Pediatrics Epidemiology Center

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Mission

- To enrich, enhance, and facilitate the University's overall mission to advance scientific knowledge through clinical research, and translate that research into improved patient care and health outcomes.
- To enhance training and career development activities in clinical investigation and health services research.
- To provide guidance to investigators on research study design, statistical methodology, data analysis, and data interpretation.
- To coordinate multi center clinical studies in compliance with Federal regulatory requirements.
Vision

To enhance clinical research through the application of state of the art technology.
Faculty and Staff

- M. Abbondondo
- T. Adams
- C. Aguado
- J. Anderson
- F. Badias
- M. Bassi
- J. Bayer
- C. Bray
- B. Calkins
- M. Clark
- M. Couluris
- D. Cuthbertson
- C. Foster
- B. Gainer

- D. Garcia
- J. Gomes
- V. Gowda
- M. Gross-King
- H. Guillette
- M. Hastings
- A. Hildoer
- V. Huegel
- J. Krischer
- S. Liu
- J. Lloyd
- J. Malloy
- C. McCarthy
- W. McLeod

- S. Moyers
- L. Nallamshetty
- R. Richesson
- B. Patterson
- K. Paulus
- P. Ruiz
- L. Shanker
- S. Smith
- S. Terkoski
- J. Tran
- E. Walker-Veras
- K. Young
- P. Xu
Expertise Represented

- Biostatistics
- Epidemiology
- Health Informatics
- Computer Science
- Genetics
- Nutrition
- Public Health
- Nursing
- Pediatrics
Research Agenda

- Prevention of T1 Diabetes
- Epidemiology of T1 Diabetes
- Clinical Trials in Rare Diseases (a disease or condition affecting fewer than 200,000 persons in the United States)
- Trials to improve the quality of life in cancer patients.
- Cancer chemo-prevention studies.
- Trials in Cystic Fibrosis.
The primary objective of this multi-center, multi-national, epidemiological study is the identification of infectious agents, dietary factors, or other environmental exposures that are associated with increased risk of autoimmunity and T1DM.

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The Environmental Determinants of Diabetes in the Young

- Screen 200,000 newborns for high risk HLA
- Enroll 7,000 and follow for 15 years
- Investigate gene-environment factors leading to autoimmunity and T1 Diabetes.
Trial to Reduce IDDM in the Genetically at Risk

Hypothesis is that weaning to an extensively hydrolyzed infant formula will decrease the incidence of type 1 diabetes, as it does in animal models for the disease.

NICHD: U01 HD051997
Trial to Reduce IDDM in the Genetically at Risk

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Trial to Reduce IDDM in the Genetically at Risk

- Randomize 2,000 high risk first-degree relative of T1D probands.
- Follow for 15 years.
The primary goal is to conduct cancer prevention and control studies in community settings.
Moffitt Community Clinical Oncology Program Research Base

- Stress management interventions.
- Preventing cancer cachexia.
- Prevention of ovarian failure secondary to chemotherapy.
- Prevention of vincristine associated neurotoxicity.
- Treatment of pulmonary fibrosis secondary to cancer therapy.
Consortia:
1. Channelopathies
2. Vasculitis
3. Genetic Steroid Disorders
4. Bone Marrow Failure
5. Urea Cycles Disorders
6. Rare Lung Diseases
7. Thrombotic Disorders
8. Genetic Diseases of Mucociliary Clearance
9. Angelman, Rett, PW Syndromes
10. Rare Liver Diseases

NCRR: RR019259
Forty One Rare Diseases are Currently Under Study in the RDCRN

| Alpha-1 Antitrypsin Deficiency | Hereditary Interstitial Lung Disease |
| Amegakaryocytic Thrombocytopenic Purpura | Large Granular Lymphocyte Leukemia |
| Andersen-Tawil Syndrome | Lymphangioleiomyomatosis |
| Androgen Receptor Defects | Microscopic Polyangiitis |
| Angelman's Syndrome | Myelodysplastic Syndromes |
| Antiphospholipid Antibody Syndromes | N-Acetylglutamate Synthase Deficiency |
| Aplastic Anemia | Non-dystrophic Myotonic Disorders |
| Apparent Mineralocorticoid Excess | Ornithine Transcarbamylase Deficiency |
| Arginase Deficiency | Ornithine Translocase Deficiency Syndrome |
| Argininosuccinate Lyase Deficiency | Paroxysmal Nocturnal Hemoglobinuria |
| Argininosuccinate Synthetase Deficiency | Polyarteritis Nodosa |
| Autoimmune Neutropenia | Prader-Willi Syndrome |
| Carbamyl Phosphate Synthetase Deficiency | Primary Ciliary Dyskinesia |
| Catastrophic Antiphospholipid Ab Syndrome | Pseudohypoaldosteronism |
| Churg-Strauss Syndrome | Pulmonary Alveolar Proteinosis |
| Citrin Deficiency | Pure Red Cell Aplasia |
| Congenital Adrenal Hyperplasia | Rett Syndrome |
| Cystic Fibrosis | Takayasu's Arteritis |
| Episodic Ataxias | Thrombotic Thrombocytopenic Purpura |
| Giant Cell Arteritis | Wegener’s Granulomatosis |
| Heparin-induced Thrombocytopenia | |
RDCRN Enrollment Sites

- Toronto, Canada
- Paris, France
- Quebec, Canada
- Sao Paulo, Brazil
- Bad Bramstedt, Germany
- Groningen, Netherlands
- Melbourne
- Tokyo
- Lyon, France
- London, UK
- Edinburgh, UK
- Cambridge, UK
Rare Diseases Clinical Research Network

- Urea cycle natural history and intervention studies.
- Angelman, Rett, Prader-Willi epidemiology studies.
- NDM and periodic paralysis natural history and intervention studies.
- The vasculitides natural history and outcomes.
- Bone marrow failures epidemiology.
- Studies of mucociliary clearance.
Cystic Fibrosis TDN

- A Multi-center, Multi-national, Randomized, Placebo-Controlled Trial of Azithromycin in Participants with Cystic Fibrosis 6-18 years old, Culture Negative for *Pseudomonas aeruginosa*
Other Projects

- Vasculitis Outcomes Measures: Development and Validation (JHU R01 AR051874)
- TrialNet (UM U01 DK061041)
- Advanced Cancer Detection Center (USF DAMD 170120056)
- Neurofibromatosis (Moffitt Foundation)
- Targeted Drug Therapy for LGL Leukemia (Moffitt R01 CA112112)
Annual Funding

- NIDDK: 12.5
- NICHD: 0.3
- NCI: 2
- NCRR: 0.25
- DoD: 0.5
- Other: 3
Common threads

- Multi-center, multi-national, multi-disciplinary studies.
- Web-based data collection.
- Management of large, diverse data bases.
- Clinical Trials.
- Epidemiology studies.
Common threads (continued)

- Live webcasts
- Internet-based videoconferencing
- Video libraries
- Electronic data exchange with labs and remote sites
- Interactive voice response systems for registration/randomization
Other Characteristics

- Multi-disciplinary, multi-college: Medicine, Nursing, Engineering
- International in scope: clinical sites in 20 countries
- Industry experience: studies include industry involvement
Strategic Plan

- **Short term:**
  - Expansion: contracts, grants, personnel
  - FDA certification
  - Disaster recovery: web connectivity
  - Develop licensing structure for IP

- **Long Term:**
  - Provide for secure base of operations and long term support.