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A. Nichole


Duke


Paul


Paul


Greg
Gibson


Greg
Gibson
Georgia Institute of Technology


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Sickle Cell Disease (SCD)
SCD afflicts ~1 in 5,000 children, and affects 90,000 Americans.
SCD results from a point mutation in the beta globin gene leading to a defective hemoglobin molecule
Platt NEJM, 1994
EXIT


Sickle Cell Disease & Bone Alterations
Children with Sickle cell disease (SCD) experience delayed skeletal maturation and over 70% of adult SCD patients' exhibit osteopenia or osteoporosis


Osteoporosis
Because inflammation significantly perturbs bone turnover we have hypothesized that bone loss in SCD is a likely consequence of the development of the inflammatory state.
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Calcium handling in cardiac myocytes
Very little is known about the fundamental characteristics of the very young human ventricle
Preoperative and postoperative management of pediatric cardiac patients is based mainly on data extrapolated from either adult heart patients or animal models
Contractility of the heart depends on calcium movement that is critical for optimal cardiac function
SR


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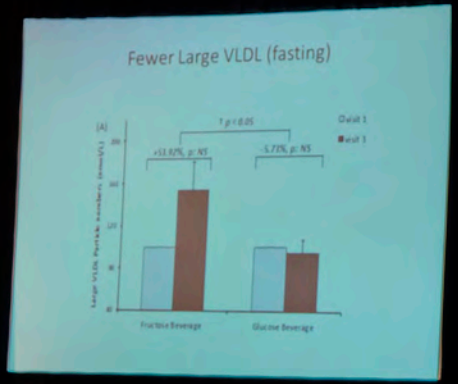
Miriam
Vos


Improved Adipose IR and CRP after Fructose Reduction


Fewer Large VLDL (fasting)
(A)
† p < 0.05
+53.82%, p: NS
-5.71%, p: NS
Visit 1
Visit 3
Fructose Beverage
Glucose Beverage


Postulated Role of Fructose in Mediating NAFLD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Paul


Paul
Spearman


Goal: HIV excision from human genome


Goal: HIV excision from human genome
TALEN
TALEN
Human genome
HIV provirus
Human provirus
Human genome
Human provirus
NHEJ
Human provirus
EXIT


Design of HIV-targeting TALENs
• Well conserved
• Exist in both 5' and 3' LTRs
• SAPTA provides high-scoring target sites.
• 6 total mismatches to the closest sites in human genome (PROGNOS)
TALEN
G-27
G-28
% SSA
26
25
EXIT
EXIT


Designer HIV-targeting TALENs


2013 Pediatric Research Retreat
Poster Award
3rd Place
Basic Research
Presented on June 26, 2013


2013 Pediatric Research Retreat
Poster Award
3rd Place
Basic Research
Presented on June 28, 2013


Duke


2013 Pediatric Research Retreat
Poster Award
1st Place
Basic Research


2013 Pediatric Research Retreat
Poster Award
1st Place
Basic Research


Poster Award


Poster Award


Poster Award
2nd Place
Clinical Research


2013 Pediatric Research Retreat
Poster Award
David
Okou


2013 Pediatric Research Retreat
Poster Award
2nd Place
David
Okou


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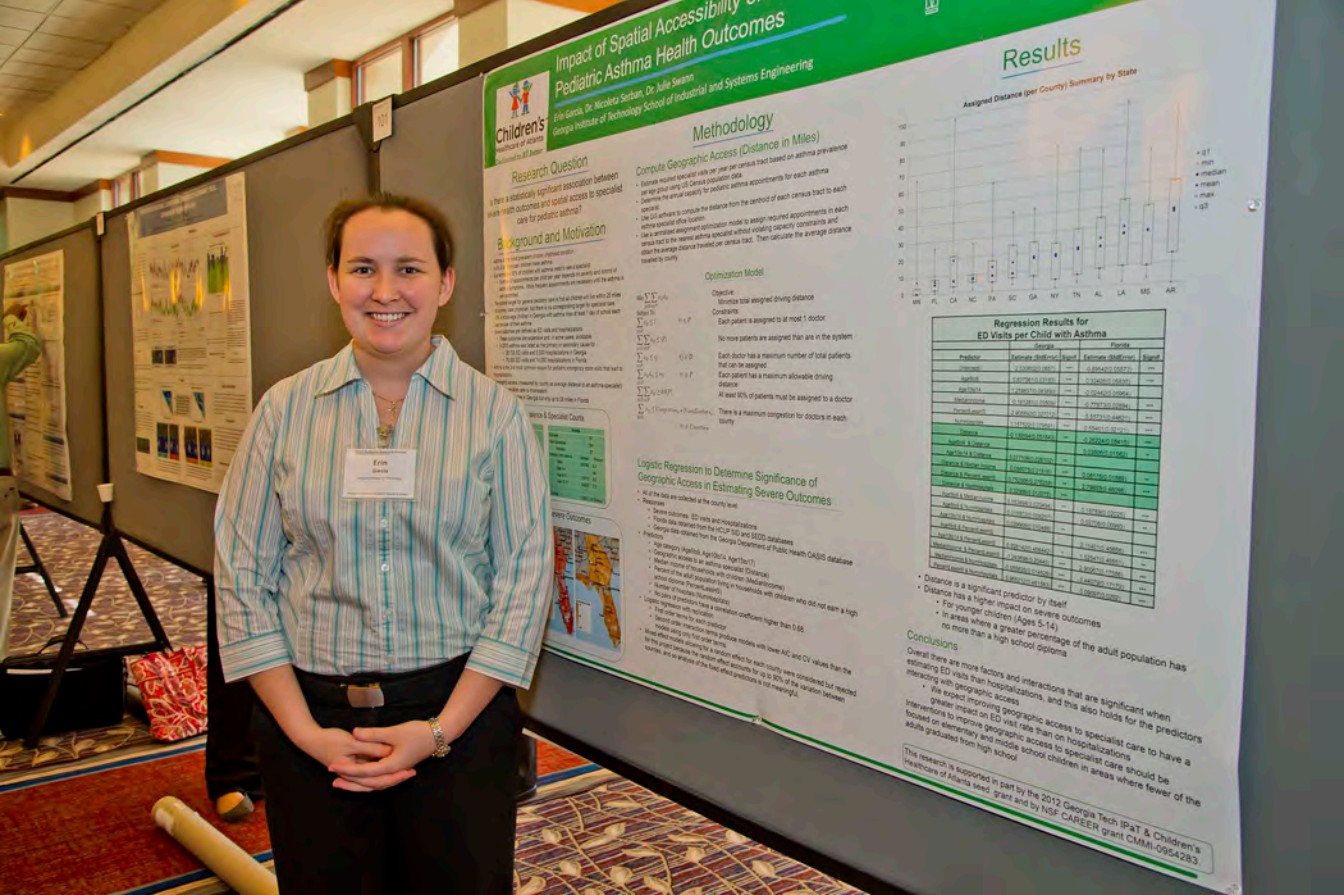
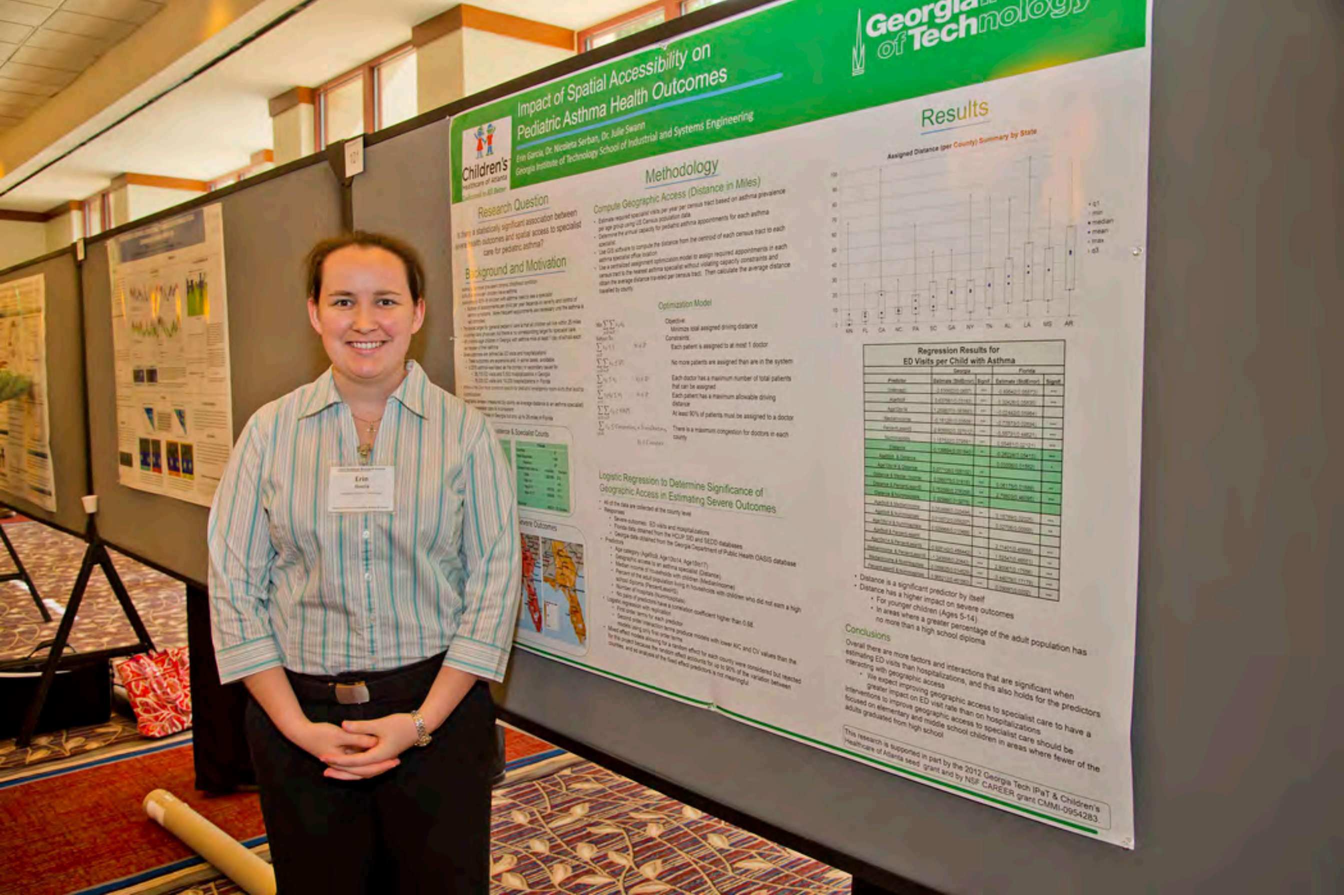

Impact of Spatial Accessibility on Severe Pediatric Asthma Health Outcomes
Erin Garcia, Dr. Nicoleta Serban, Dr. Julie Swann
Georgia Institute of Technology School of Industrial and Systems Engineering
Children's Healthcare of Atlanta
Research Question
Background and Motivation
Methodology
Compute Geographic Access (Distance in Miles)
Optimization Model
Logistic Regression to Determine Significance of Geographic Access in Estimating Severe Outcomes
Results
Assigned Distance (per County) Summary by State
Regression Results for ED Visits per Child with Asthma
Conclusions
This research is supported in part by the 2012 Georgia Tech IPaT & Children's Healthcare of Atlanta seed grant and by NSF CAREER grant CMMI-0954283.



Georgia
of Technology
Impact of Spatial Accessibility on
Pediatric Asthma Health Outcomes
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Georgia Institute of Technology School of Industrial and Systems Engineering
Children's
Healthcare of Atlanta
Research Question
Is there a statistically significant association between severe health outcomes and spatial access to specialist care for pediatric asthma?
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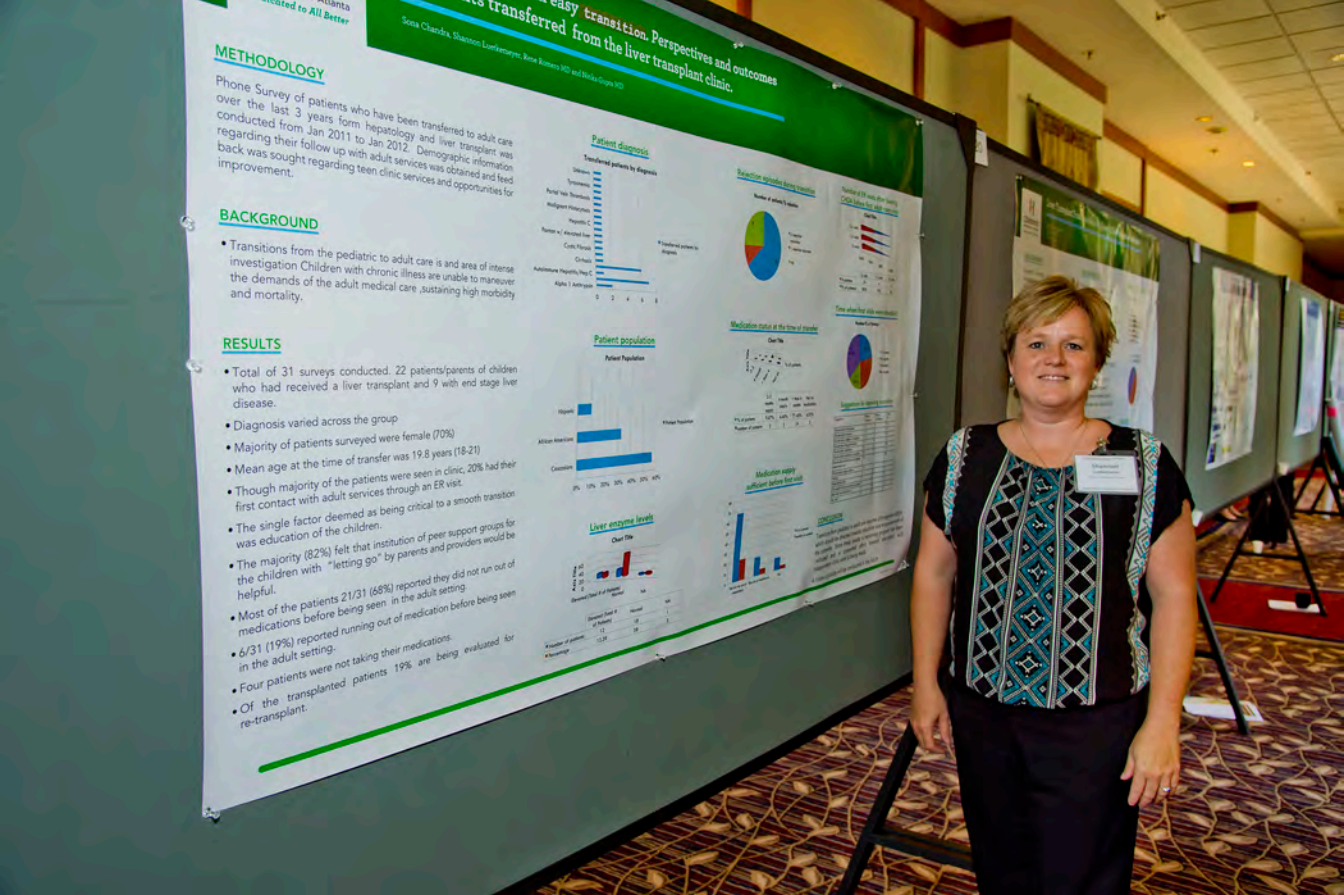
Logistic Regression to Determine Significance of Geographic Access in Estimating Severe Outcomes
Results
Assigned Distance (per County) Summary by State
Regression Results for
ED Visits per Child with Asthma
Distance is a significant predictor by itself
Distance has a higher impact on severe outcomes
For younger children (Ages 5-14)
In areas where a greater percentage of the adult population has no more than a high school diploma
Conclusions
Overall there are more factors and interactions that are significant when estimating ED visits than hospitalizations, and this also holds for the predictors interacting with geographic access
We expect improving geographic access to specialist care to have a greater impact on ED visit rate than on hospitalizations
Interventions to improve geographic access to specialist care should be focused on elementary and middle school children in areas where fewer of the adults graduated from high school
This research is supported in part by the 2012 Georgia Tech IPaT & Children's Healthcare of Atlanta seed grant and by NSF CAREER grant CMMI-0954283.
Erin


Children's Healthcare of Atlanta
Dedicated to All Better
easy transition. Perspectives and outcomes
ts transferred from the liver transplant clinic.
Sona Chandra, Shannon Luetkemeyer, Rene Romero MD and Nicka Goyle MD
METHODOLOGY
Phone Survey of patients who have been transferred to adult care over the last 3 years form hepatology and liver transplant was conducted from Jan 2011 to Jan 2012. Demographic information regarding their follow up with adult services was obtained and feed back was sought regarding teen clinic services and opportunities for improvement.
BACKGROUND
• Transitions from the pediatric to adult care is and area of intense investigation Children with chronic illness are unable to maneuver the demands of the adult medical care ,sustaining high morbidity and mortality.
RESULTS
• Total of 31 surveys conducted. 22 patients/parents of children who had received a liver transplant and 9 with end stage liver disease.
• Diagnosis varied across the group
• Majority of patients surveyed were female (70%)
• Mean age at the time of transfer was 19.8 years (18-21)
• Though majority of the patients were seen in clinic, 20% had their first contact with adult services through an ER visit.
• The single factor deemed as being critical to a smooth transition was education of the children.
• The majority (82%) felt that institution of peer support groups for the children with "letting go" by parents and providers would be helpful.
• Most of the patients 21/31 (68%) reported they did not run out of medications before being seen in the adult setting.
• 6/31 (19%) reported running out of medication before being seen in the adult setting.
• Four patients were not taking their medications.
• Of the transplanted patients 19% are being evaluated for re-transplant.
Patient diagnosis
Transferred patients by diagnosis
Unknown
Tyrosinemia
Portal Vein Thrombosis
Malignant Hepatoma
Hepatitis A
Trauma w/ elevated liver
Cystic Fibrosis
Cirrhosis
Autoimmune Hepatitis/Hep C
Alpha 1 Antitrypsin
0 2 4 6 8
Transferred patients for diagnosis
Reaction episodes during transition
Number of patients by reaction
Patient population
Patient Population
Hispanic
African American
Caucasian
0% 10% 20% 30% 40% 50% 60%
Liver enzyme levels
Chart Title
Medication status at the time of transfer
Chart Title
Medication supply sufficient before first visit
CONCLUSION

METHODOLOGY

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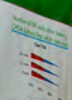
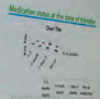
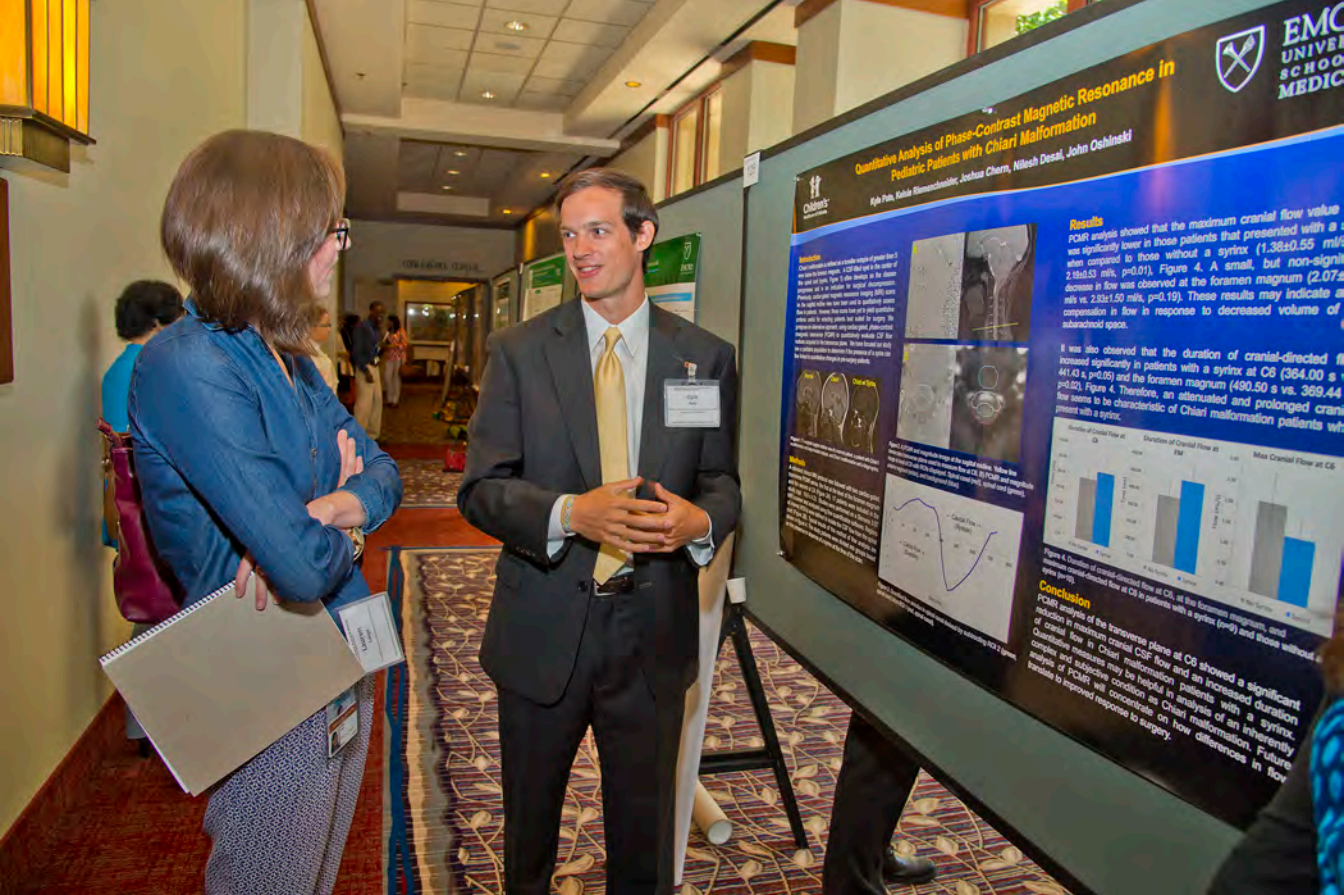





Children's
Healthcare of Atlanta
owing up: not an easy transition. Perspectives and outcomes
of patients transferred from the liver transplant clinic.
METHODOLOGY
BACKGROUND
RESULTS
Patient diagnosis
Patient population
Liver enzyme levels


Quantitative Analysis of Phase-Contrast Magnetic Resonance in Pediatric Patients with Chiari Malformation
Kyle Pate, Kelsie Kleeman-Swindler, Joshua Chern, Nilesh Desai, John Oshinski
EMORY UNIVERSITY SCHOOL OF MEDICINE
Abstract
Introduction
Methods
Results
Conclusion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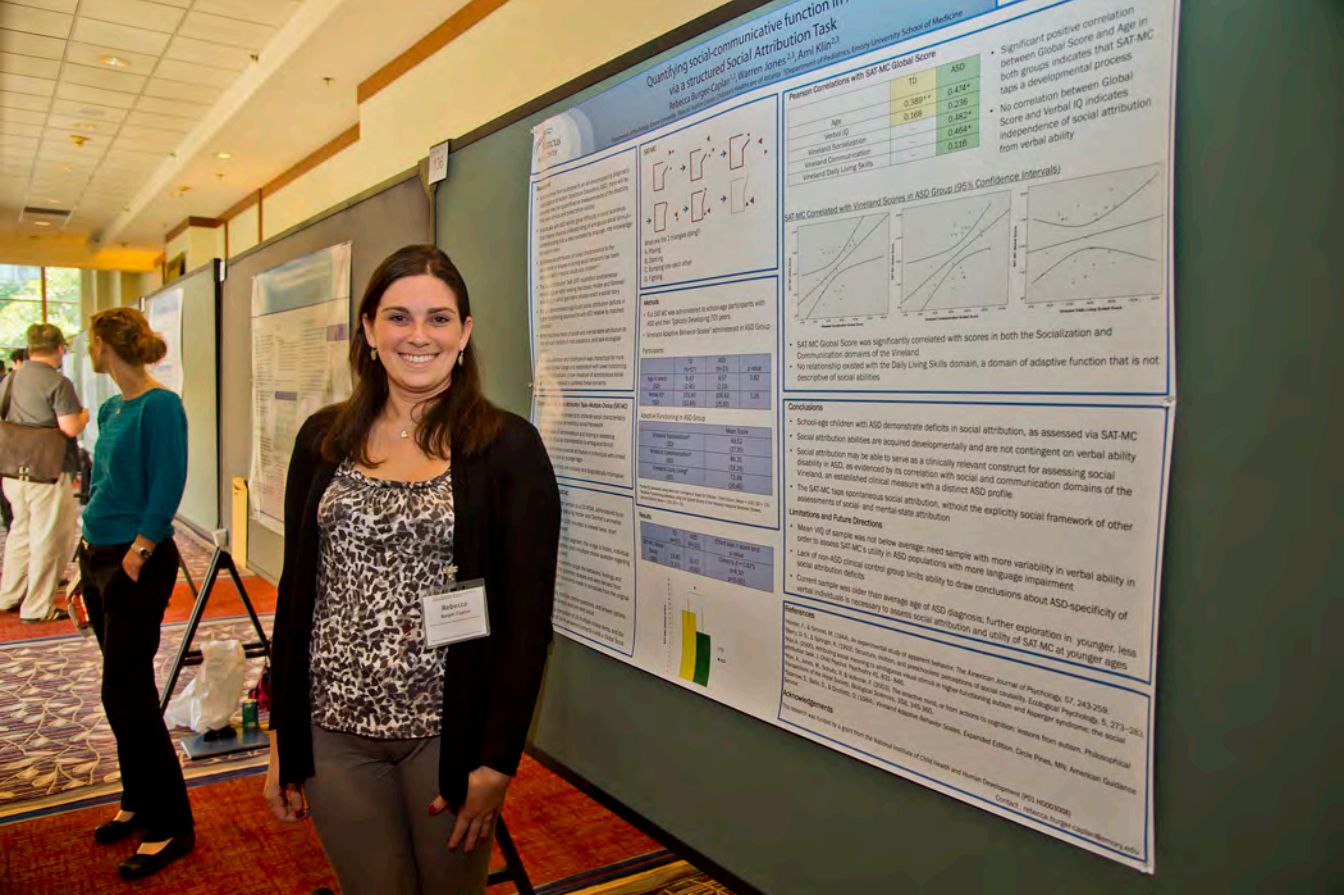
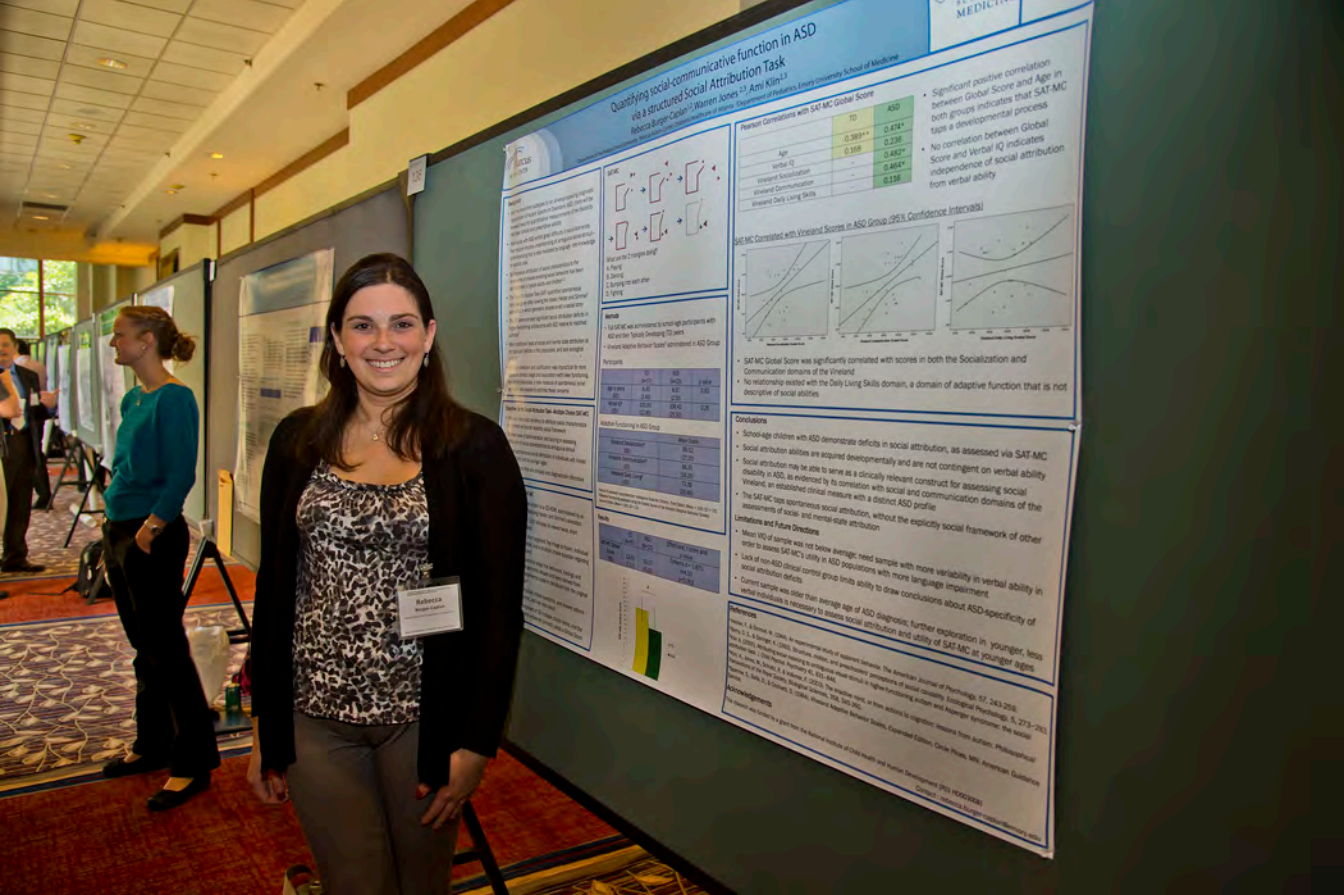

Quantifying social-communicative function in
via a structured Social Attribution Task
Rebecca Burger-Caplan
Warren Jones
Ami Klin
Pearson Correlations with SAT-MC Global Score
TD
ASD
0.389**
0.474*
0.168
0.236
0.482*
0.464*
0.516
Age
Verbal IQ
Vineland Socialization
Vineland Communication
Vineland Daily Living Skills
Significant positive correlation between Global Score and Age in both groups indicates that SAT-MC taps a developmental process
No correlation between Global Score and Verbal IQ indicates independence of social attribution from verbal ability
SAT-MC Correlated with Vineland Scores in ASD Group (95% Confidence Intervals)
SAT-MC Global Score was significantly correlated with scores in both the Socialization and Communication domains of the Vineland
No relationship existed with the Daily Living Skills domain, a domain of adaptive function that is not descriptive of social abilities
Conclusions
School-age children with ASD demonstrate deficits in social attribution, as assessed via SAT-MC
Social attribution abilities are acquired developmentally and are not contingent on verbal ability
Social attribution may be able to serve as a clinically relevant construct for assessing social disability in ASD, as evidenced by its correlation with social and communication domains of the Vineland, an established clinical measure with a distinct ASD profile
The SAT-MC taps spontaneous social attribution, without the explicitly social framework of other assessments of social- and mental-state attribution
Limitations and Future Directions
Mean VIQ of sample was not below average; need sample with more variability in verbal ability in order to assess SAT-MC's utility in ASD populations with more language impairment
Lack of non-ASD clinical control group limits ability to draw conclusions about ASD-specificity of social attribution deficits
Current sample was older than average age of ASD diagnosis; further exploration in younger, less verbal individuals is necessary to assess social attribution and utility of SAT-MC at younger ages
References
Acknowledgments
Rebecca
Burger-Caplan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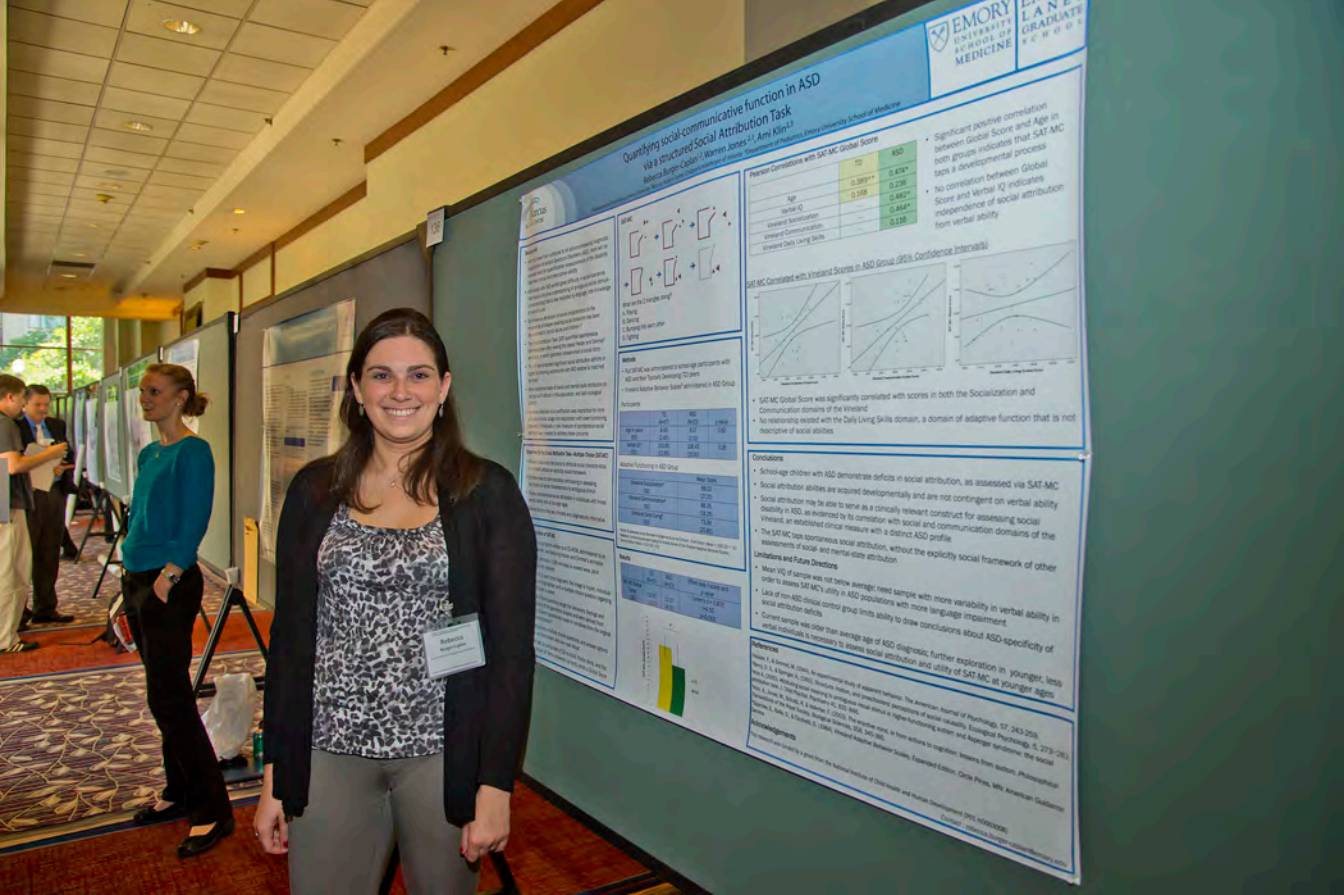
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Rebecca
Bunce-Caplan


Warren
Children's


Warren
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Michael


Michael


2nd Place
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METHODS
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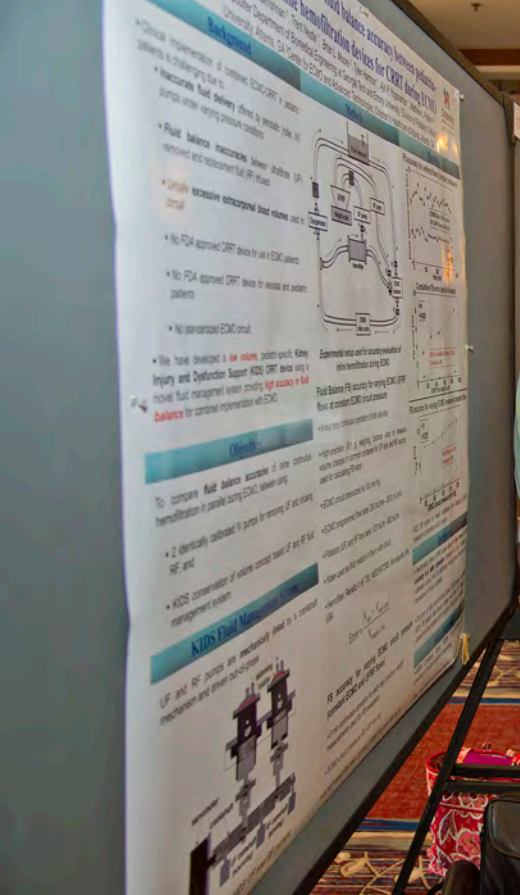
EMORY
UNIVERSITY
CONCLUSIONS




Fructose reduction improves CVD risk in adolescents with NAFLD
RESULTS
CONCLUSIONS
Background and Objective
Methods
Results
Discussion


Miriam

Quantitative Analysis of Phase-Contrast Magnetic Resonance in Pediatric Patients with Chiari Malformation

Kyle Pate, Kelsie Riemenschneider, Joshua Chern, Nilesh Desai, John Oshinski

Children's Healthcare of Atlanta

EMORY UNIVERSITY SCHOOL OF MEDICINE

Introduction

Chiari malformation is defined as a tonsillar ectopia of greater than 5 mm below the foramen magnum. A CSF-filled cyst in the center of the spinal cord cyrinx, Figure 1), often develops as the disease progresses and is an indication for surgical decompression. Previously, cardiac-gated magnetic resonance imaging (MRI) scans in the sagittal midline view have been used to qualitatively assess flow in patients. However, these scans have yet to yield quantitative criteria useful for selecting patients best suited for surgery. We propose an alternative approach, using cardiac-gated, phase-contrast magnetic resonance (PCMR) to quantitatively evaluate CSF flow values acquired in the transverse plane. We have focused our study on a pediatric population to determine if the presence of a syrinx can be linked to quantitative changes in pre-surgery patients.

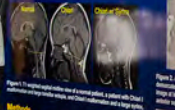


Figure 1. T1-weighted sagittal midline view of a normal patient, a patient with Chiari I malformation and large tonsillar ectopia, and Chiari I malformation and a large syrinx.

Methods

A standard clinical MRI protocol was followed with two cardiac-gated, transverse PCMR series, the first of the level of the foramen magnum and the second at C6 (Figure 2A). 15 patients were included in the study (age: 10.6 ± 4.3). Studies were performed on a Siemens 3.0T MRI scanner and post-processing was performed on a Siemens 3.0T workstation using flow quantification software. Regions of interest (ROI's) were defined to isolate the CSF flow from the spinal cord (Figure 2B). Typical results of this method of flow analysis are shown in figure 3. The study patients were divided into groups based on the presence or absence of a syrinx at the time of the scan.

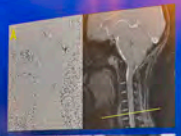


Figure 2. A) PCMR and magnitude image at the sagittal midline. Yellow line demarcates transverse plane used to measure flow at C6. B) PCMR and magnitude image at level of C6 with ROIs displayed. Spinal canal (red), spinal cord (green), exterior segment (white), and background (blue).

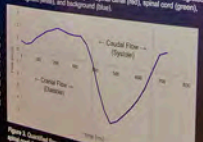


Figure 3. Quantitative flow analysis in spinal canal derived by subtracting ROI 2 (green, spinal cord) from ROI 1 (red, spinal canal).

Results

PCMR analysis showed that the maximum cranial flow value at C6 was significantly lower in those patients that presented with a syrinx when compared to those without a syrinx (1.38±0.55 ml/s vs. 2.19±0.53 ml/s, p=0.01). Figure 4. A small, but non-significant decrease in flow was observed at the foramen magnum (2.07±0.91 ml/s vs. 2.93±1.50 ml/s, p=0.19). These results may indicate a decompensation in flow in response to decreased volume of the subarachnoid space.

It was also observed that the duration of cranial-directed flow increased significantly in patients with a syrinx at C6 (364.00 s vs. 441.43 s, p=0.05) and the foramen magnum (490.50 s vs. 369.44 s, p=0.02), Figure 4. Therefore, an attenuated and prolonged cranial flow seems to be characteristic of Chiari malformation patients who present with a syrinx.

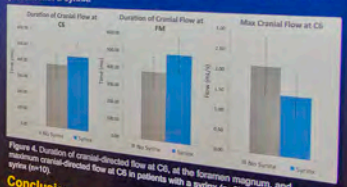
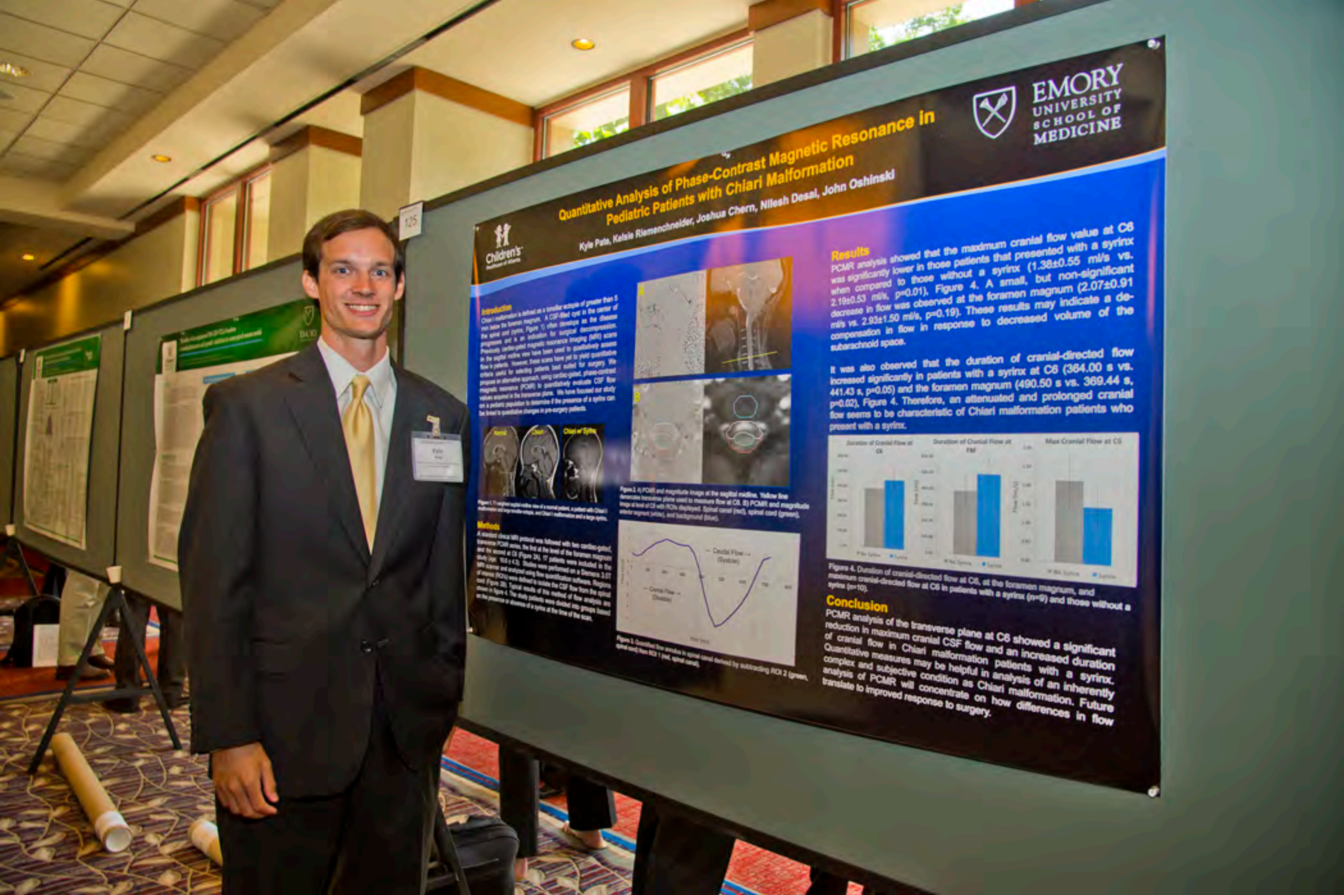


Figure 4. Duration of cranial-directed flow at C6, at the foramen magnum, and maximum cranial-directed flow at C6 in patients with a syrinx (n=9) and those without a syrinx (n=10).

Conclusion

PCMR analysis of the transverse plane at C6 showed a significant reduction in maximum cranial CSF flow and an increased duration of cranial flow in Chiari malformation patients with a syrinx. Quantitative measures may be helpful in analysis of an inherently complex and subjective condition as Chiari malformation. Future analysis of PCMR will concentrate on how differences in flow translate to improved response to surgery.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Introduction
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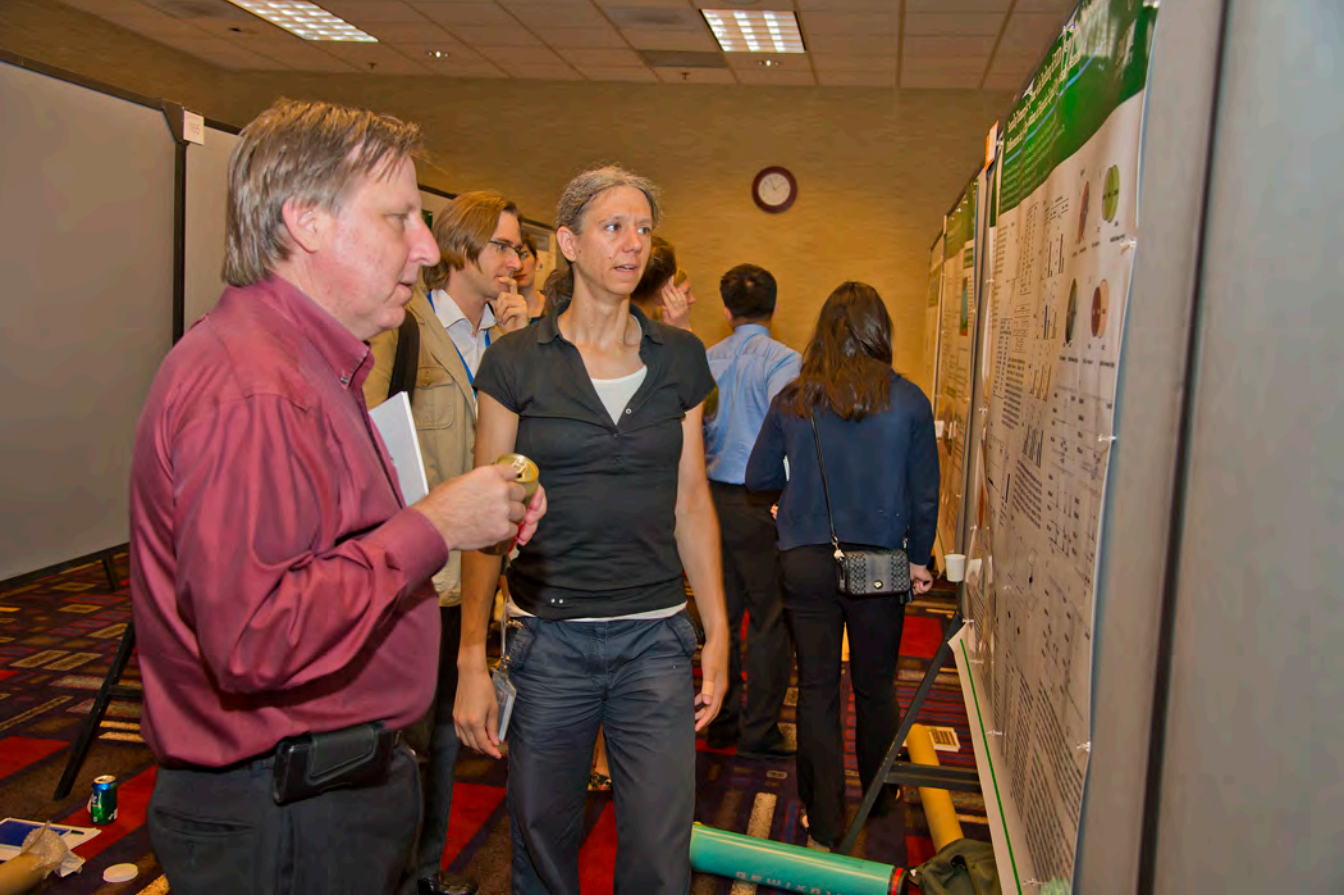
Methods
Results

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Mina


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Sarah
Shultz


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Nuclease-based Gene Correction for Treating Spinal Muscular Atrophy
Wilfried Rossoll, PhD[1], TJ Cradick, PhD[2], Han Phan, MD[3], Gang Bao, PhD[2]
[1] Dept. of Cell Biology, Emory University [2] Dept. of Biomedical Engineering, Georgia Tech and Emory University [3] Pediatric Neurology, Emory University and Children's Healthcare of Atlanta
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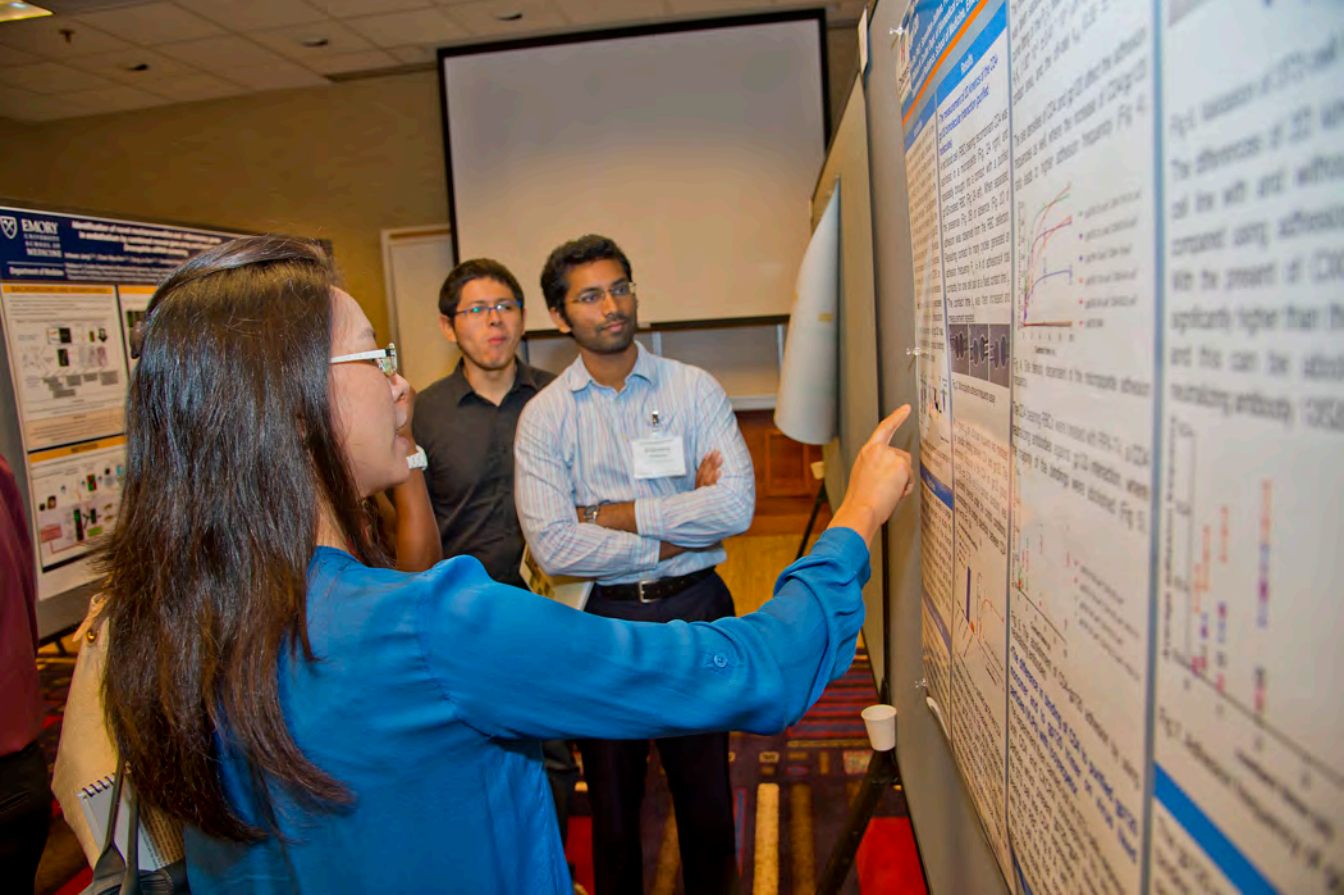
Abstract
Spinal muscular atrophy (SMA) type I, caused by homozygous deletions or mutations in the SMN1 gene, which leads to a very specific degeneration of motor neurons in the ventral horn of the spinal cord.
Humans also possess a second copy of the gene, SMN2, which encodes the same protein, but harbors a splice site mutation that causes skipping of exon 7 in approximately 90% of the SMN2 transcripts, resulting in a truncated and unstable protein.
Reasoning SMN2 splicing can rescue the spinal muscular atrophy disease phenotype. We are optimizing TAL Effector Nucleases (TALENs) to correct the splice site mutation in the SMN2 locus as a novel approach for treating SMA.
Spinal Muscular Atrophy (SMA)
- Prognosis: Babies diagnosed with SMA type I do not usually live past two years of age.
- Genetics = 95% are caused by mutations in the survival motor neuron 1 (SMN1) gene.
SMN2 TALEN-Pairs Validated
Humans have two SMN genes, SMN1 and SMN2, with identical protein-encoding capacity.
genes
mRNA
proteins
SMN1 gene deletion/mutation in SMA leaves SMN2 as the only source of SMN protein.
TALEN-mediated editing of the SMN2 locus Restores SMN Protein Levels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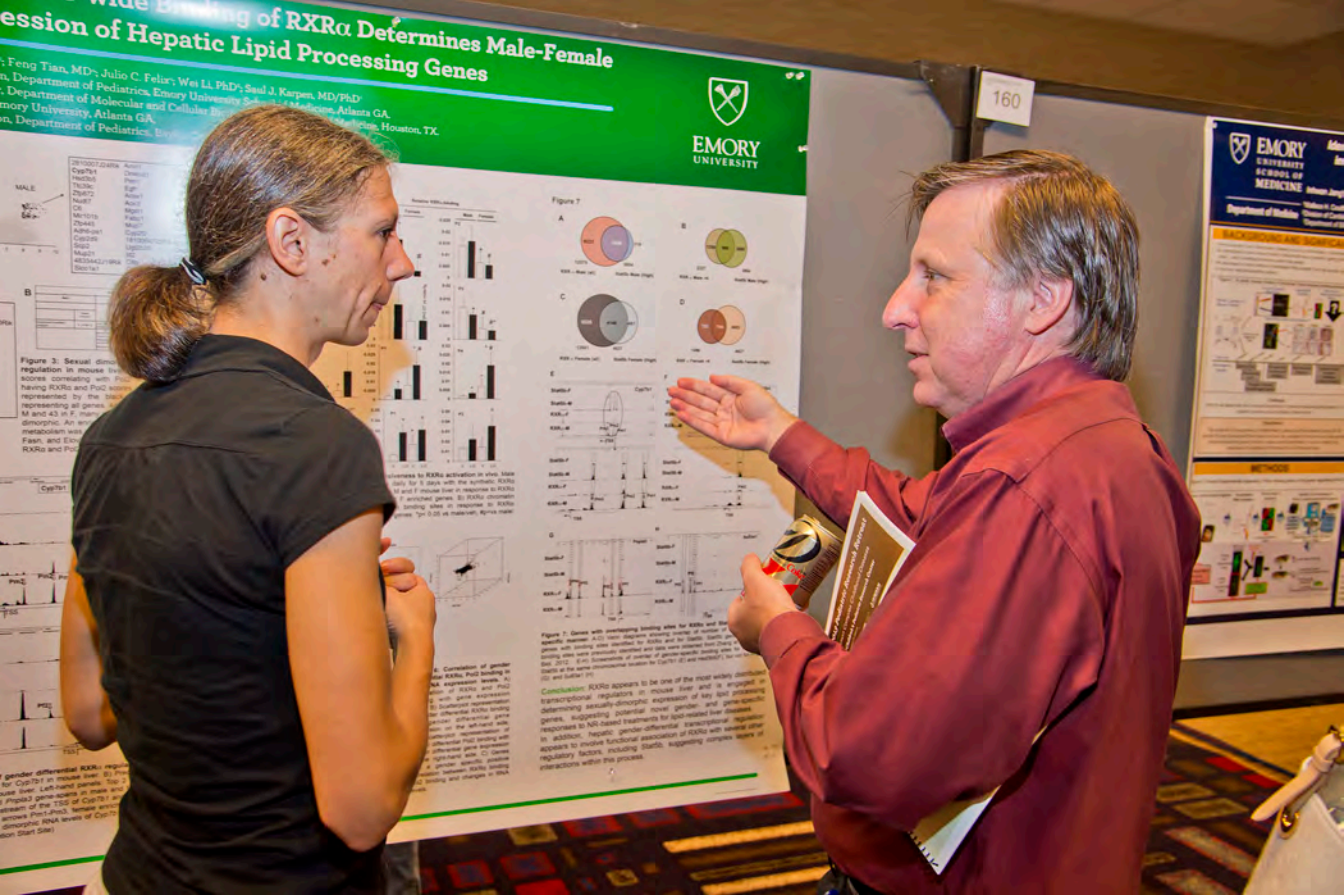
RXRα Determines Male-Female
Hepatic Lipid Processing Genes
Feng Tian, MD; Julie C. Felix; Wei Li, PhD; Saul J. Karpen, MD/PhD
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Department of Medicine
BACKGROUND AND SIGNIFICANCE
METHODS


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rabbit hearts
apoptosis induced increase in
OA binding and ubiquitination
in adult CMs


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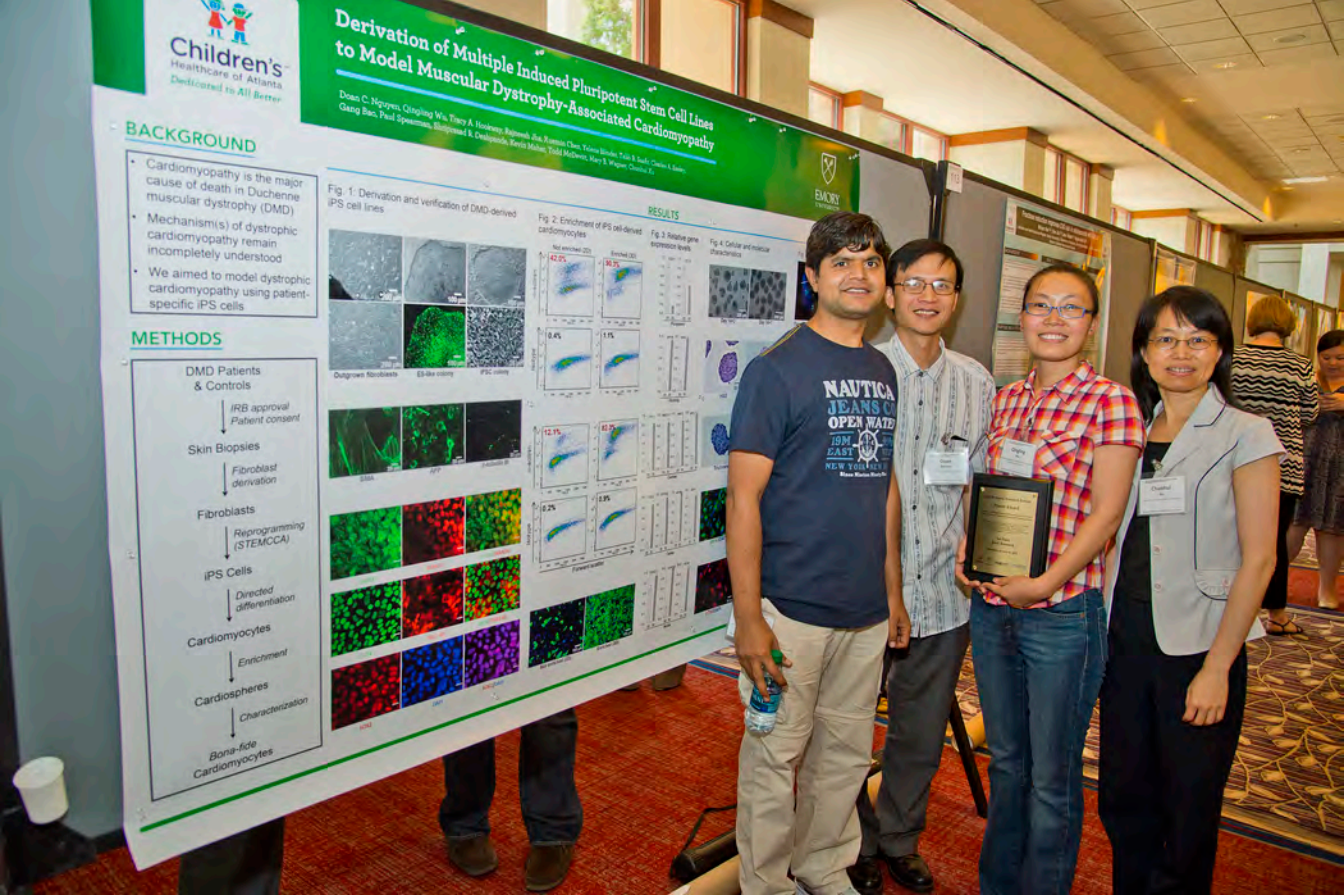
Growing up: not an easy transition. Perspecti
of patients transferred from the liver transpla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Derivation of Multiple Induced Pluripotent Stem Cell Lines to Model Muscular Dystrophy-Associated Cardiomyopathy
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BACKGROUND
Cardiomyopathy is the major cause of death in Duchenne muscular dystrophy (DMD)
Mechanism(s) of dystrophic cardiomyopathy remain incompletely understood
We aimed to model dystrophic cardiomyopathy using patient-specific iPS cells
METHODS
DMD Patients & Controls
IRB approval Patient consent
Skin Biopsies
Fibroblast derivation
Fibroblasts
Reprogramming (STEMCCA)
iPS Cells
Directed differentiation
Cardiomyocytes
Enrichment
Cardiospheres
Characterization
Bona-fide Cardiomyocytes
RESULTS
Fig. 1: Derivation and verification of DMD-derived iPS cell lines
Fig 2: Enrichment of iPS cell-derived cardiomyocytes
Fig. 3: Relative gene expression levels
Fig 4: Cellular and molecular characteristics
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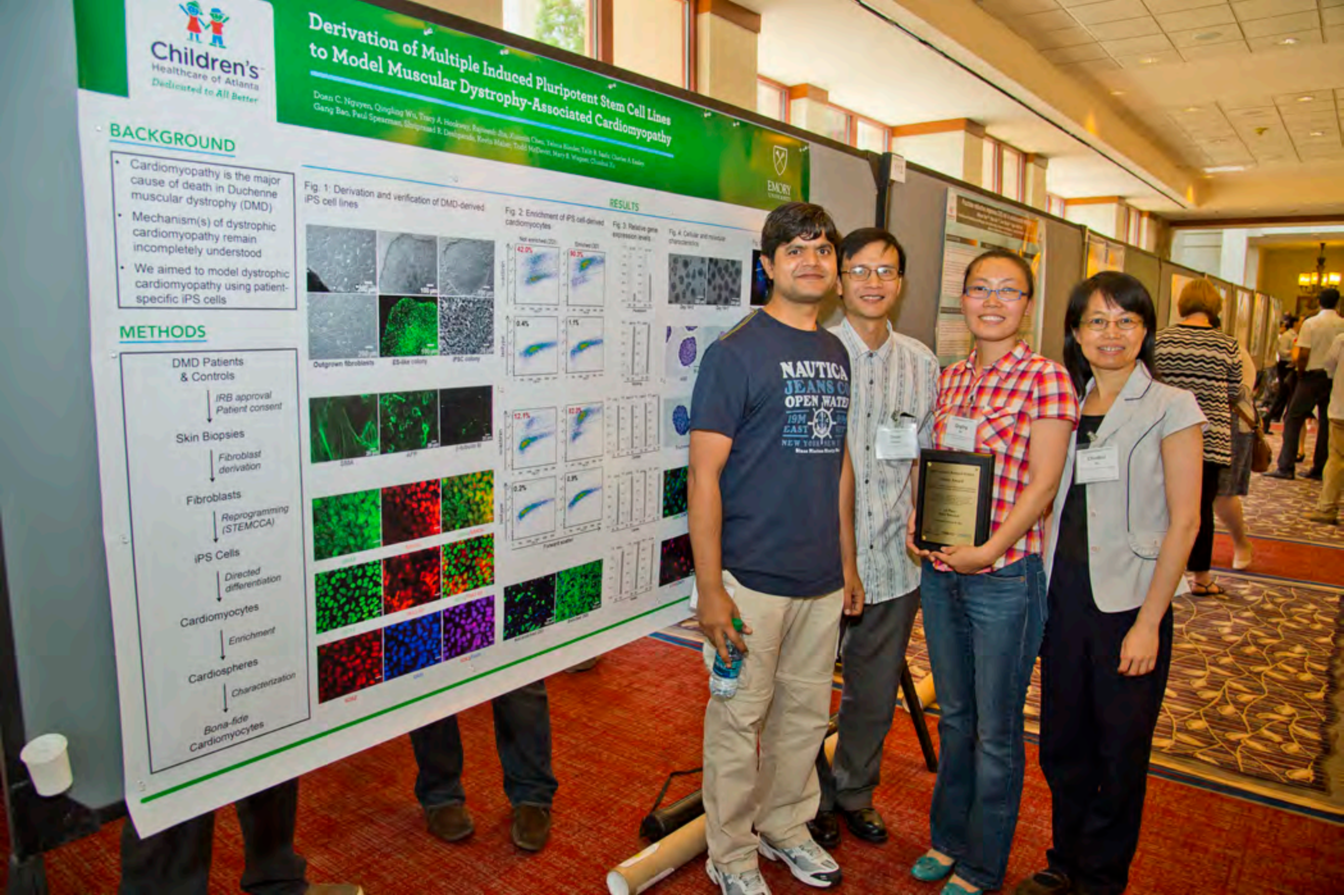
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Cardiomyocytes
Enrichment
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Characterization
Bona-fide Cardiomyocytes
RESULTS
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Outgrown fibroblasts
ES-like colony
iPSC colony
Fig. 2: Enrichment of iPS cell-derived cardiomyocytes
Fig. 3: Relative gene expression levels
Fig. 4: Cellular and molecular characteristics
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Ignacio
Sanz


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1st Place
Clinical Research


2013 Pediatric Research Retreat
Poster Award
1st Place
Clinical Research
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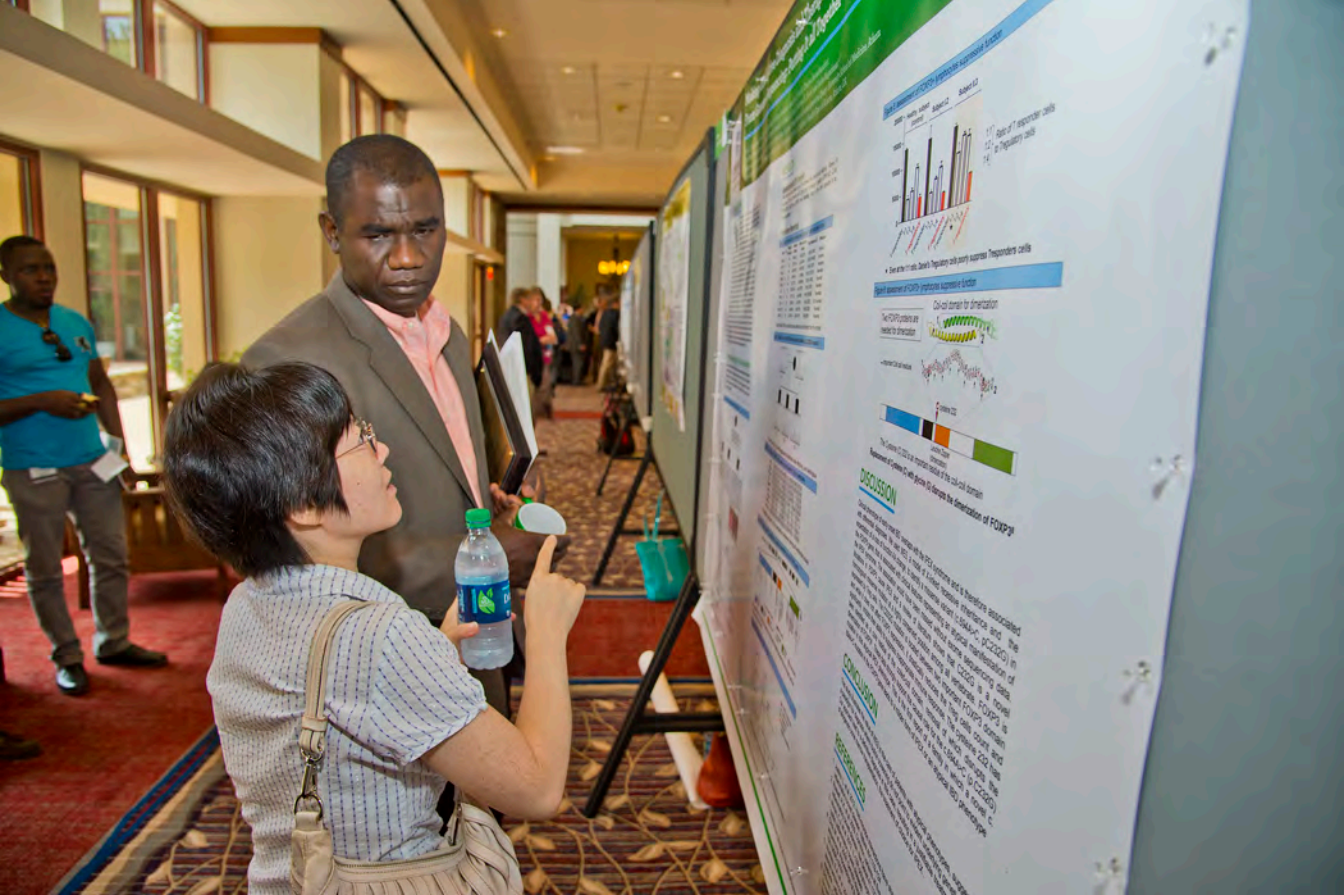
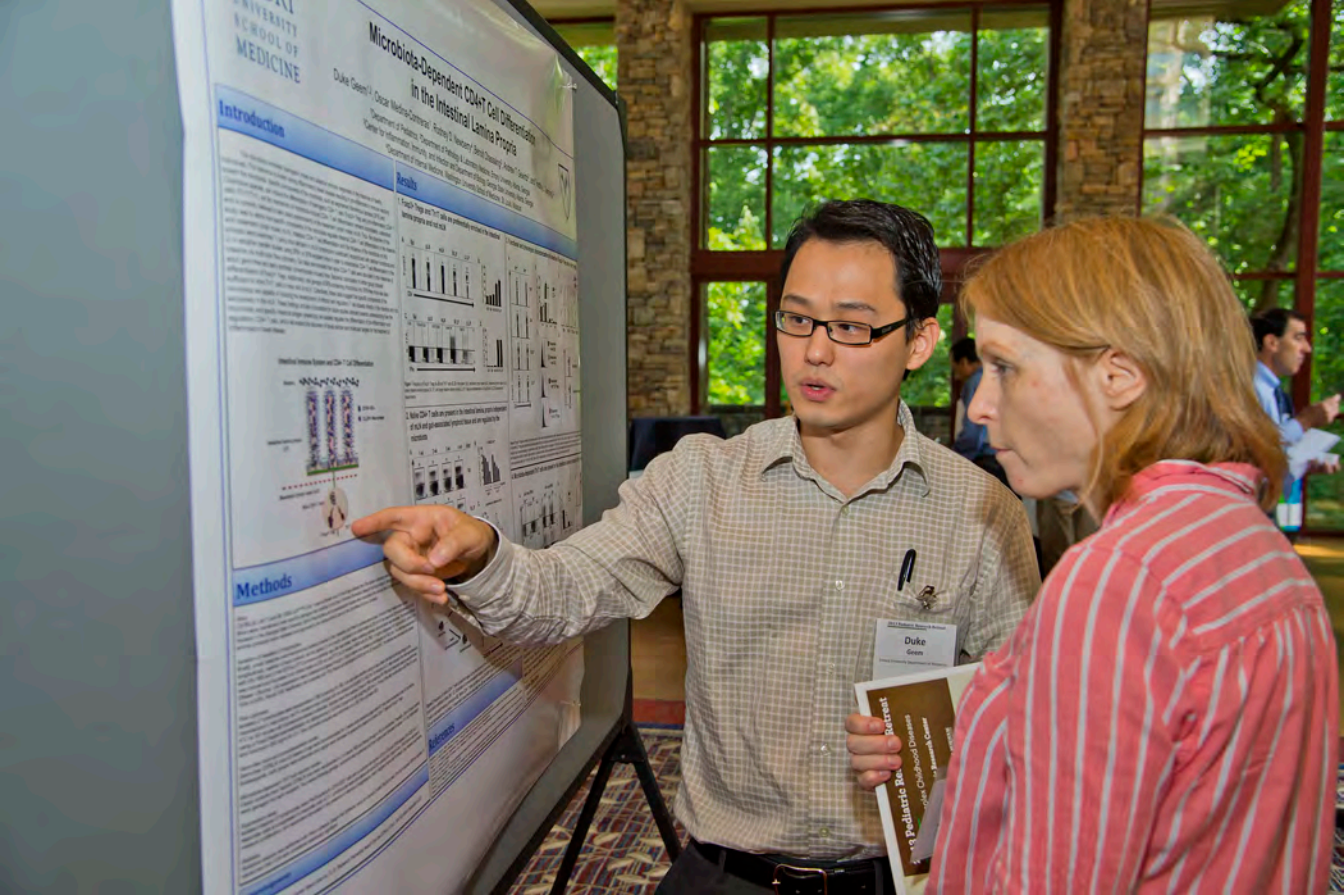

DISCUSSION
CONCLUSION
REFERENCES


Microbiota-Dependent CD4+ T Cell Differentiation in the Intestinal Lamina Propria
Introduction
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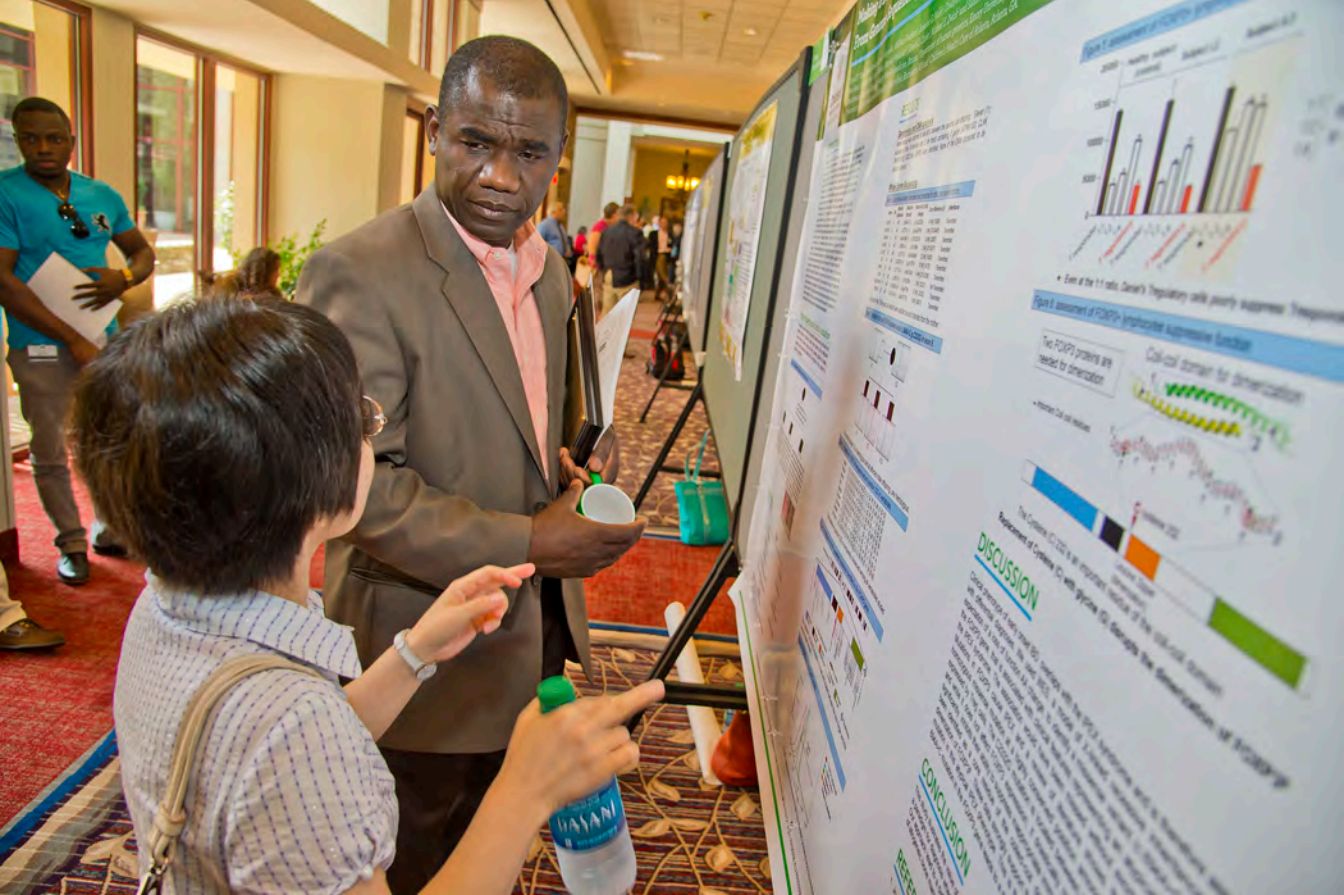
Geem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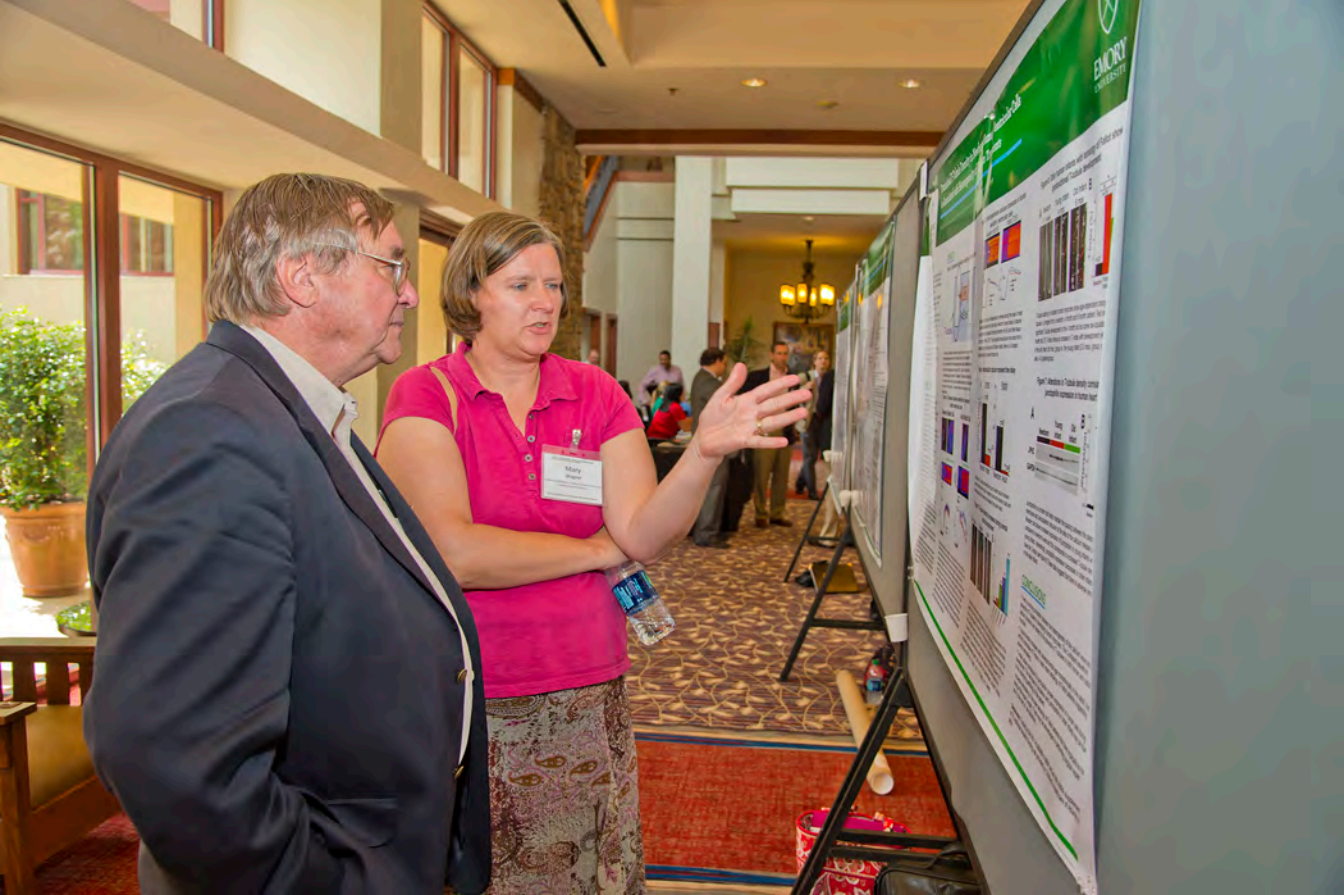
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CONCLUSIONS


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E-mail your study protocol to PRC@choa.org or call 404-785-0400
The PRC staff are available to assist you through the protocol development and administrative processes.
Administrative services provided include:
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• Feasibility assessment and ancillary department approvals
• Assistance with development of physician orders
• Clinical staff education and outreach
Additional tools and resources are available on www.ACTSI.org.
Current Users
Pulmonary
Nephrology
Endocrinology
Hepatology
Infectious Disease
Number of Active Studies by Department
PRC Volumes
2012
2011
Visits
Hours
The Pediatric Research Center mission is to provide an environment for the advancement of clinical excellence through clinical research. An essential component of this mission is to support the development of pediatric investigators by providing an environment that is supportive and conducive to the execution of quality clinical research.


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Barbara
David


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Kevin
Descriptive Table of Follow Up Patients with Self-Reported Recurrence of SSTI
Conclusions
References


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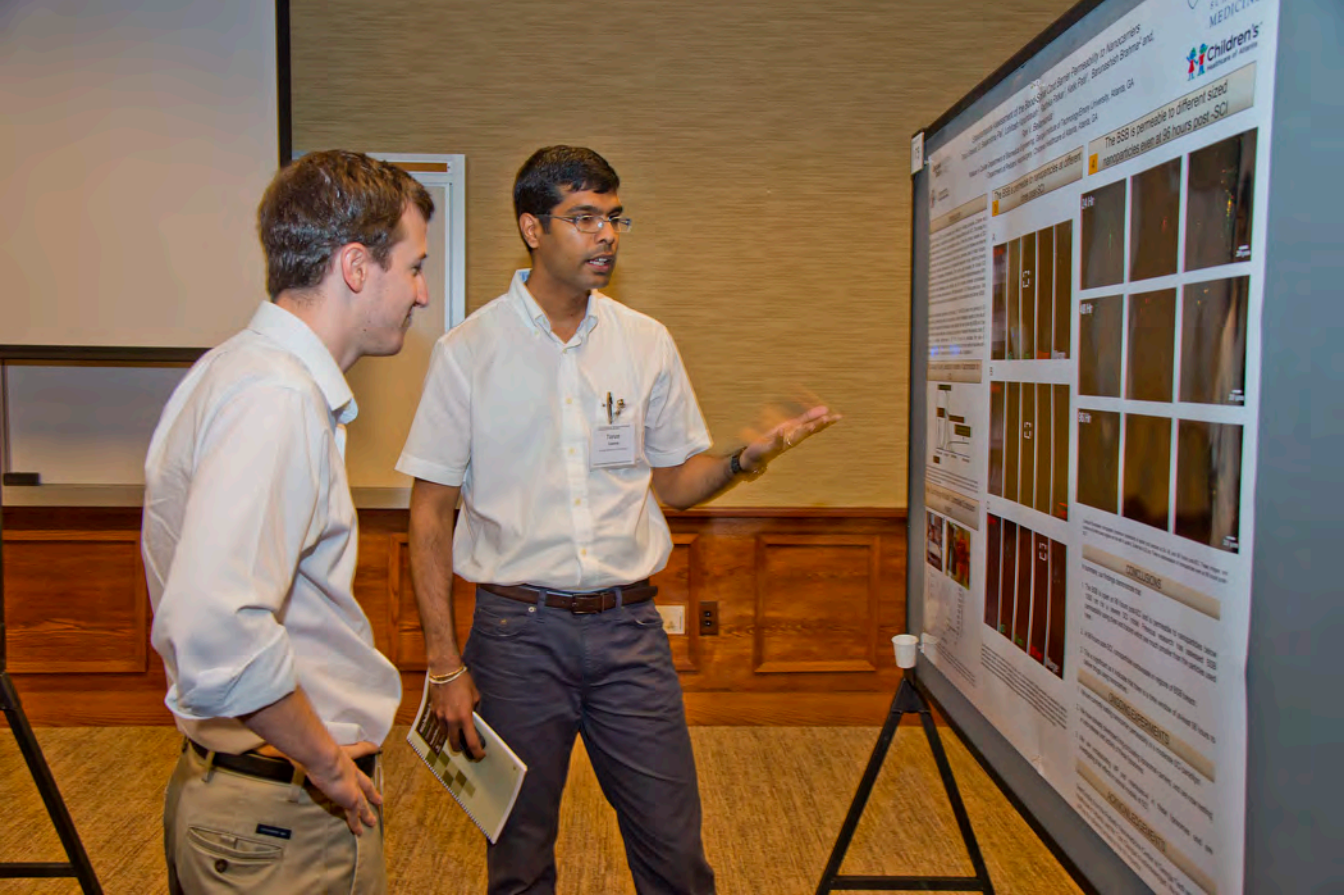
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The SSS is permeable to different sized
nanoparticles even at 96 hours post -SCI


Georgia Tech
Impact of Spatial Accessibility on
Health Outcomes
Results
Regression Results for
ED Visits per Child with Asthma
Conclusions


Pediatric Asthma
Results


Pediatric Asthma Health Outcomes
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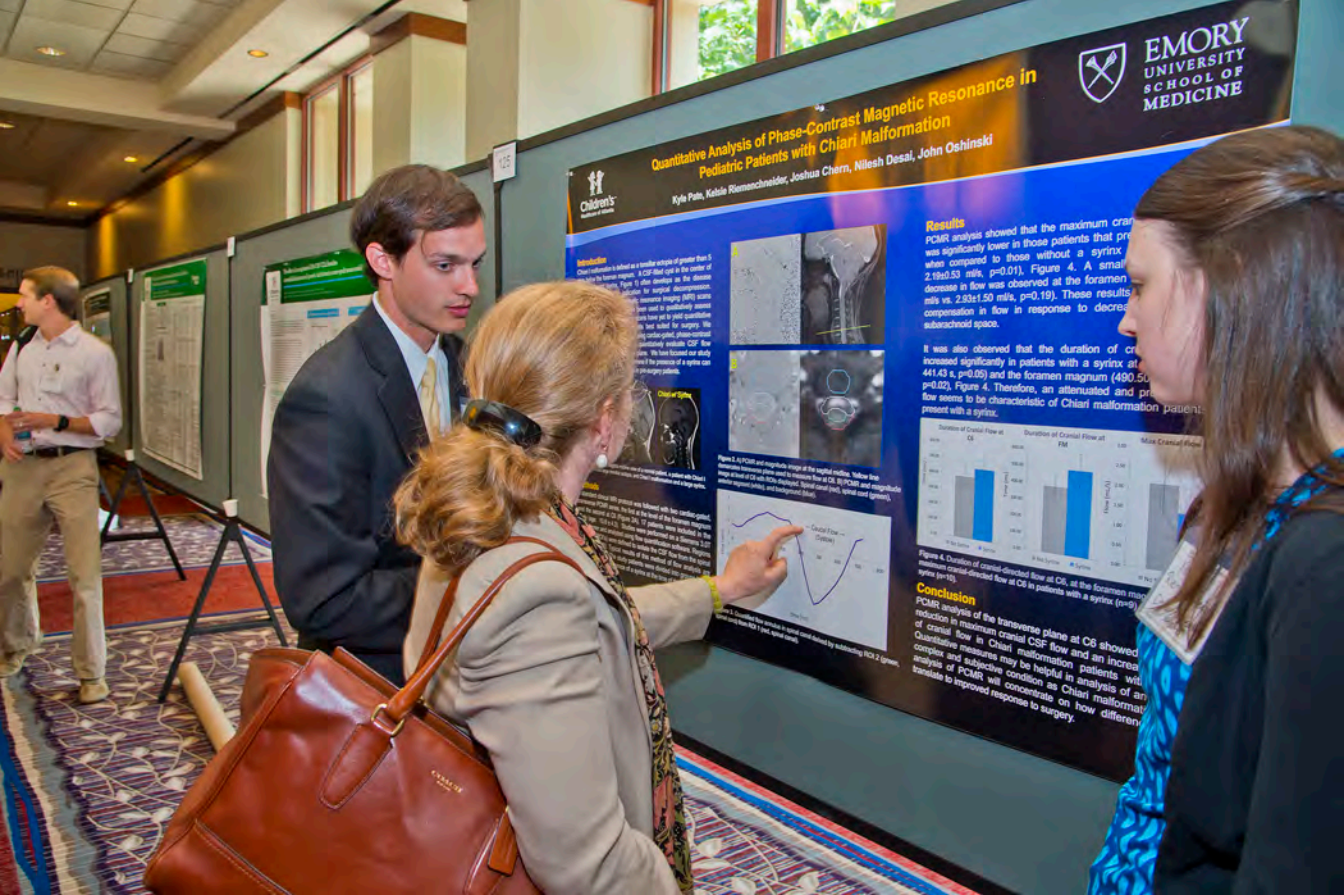
Methodology
Research Question


Quantitative Analysis of Phase-Contrast Magnetic Resonance in Pediatric Patients with Chiari Malformation
Kyle Pate, Kalsie Riemenschneider, Joshua Chern, Nilesh Desai, John Oshinski
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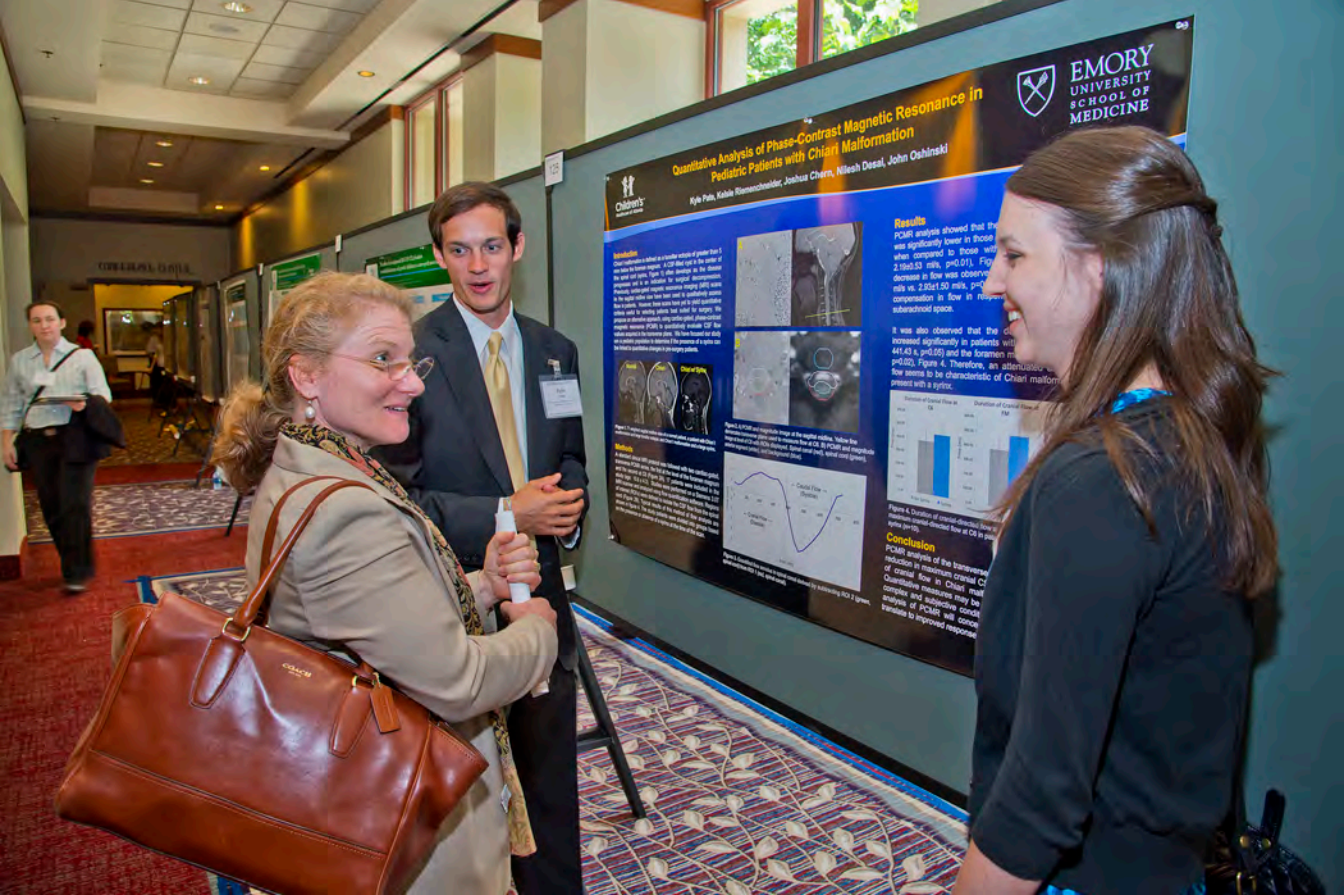
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Integrated Cellular Imaging


Pneumonia Care


Coca


Overview


Ignacio
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Saulnier


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Kiaiman
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Cheryl
Klaiman
Celine
Saulnier


2013 Pediatric Research Retreat
Common Complex Childhood Diseases
April
Dawson
Richard
Olney


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Common Complex Childhood Diseases
Emory+Children's Pediatric Research Center
April
Richard