

RESEARCH AND DISCOVERY IN THE EMORY-CHILDREN'S PEDIATRIC RESEARCH CENTER

And... we have a name!

We are very happy to announce the name of the joint venture between Children's and Emory to build a top research enterprise. We are now officially the **Emory-Children's Pediatric Research Center!** This name formally signifies our partnership to grow basic, translational, and clinical research that will benefit the lives of children. Look for this name on the new website that will be released shortly to highlight the research efforts of the Emory DOP, Children's medical staff, and partners!

Pediatric Research Retreat a Success!



Children's Healthcare of Atlanta and the Emory University Department of Pediatrics held the first Pediatric Scientific Research Retreat at the Loudermilk Conference Center in Atlanta this past month. The event was attended by over 200 scientists from Children's, Emory University, Georgia Tech, Morehouse School of Medicine and the Medical College of Georgia.

The day began with a presentation of the research strategic plan followed by brief presentations on the Pediatric Research Centers by the Center Directors. Scientific presentations included Dr. Nael McCarty and Dr. Michael Schechter on the Center for Cystic Fibrosis Research, Dr. Parmi Suchdev on global health and nutrition, Dr. Vyas-Read on lung injury, Dr. Bill Woods

on the Aflac Cancer Center and Dr. Todd Cooper on new leukemia therapies. The lunch hour included three roundtable discussions on



Genetics, Mucosal Immunology, and Nanotechnology along with a scientific poster session with 27 posters from a wide array of disciplines including basic, translational and clinical research. The day was concluded by a presentation of the plans for the new Woodruff Health Sciences Building and a panel discussion led by Dr. Barbara Stoll, Dr. Paul Spearman, Beth Howell, Dr. Beatrice Gee (Morehouse SOM), Dr. Steve Cross (Georgia Tech) and Dr. Bernard Maria (MCG).

The day was filled with opportunities to build new collaborations with other researchers both from within Emory and from other major institutions!

We're looking for two faculty leaders to take the lead in organizing the event next year. With a seasoned staff of program coordinators from the research centers to assist in the logistics

Congratulations to Dr. Raymond Schinazi!

Earlier this month it was announced that a new study found that Emory University is the fourth largest contributor in the nation to the discovery of new drugs and vaccines by public-sector research institutions. The research was published in the Feb. 10 issue of the *New England Journal of Medicine*.



The researchers identified 153 FDA-approved drugs and vaccines that were discovered at least in part by

public-sector research institutions during the past 40 years. The top five contributors were the NIH (22), the University of California System (11), Memorial Sloan-Kettering (8), Emory University (7), and Yale University (6).

The seven Emory products named included HIV/AIDS drugs lamivudine (3TC) and emtricitabine (FTC), discovered by Emory scientists **Raymond Schinazi, PhD, DSc**, Dennis Liotta, PhD, and their former colleague Woo-Baeg Choi, PhD. These two drugs are among the most commonly used and most successful HIV/AIDS drugs in the world, taken in some form by more than 94 percent of U.S. patients on therapy and by thousands more globally. Lamivudine is included in the drugs Eпивir, Combivir, Epzicom, and Trizivir, manufactured by GlaxoSmithKline. Emtricitabine is included in the drugs Emtriva, Atripla, and Truvada, manufactured by Gilead Pharmaceuticals.

But interestingly enough, **there have actually been 9 new drug applications at Emory, rather than just the 7 cited in the article, which would place Emory at number 3 in contributions.** The 9 include:

- FTC (Emtriva)
- 3TC (Lamivudine)
- D4T (Stavudine)
- LdT (Telbivudine)
- Truvada (FTC/TDF)
- Atripla (FTC/TDF/EFV)
- Combivir (3TC/AZT)
- Trizivir (3TC/AZT/ABC)
- Epzicom (3TC/ABC)

Dr. Schinazi noted that Emory is really # 1 in terms of drug innovators as a university since NIH (ranked #1) is composed of several institutes with billions in R&D research funds; UC system (#2) is a huge system and not a single university; and Memorial Sloan Kettering (#3) is a private Cancer Center. Nevertheless, we are pleased to be listed among these outstanding giant institutions.

Dr. Schinazi spoke at the research retreat regarding the **Center for Drug Discovery**, which is currently housed in his lab area at the VA, but which will have a significantly larger presence in the new research building adjacent to ECC when it comes on line. This will be an exciting opportunity for continued growth and discovery in this crucial area of research.

REMINDER to those PIs involved in clinical trials:

Specific training on “Key Concepts in Clinical Trial Research” are now required and must be completed by June 1, 2011. The modules can be found at Emory Learning Management System website: www.emory.edu/elms-training.

Recipient of the Crooks Scholarship



Dr. Parmi Suchdev is the recipient of a Crooks Scholarship to support his efforts in spearheading a global health program for the Emory Pediatrics Residency, and in recognition of his research in global nutrition.

Dr. Suchdev founded an organization in 2008 to increase awareness of global health issues in the Emory community (<http://sites.google.com/site/emoryghope/>) and has been working with colleagues in the Department to develop a sustainable global health curriculum for Pediatrics residents. In 2010, a series of lectures in global health were integrated into the core curriculum, and local and international sites are currently being established to provide field experiences in clinical care and research. Dr. Suchdev will direct the global health track, which will be formally launched for the incoming intern class starting in 2011-2012.

Dr. Suchdev's research interests are in micronutrient malnutrition. He has been the principal investigator of a CDC-supported longitudinal study assessing the effectiveness of micronutrient "Sprinkles" in Western Kenya since 2006. This study found that selling Sprinkles through community vendors was effective in reducing anemia, iron deficiency, and vitamin A deficiency in children less than 5 years of age.

Funds for the Crooks Scholarships were provided by Drs. R. Schinazi and D. Liotta in honor of Joseph W. Crooks, Senior VP and General Counsel of Emory University from 1981 to 1999 when he suddenly died.

Congratulations to Dr. Suchdev!

EECRC 2011 Seed Grant Recipients

Dr. Ron Joyner retired as of December 31, 2010, but the EECRC reins have been passed to a new Scientific Director – Dr. Nael McCarty. Dr. Joyner's closing effort was to award the 2011 EECRC Seed Grant recipients. Each recipient was chosen through a peer-review evaluation of proposals and receives \$50,000 to support the stated research project. The recipients include:

Dr. Jason Hansen, Pediatrics - PACS: "Extracellular Redox Regulation of Stem Cell Differentiation"

Dr. Jeanne Hendrickson, Pediatrics - HemOnc: "Development and characterization of a novel mouse model containing an authentic human RBC antigen, with implications for RBC alloimmunization and hemolytic disease of the fetus and newborn"

Dr. Paul Kirshbom, Surgery - Cardiovascular:
"Development of a pulmonary insufficiency model in the rat"

Dr. Lisa Kobrynski, Pediatrics - PACS: "Development of novel assays for newborn screening for 22q11 deletion syndrome"

Dr. Nancy Louis, Pediatrics - Neonatology:
"Contributions of TPF-2 to innate immune cell function in developing murine intestine"

Dr. Tracy-Ann Read, Surgery – Neurosurgery:
"Characterization and targeting of cancer stem cells in medulloblastoma"

Dr. Cary Sauer, Pediatrics – GI: "Magnetic resonance enterography (MRE) utility in Crohn disease activity measurement, confirmation of clinical remission and planning of surgical treatment"

Dr. Elizabeth Wright, Pediatrics – ID: "Determining the factors that regulate respiratory syncytial virus assembly and ultrastructure by cryo-electron tomography"

Congratulations again to all!

"Neither rain, nor sleet, nor snow, nor hail shall keep the RETAIN Study Team from their appointed rounds."



The Emory-Children's team for the national RETAIN Study on Type-1 Diabetes received recognition from their national sponsor, the Immune Tolerance Network for blazing the trail and enrolling the first RETAIN participant, screening the first 3 patients amidst adversity, and going the extra mile for their patients. The team is led by **Drs. Mark Rigby and Eric Felner**, and includes **Drs. Milton Brown and Greg Smallwood**, plus **Nick Raviele, Stephanie Meissner, and Rita Willis**.

In late December, the Emory RETAIN staff had been in contact with a very interested and motivated newly

diagnosed patient who had heard about the RETAIN trial and was eager to screen for the trial. So much so, that the Colorado-based young man was willing to forgo his winter college semester in hopes of being eligible to participate in RETAIN. The patient was referred to the Emory/Children's staff where the patient had friends and family in the area.



The patient was scheduled to be screened in early January; however, as we all recall, Atlanta got hit with a major snow and ice storm. The study team didn't let that stop them, with Dr. Brown actually walking 4 miles in the snow to the clinic to manage the processing and shipping of samples! The great news is that the patient was found eligible to enroll, completed the first Aralast NP infusion and became the first study participant to enroll in the RETAIN trial!

New Extramural Funding Received to date in FY11

Cumulative total through January 31, 2011:
\$7,189,092 DIRECT COSTS
\$9,380,944 TOTAL COSTS

David Archer, PhD, HemOnc: GEORGIA TECH, "Establishing a novel gene correction method for treating sickle cell disease".

Donald Batisky, MD, Nephrology: UNIVERSITY OF ROCHESTER, "Executive Function in Pediatric Hypertension".

Clark Brown, MD, HemOnc: ST JUDE CHILDRENS RESEARCH HOSPITAL, "TCD with Transfusions changing to Hydroxyurea (TWITCH)"

Kevin Bunting, PhD, HemOnc: CHOA/CURE, "Targeting Aberrant STAT5-Mediated Signaling in Myeloid Leukemias".

Andres Camacho-Gonzalez, MD, ID: BRISTOL MYERS SQUIBB, "Proximal Renal Tubular Dysfunction from HIV infection and Therapy in Children...".

Robert Castellino, MD, HemOnc: CHOA/CURE, "Novel Therapies for Medulloblastoma: Promoting p53-Mediated Pathways to Cell Death".

Todd Cooper, MD, HemOnc: CHILDRENS HOSPITAL OF LOS ANGELES, "Phase I trial of NECTAR -TACL 08-02".

Todd Cooper, MD, HemOnc: BRISTOL MYERS SQUIBB, "A Phase II Study of Dasatinib Therapy in Children and Adolescents with Newly Diagnosed...".

Carlton Dampier, MD, HemOnc: UNIVERSITY OF NORTH CAROLINA, "Longitudinal validation of PROMIS Pediatrics item banks".

Eric Felner MD, Endocrinology: GENETECH, "Insulin Coated Microneedles for Bolus Insulin Delivery to the Diabetic Rat".

Eric Felner MD, Endocrinology: UCSF, "ITN041A: EFFECT OF ANTITHYMOCYTE GLOBULIN ON PRESERVING BETA-CELL...".

Eric Felner MD, Endocrinology and Mark Rigby, Critical Care: UCSF, "ITN Consortium: The ITN-JDRF Partnership for Innovative Clinical Trials in Type 1 Diabetes".

Robert Geller, MD, Gen Peds – HSCH: ASSOC OF OCCUPATIONAL AND ENV CLINICS, "Pediatric Environmental Health Speciality Unit (PEHSU)".

Kelly Goldsmith, MD, HemOnc: CHOA/CURE, "BH3 Death Domain Peptides in Neuroblastoma".

Larry Greenbaum, MD, Nephrology: UNIVERSITY OF MICHIGAN, "PROMIS II: Validation of PROMIS Pediatric Banks with Incident Nephrotic Syndrome".

Lu Bing Gu, PhD, HemOnc: CHOA/CURE, "Identification of a signal pathway mediated by MDM2 and MYCN:...".

Ann Haight, MD, HemOnc: NATL CHILDHOOD CANCER FOUNDATION, "BMT CTN 0801".

Ann Haight, MD, HemOnc: NATL CHILDHOOD CANCER FOUNDATION, "BMT CTN 0802".

Shabnam Jain, MD, Emergency Medicine: ATLANTA RESEARCH AND EDUCATION FDTN, "Pneumococcal Carriage Phase II".

Shabnam Jain, MD, Emergency Medicine: MOREHOUSE SCHOOL OF MEDICINE, "Assessment of the effectiveness of Rotarix vaccine under routine use in Georgia".

Pradip Kamat, MD, Critical Care: PENNSYLVANIA STATE UNIVERSITY, "Phase III Trial of Calfactant for ALI in Pediatric Leukemia and HSCT Patients".

Howard Katzenstein, MD, HemOnc: CHILDRENS HOSPITAL OF LOS ANGELES, "NANT 2007-2".

Howard Katzenstein, MD, HemOnc: CHILDRENS HOSPITAL OF LOS ANGELES, "NANT 2007-3".

Christine Kempton, MD, HemOnc: UNIVERSITY OF NORTH CAROLINA CHAPEL HILL, "DVT/Surgery Study: An Observational Study of Postoperative Deep Venous Thrombosis".

Harry Keyserling, MD, ID: CDC, "Anthrax Vaccine Research Program (AVRP) Records and FDA Audit Requirements".

Lisa Kobrynski, MD, PACS: BAXTER HEALTHCARE CORP, "Baxter 902: LONG-TERM TOLERABILITY AND SAFETY OF IMMUNE GLOBULIN SUBCUTANEOUS (IGS...".

Subra Kugathasan, MD, GI: CONNECTICUT CHILDREN'S HOSPITAL, "Pediatric Inflammatory Bowel Disease Collaborative Group Registry".

Tobey MacDonald, MD, HemOnc: NIH R01, "The Role of PDGFR in Medulloblastoma Progression".

Tobey MacDonald, MD, HemOnc: OSI PHARMACEUTICALS, "A randomized phase 2 study of single-agent erlotinib versus oral etoposide...".

Tobey MacDonald, MD, HemOnc: CHOA/CURE, "Pre-clinical testing of dasatinib in pediatric medulloblastoma".

Tobey MacDonald, MD, HemOnc: Georgia Tech, "Exvading Invasive Pediatric Brain Tumors".

Gregory Melikian, PhD, ID: NIH R01, "Biophysics of Protein-Mediated Membrane Fusion".

Gregory Melikian, PhD, ID: NIH R21, "Kinetic Determinants of HIV Neutralization".

Gregory Melikian, PhD, ID: NIH R21, "Functional Characterization of the Hepatitis C Virus E1-E2 Glycoproteins".

Sowya Mohan, MD, NIH F32, "Fatty Acid Ethyl Esters in Developing Lung".

Martin Moore, PhD, ID: ZETRA BIOLOGICS, "Virus-like particle (VLP) vaccines against respiratory syncytial virus (RSV)".

Andrew Muir, MD, Endocrinology: UNIV SOUTH FLORIDA and UNIV OF FLORIDA, "Natural History Study of Type 1 Diabetes".

Ifeyinwa Osunkwo, MD, HemOnc: EMMAUS MEDICAL, "A Phase III, Prospective, Randomized, Double-Blind, Placebo-Controlled, Parallel-G...".

Matthew Paden, MD, Critical Care: ABT ASSOCIATES, "Abt Influenza Effectiveness in High Risk Children-...".

Zhiqi Pan, PhD, HemOnc: NIH R03, "NUCLEOSIDES AND TREM2 MEDIATED INFLAMMATION".

Sampath Prahalad, MD, Rheumatology: DUKE UNIVERSITY, "CARRA REGISTRY: Childhood Arthritis and Research Alliance Network".

Michael Schechter, MD, PACS: CYSTIC FIBROSIS FOUNDATION, "Application for CFF Accreditation and Funding 2010/2011" (renewal).

Michael Schechter, MD, PACS: VERTEX PHARMACEUTICALS, "Vertex VX09-809-102".

Michael Schechter, MD, PACS: VERTEX PHARMACEUTICALS, "Vertex 770-105".

Hal Simon, MD, Emergency Medicine: UNIVERSITY OF MICHIGAN, "Progesterone for Traumatic Brain Injury in Children: Planning a Safety and Efficacy...".

Paul Spearman, MD, ID: NIH R01, "Role of Vpu in Retroviral Particle Assembly" (renewal).

Trent Spencer, PhD, HemOnc: CHOA, "Co-administering Drug Resistant Immune Cells with Chemotherapeutic Drugs...".

Robert Vincent, MD, Sibley: AGA MEDICAL, "ASD PMS II".

Miriam Vos, MD, GI: Cytonet GmbH & Co. KG, "CCDO5: Open Prospective, Historic Controlled, Multicentre Study to Evaluate...".

Zenqhi Wang, PhD, HemOnc: CHOA/CURE, "MicroRNAs as targets for the treatment of STAT5-driven myeloid leukemias"

Elizabeth Wright, PhD, ID: UNIVERSITY OF GENEVA, "Viral Docking and Maturation in Whole Bacterial Cells at Near Atomic Resolution...".

James Zimring, MD, HemOnc: NIH R01, "Platelet Transfusion Induced Transplant Rejection Across mHA barriers".

Muxiang Zhou, PhD, HemOnc: CHOA/CURE, "Targeting of MDM2 and XIAP for Therapeutic Drug Discovery in Pediatric Leukemia".