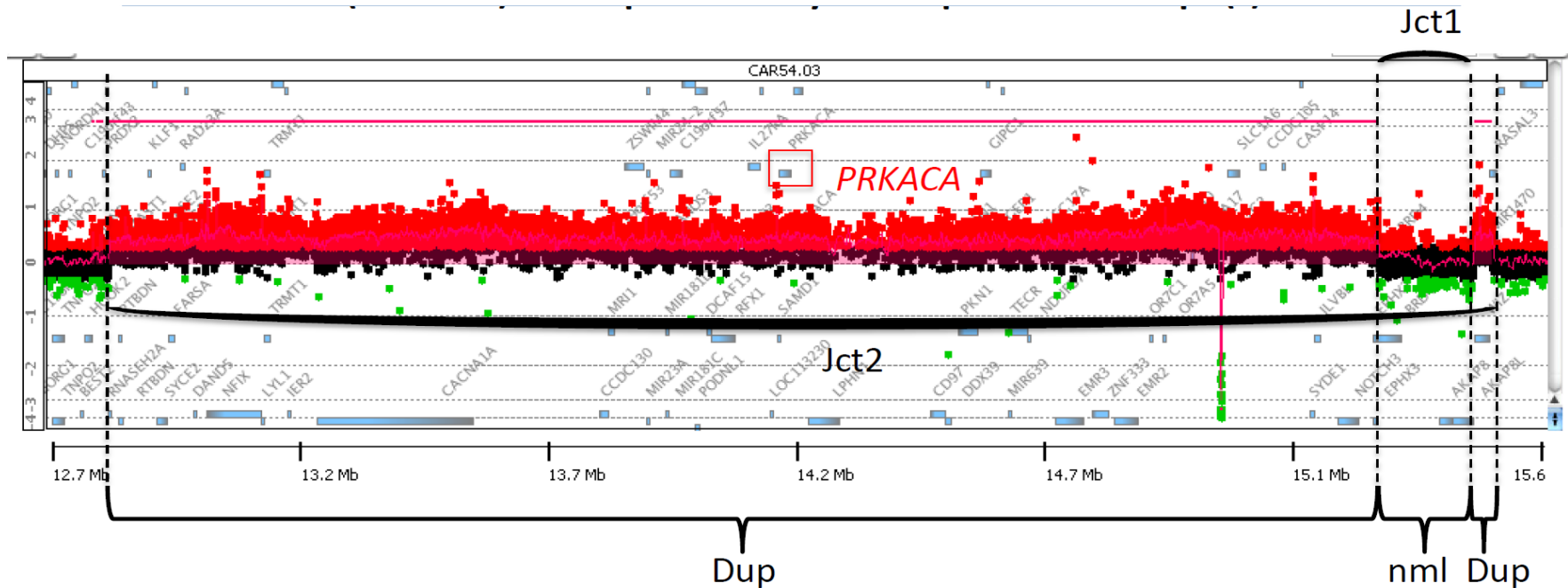
- Inter-Institute Endocrine Training Program
 - Clinical Sites
 - NIH Clinical Center
 - Georgetown University
 - Washington Hospital Center
 - Five fellows per year (2 NICHD, 3 NIDDK)

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



ARMC5 gene: *N Engl J Med.* 2013;369:2105

PRKACA gene: *N Engl J Med.* 2014;370:1019

PRKACB gene: *N Engl J Med.* 2014;370:1065

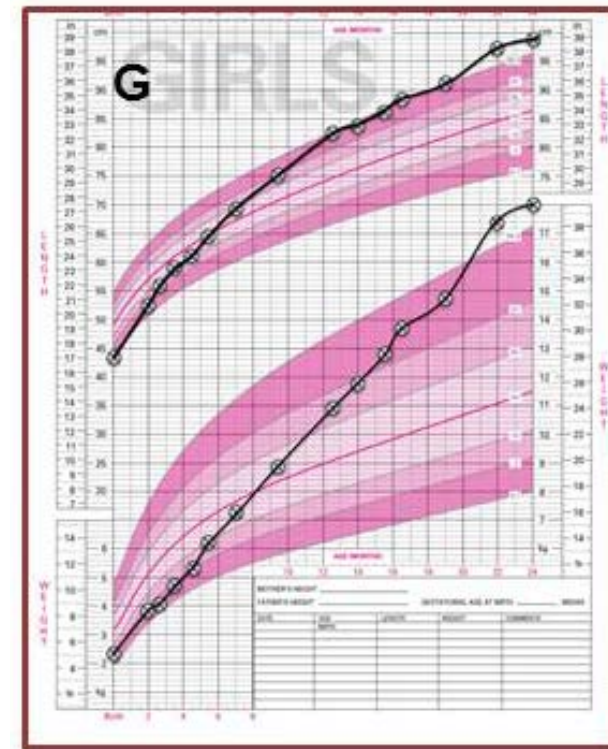
Section on Genetics and Endocrinology

The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

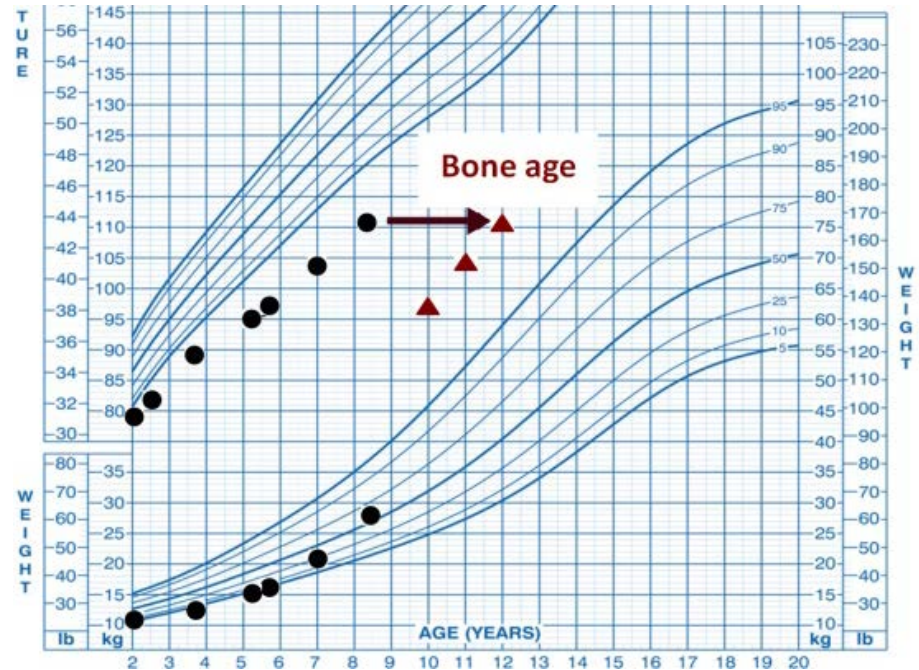
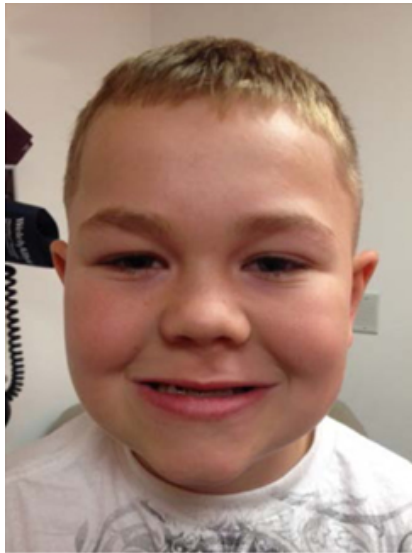
Gigantism and Acromegaly Due to Xq26 Microduplications and *GPR101* Mutation

G. Trivellin, A.F. Daly, F.R. Faucz, B. Yuan, L. Rostomyan, D.O. Larco, M.H. Scherthaner-Reiter, E. Szarek, L.F. Leal, J.-H. Caberg, E. Castermans, C. Villa, A. Dimopoulos, P. Chittiboina, P. Xekouki, N. Shah, D. Metzger, P.A. Lysy, E. Ferrante, N. Strebkova, N. Mazerkina, M.C. Zatelli, M. Lodish, A. Horvath, R. Bertollo de Alexandre, A.D. Manning, I. Levy, M.F. Keil, M. de la Luz Sierra, L. Palmeira, W. Coppieters, M. Georges, L.A. Naves, M. Jamar, V. Bours, T.J. Wu, C.S. Choong, J. Bertherat, P. Chanson, P. Kamenický, W.E. Farrell, A. Barlier, M. Quezado, I. Bjelobaba, S.S. Stojilkovic, J. Wess, S. Costanzi, P. Liu, J.R. Lupski, A. Beckers, and C.A. Stratakis



Section on Growth and Development

Exome sequencing identifies *ACAN* mutations in AD short stature



Aggrecan: Proteoglycan in cartilage matrix

Short stature

Advanced bone age

Joint disease

Nilsson et al. (2014) JCEM 99: E1510-18

Section on Growth and Obesity

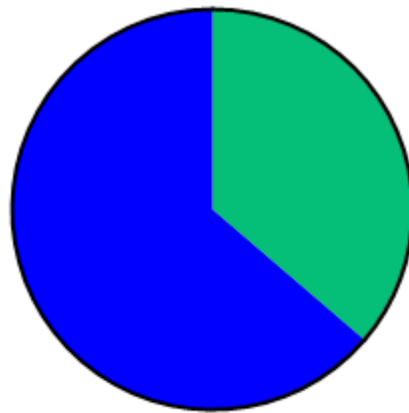
- **RCT** of effects of a low glycemic load or a low-fat dietary intervention on body weight in obese Hispanic American children and adolescents. Am J Clin Nutr 97(2): 276-85, 2013
- **RCT** of Effects of Interrupting Sedentary Behavior on Metabolic and Cognitive Outcomes in Children. JCEM 100: 3735-3743, 2015
- **RCT** of effects of metformin on body weight, food Intake and satiety in obese children. Diabetes 60:477-85, 2011; Diabetes Obes Metab 17:363-370, 2015
- **RCT** for excess weight gain prevention in adolescents: one and three-year outcomes. Am J Clin Nutr 100: 1010-1018, 2014; Int J Eat Disord 49: 490-498, 2016;
In Press, J Consult Clin Psych
- **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

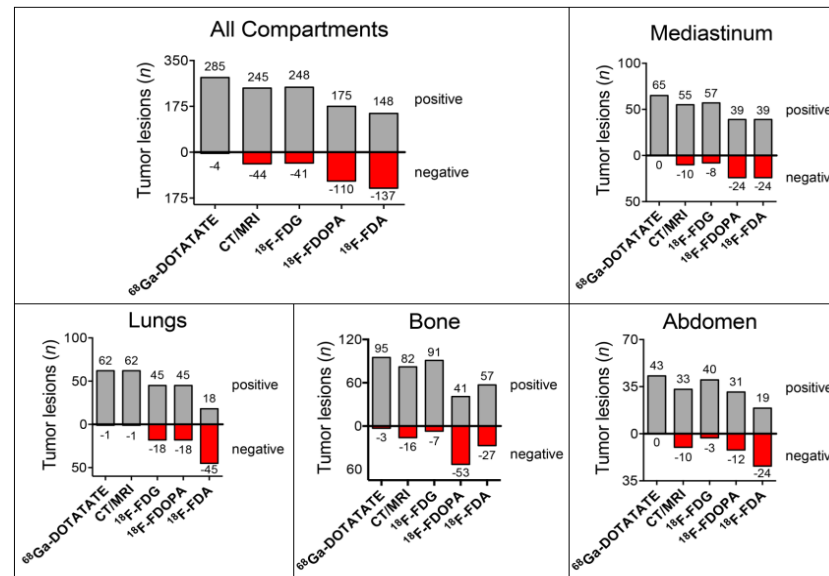
Pheochromocytoma and Paraganglioma

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

Patient Visits
2011-Present



819 New Subjects
1438 Follow-up Visits

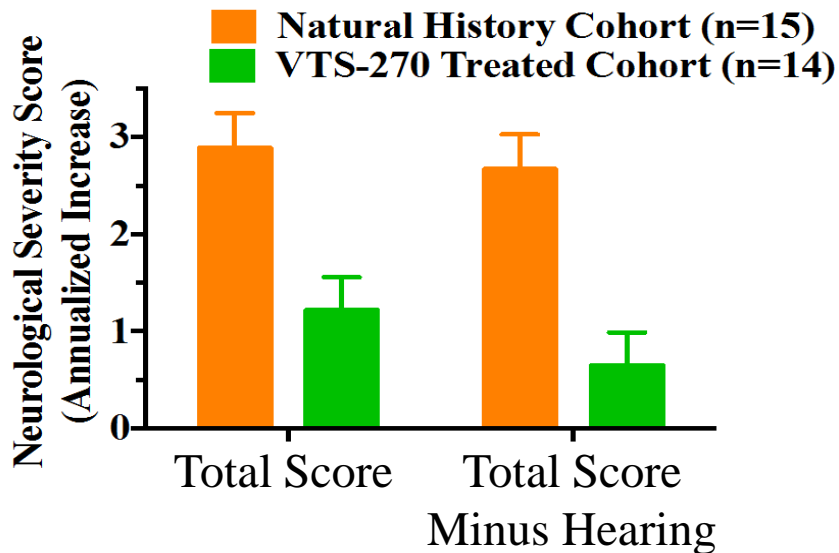


Overall
detection
rate
98.6%

Janssen et al. Clin. Cancer Res. 2015; 21:3888

Section on Molecular Dysmorphology

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

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