

Emory+Children's Pediatric Research Center

A newsletter from the Center for Clinical and Translational Research



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SCHOOL OF MEDICINE

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Center Update: Cynthia Wetmore, MD, PhD

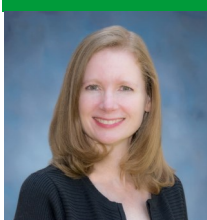
I extend a warm welcome to all investigators, collaborators and staff who have worked to make this first year a success in CCTR! We are nearing the one year anniversary of the opening of the Scottish Rite Research office and will be celebrating at a Holiday Breakfast on December 18th. We will continue to host Research Grand Rounds at Scottish Rite. Dr. Laura Hayes will present on October 20th and Dr. Susan Palasis December 15th. If you would like to present at a 2016 Scottish Rite Research Grand Rounds, please email Kristen Herzegh at kcoshau@emory.edu

We recently released the third call for funding proposals through the Friends' Grant mechanism. For more information, please visit [Friends Grant funding](#) (deadline October 19, 2015). We encourage all eligible applicants to apply!

We are also working to establish more regulatory expertise within our Center and are available to help with Investigational New Drug applications to the FDA. If you are interested in submitting an IND, or would like assistance maintaining your IND, please let us know.

I would like to invite investigators, and any individual interested in learning more about research resources, to attend the October 13th Physicians Research Dinner **"Taking Your Idea from the Back of a Napkin to an Open Study: Research Resources for Investigators"** [Dr. Stacy Heiman](#) and I will co-present. For more information, please [click here](#).

Please continue to let us know your ideas and suggestions as we continue to grow and establish CCTR.



Director of Clinical Research Services: Amanda Cook Coming Soon— Emory Clinical Research Space in the ECC Clinic

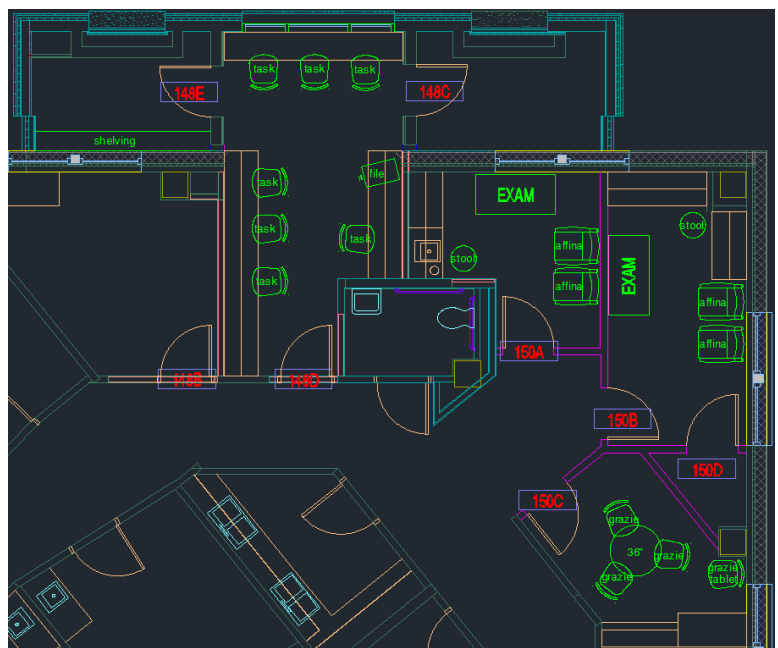
Construction is under way to remodel the old infusion room in the ECC clinic.

This will become Emory University space that will be dedicated to clinical research. There will be 2 exam rooms complete with exam tables and comfortable guest chairs. There will also be an interview room for consenting, surveys or questionnaires which can also be utilized for simple blood draws. Close by there will be a work room with touch down space for 6 people and plenty of storage.

The anticipated completion date is 11/2/15. Over the next month, we will be working out a system for scheduling and SOPs for the space. Stay tuned for additional details.

For more information, contact Amanda Cook:

amcook@emory.edu





MEDICAL CANNABIS IN GEORGIA

In April 2015, Georgia Governor Nathan Deal signed into law, “Haleigh’s Hope Law.” The law legalizes the medical use of Low THC Oil (cannabidiol or cannabis oil) for alternative treatment of eight medical conditions. Patients may possess up to 20 fluid ounces of the oil once they have applied for, and received, their low THC registry card.

Physicians cannot prescribe, supply or administer the oil; however, they can register patients. The act of registering a patient certifies that you have established a relationship with the patient, have examined them and determined they have one of the approved medical conditions (listed below). Physicians must register with the Georgia Department of Public Health and complete a physician certification form for a patient family to be eligible for the card. If a patient obtains the oil, physicians cannot administer or possess the oil on behalf of the patient.

To date more than 220 cards have been registered, of which 45% are for patients under the age of 18. Cancer, seizures, and ALS represent the top three conditions. Cards cost \$25 and must be renewed every two years.

Shortly after the passing of the law, Gov. Deal appointed a select group of lawmakers, healthcare professionals, and law enforcement officials to serve on the GA Commission on Medical Cannabis. Dr. Cynthia Wetmore serves on the Commission as the sole pediatric expert. The Commission meets regularly to discuss recommendations regarding the potential regulation and in-state growth of medical cannabis.



Dr. Cynthia Wetmore
signs her oath of office

&

Georgia’s Medical
Cannabis Commission



Qualifying Medical Conditions:

- Amyotrophic lateral sclerosis (ALS)
 - Cancer
 - Seizure disorder
 - Multiple sclerosis
 - Crohn’s disease
 - Mitochondrial disease
- Parkinson’s disease (when diagnosis is severe or end stage)
- Sickle cell disease (when diagnosis is severe or end stage)



Haleigh, whom Haleigh’s Hope Act is named, and Hawk pose with their Moms and Dr. Wetmore. Haleigh and her family recently moved back Georgia following the passage of the law.





“Advances in Pediatric Neurology: Improving the lives of Duchenne/Becker Muscular Dystrophy (D/BMD) Patients”

Han C. Phan, MD is a pediatric neurologist and sleep specialist who practices neurology at both Scottish Rite and Egleston Children’s hospitals and is Assistant Professor at Emory University School of Medicine. Her primary interest is in clinical research in children with neuromuscular disorders specifically seen in Muscular Dystrophy Association (MDA) clinic at Scottish Rite hospital. She sees patients in MDA clinic alongside with Drs. Sumit Verma and Saila Upadhyayula.

Over the past 3 years, she has established a comprehensive database of neuromuscular patients in MDA clinic which has attracted cutting edge multicenter clinical trials that are funded by both NIH and pharmaceutical sponsors – it is now open for enrollment in Duchenne/Becker Muscular Dystrophy (D/BMD) patients.

Because there is no known cure for DMD, current clinical trials offer treatments in a manner that allow hope for improvement of patients’ clinical outcome and thereby increasing survival rate. Due to the ongoing clinical trials at our site, we’ve attracted not only patients from our state but also neighboring states as well, increasing our reputation as a translational research center. Dr. Phan has also partnered with Emory Genetics on newborn screening projects for DMD and Cell Biology department on stem cell research for spinal muscular atrophy (SMA). These collaborations have emphasized her research interest in neuromuscular patients both on the molecular level and on clinical aspects to improve the clinical outcome of these patients.

A recent major focus of her work has been in collaboration with Centers for Disease Prevention (CDC) with the primary goal to establish SMA as a newborn screening condition. SMA is an autosomal recessive condition with incidence of 1 in 10,000 and carrier state of 1 in 40-60. It is a motor neuron disease, causing progressive weakness that began at birth and is considered to be the leading genetic cause of infant death. The infantile onset - SMA type I, clinical presentation and severity is dependent upon the copy number of survival motor neuron (SMN) copies numbers.

Working with CDC, Dr. Phan is involved in identifying the appropriate methodology that would allow for the diagnosis of SMA on dry blood spot, a biosampling of newborn blood obtained from pricking the heel or finger and blotted onto filter paper use widely as newborn screening for neonates. A new molecular therapy that increases *SMN2* functionality has shown promising results in clinical trials and is currently under investigation in presymptomatic newborns, indicating that use newborn bloodspot screening to identify affected infants at the earliest possible stage is paramount and near future of newborn screening for SMA.

Being a member of the Clinical Translational Advisory Committee (CTAC), she is given the opportunity to participate in the discussion of quality improvement in the many aspects of conducting clinical trials at Emory and Children’s Healthcare of Atlanta. Her goals are to continue with collaboration among the various departments within Emory University and with external institution including CDC to improve clinical outcomes in patients with neuromuscular disorders.



Graeme M. is a patient of Dr. Phan’s

To read a recent news story featuring Duchenne Muscular Dystrophy patient Addison Jones please, [click here](#)



After a year of planning and work, ACTSI (Atlanta Clinical & Translational Science Institute) is happy to announce the launch of a new brand. To keep pace with the NIH Clinical and Translational Science Award's changing mission we are proud to announce a [suite of new marketing tools](#). Today, ACTSI releases a new logo, website, and e-newsletter.

The new brand continues to target researchers and students and adds the state-wide public by offering educational information on clinical and translational research, news on how research affects our community, and resource and funding opportunities.

The new www.ACTSI.org eliminates program silos and brings ACTSI successes, mission, and resources to the forefront. Program organization was replaced with Community-Discover-Training. For example, find Community Engagement Research Program (CERP) resources in Community Resources, Clinical Research Network (CRN) resources in Discovery Resources, Research Education, Training, & Career Development (RETCD) resources in Training Resources, and so on. Each page contains a section specific spotlight, resources, and news.

A huge thank you to Emory's Web Design Group and each ACTSI website stakeholder and tester across all three academic partners and 10 ACTSI programs. Hopefully you will use these resources often. Please let me know if you have any questions or feedback. A planned communications survey will also help capture feedback.

Job Opening: Research Technical Specialist

Job Description: Functions as a skilled individual contributor or lead researcher on one or more research projects, serving as an expert in specialized area(s). Designs, develops and conducts large and/or complex research experiments. Reviews progress of research and evaluates results. Shares expertise in equipment operation and laboratory techniques. Refines/adapts methodologies to fit specific experiment requirements. Collaborates in the development of new techniques. Co-authors publications and may co-present findings with Principal Investigator at meetings/conferences. Performs related responsibilities as required.

PREFERRED QUALIFICATIONS: PhD in basic science area, at least 4 years post-doctoral experience; good written and verbal communication; excellent organizational skills; good working knowledge of Excel and PowerPoint, able to communicate with clinical investigators to help them draft biology aims and determine which molecular studies will be used to answer the clinical question; ability to synthesize data and help with grant and manuscript submissions. Be able to set up new assays with positive and negative controls..

To learn more, and apply, please [click here](#)

FRIENDS GRANT FUNDING OPPORTUNITIES

Applications due Monday, October 19th by 5:00pm

Members of Children's Professional Staff who do not receive compensation from Emory are eligible to apply

Quick Facts:

1. Funding Limit-\$25,000
2. Funding Term-6 -18 months
3. Post Award Expectations-must provide final report; must be willing to present finding to Friends groups, Children's leadership, etc.
4. Additional Information-fund does not provide for investigator salary support

For more information, including how to apply, please [click here](#)