# THE ART AND SCIENCE OF PEDIATRICS

### VOLUME 12, DECEMBER 2014



### PEDIATRIC INTEREST GROUP WEILL CORNELL MEDICAL COLLEGE





Weill Cornell Medical College

Dear Weill Cornell Medical Students and Faculty:

One measure of the quality and success of a special event lies in the support it generates in subsequent years. The reviews of all of previous Pediatric Research Days have been overwhelmingly positive. All in attendance at these events agree they are a tradition worth continuing.

On behalf of the Department of Pediatrics and the Weill Cornell Medical Student Pediatric Interest Group, it is a pleasure for me to welcome you to the Twelfth Annual Pediatric Research Day. In addition to medical student research project abstracts, this year's Journal "The Art and Science of Pediatrics," contains original essays by students about their experiences in pediatrics, and features on community service opportunities. The work presented in this journal and displayed at Pediatric Research Day is the product of a wonderful collaboration between our medical students and faculty committed to developing the next generation of pediatric scientists. What makes this work even more special is that our students accomplished this work in spite of the tremendous demands placed on their time by medical school. We believe this exposure to research early in one's medical career is an essential first step not only in launching a successful career in investigation but also in establishing a foundation for lifelong learning for those who choose to pursue clinical medicine.

As Chairman of the Department of Pediatrics, I congratulate the students and their faculty mentors on the success of their research efforts, and acknowledge the strong leadership of the Pediatric Interest Group – Rebecca D. DeMaria, Megan A. McGeehan and Khalil Anchouche - and their advisors, Drs. Susanna Cunningham-Rundles and Thanakorn Jirasevijinda, on organizing and continuing this important pediatric program.

Sincerely,

Aerald M. Loughli

Gerald M. Loughlin, M.D., M.S. Nancy C. Paduano Professor and Chairman Department of Pediatrics Weill Cornell Medical College

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### Acknowledgements

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Cover Art: Girl being treated for a broken leg, by Henley Mueller, age 9

Student Leadership: Khalil Anchouche, Rebecca DeMaria, and Megan McGeehan

Faculty Advisors: Susanna Cunningham-Rundles, PhD and Thanakorn Jirasevijinda, MD

## STUDENT WRITING FACULTY INTERVIEWS AND ESSAYS

#### INTERVIEW WITH DR. ERIKA ABRAMSON

#### Kristin Oshiro, Weill Cornell Medical College, Class of 2016

"Keep an open mind. I think it is very easy to pigeonhole yourself into a career path. Medical school should be a journey," said Dr. Erika Abramson, who is currently the Associate Director of Pediatric Graduate Medical Education. Dr. Abramson is very involved in both resident and medical student education. Last year, she served as the Associate Course Director for MPS I. This year, she is working on the new pre-clinical curriculum while teaching clerkship students and residents. Dr. Abramson spoke about her career journey, research interests, and roles within Weill Cornell in medical education.

#### How did you become interested in pediatrics and specifically general academic pediatrics?

I always had an inkling that I wanted to work with children. I remember always gravitating toward being a camp counselor or a baby sitter. Aside from working with children, I really liked the family-centered approach to pediatrics. I liked that you had the patient and the parents and grandparents, and you have to work as a team in order to take care of the patient. Also, I liked all of the people in pediatrics. I thought they were wonderful role models who were able to have work-life balance in the way I envisioned myself to do it.

I thought I was going to do subspecialty medicine until I came into residency. I ended up enjoying every rotation and organ system I came into contact with. In my chief resident year, when I still hadn't decided what I was going to do, it dawned on me that I could become a hospitalist. I could take care of everything and be the care coordinator for patients. It seemed like the most obvious choice, but it took me a long time to figure out.

#### Did you do research as a medical student? Was it in pediatrics?

In medical school, I did basic science research. I worked in a pediatric cardiology lab for two summers plus time in my third year. This experience allowed me to explore the basic sciences and showed me that everyone can work together on small aspects of a larger problem. It also taught me a lot about failure because not all of my experiments were successful. My PI taught me how to get up in front of an audience who didn't know what I was working on and be able to intelligently describe why my research was important. That was a very important and valuable skill set for me.

#### What kind of research did you do in residency at Cornell?

We have a research requirement for all pediatric residents. I totally switched gears and did a clinical project. I worked with another resident and looked at mothers at risk for anxiety and depression and their knowledge of basic newborn care skills compared to those who were not at risk. It was the first time I ever took something I was interested in and completed the project from start to finish. It was a game changing experience because this was the first time I felt internally driven to answer a research question.

### You have recently worked on numerous projects in Health Services. How did you become interested in this area?

In my chief resident year, I was involved in Quality and Safety in the pediatrics department. At the time, I worked a lot with Dr. Rainu Kaushal, who was in charge of Quality and Safety and is now head of Health Care Policy and Research. When I decided on academic medicine, I realized that research is a large part of that experience, and I didn't have enough skills to do it successfully. So I decided to do a Masters in Clinical Science Investigation here at Cornell. After this, I understood the Health Services research methodology, and I continued working on projects with Dr. Kaushal. I think Health Information Technology is very important in today's healthcare world. Some of the research I do is based on personal interest. Some of the research I do is because I believe you need to develop certain skills. Sometimes the projects where you can develop those research skills may not be what you would pick if you could have your sampling. These are great projects to gain skills from to later set up future opportunities.

#### What are your roles as Associate Director of Pediatric GME?

A large portion of my time is spent is working with residents in the advising and career development process. I am assigned a group of residents and meet with them at least twice a year, but it ends up being a lot more than that. I support them through personal, clinical, and career issues that they're dealing with.

I run the research program for residents, which is a huge task if you imagine 40 residents working on a project at a given time. Also, I head the Resident as Teacher curriculum. This is a program where residents work to become educators through experiential and didactic learning sessions. In their third year of residency, they have a rotation where they just have to teach medical students in the inpatient and outpatient settings.

Another set of my responsibilities involves teaching. I teach about 30 morning report conferences on different topics (Quality and Safety, Quality Improvement, High Value-Cost Conscious Care, Research, and the Resident as Teacher curriculum). I also go over teaching cases with interns and medical students on the floor.

#### What kind of research do you expect residents to participate in?

The goal is not to get every resident to become a researcher. Our hope is that through doing your own research, one can understand critical appraisal and know what is out there and whether it is valid research that is applicable to patients. Part of it is allowing residents to have exposure to a field that they are considering pursuing in the future. Many fellowships require research as part of their training, and about 75% of our residents go into fellowship. Research allows you to meet mentors, which is always valuable. We give our residents a lot of support and structure, and at least half of them go on to present their work at national conferences.

You recently worked on a project where residency program directors were surveyed about research training in their programs. Using these results, what improvements can be made to improve research in our residency program?

From that study, we realized that faculty development and mentoring are critical to research training success. Many faculty do not have specific mentorship skills because they have not focused on research themselves. So for the past year, I worked with a couple of groups to develop tool kits for faculty that provide them with resources to become better research mentors.

As a follow up to that study, I am doing a national survey where we are asking residents as well as their program directors about their thoughts on research and barriers to training. We are interested in this because every year the graduating residents are surveyed about different aspects of the program, and research training is universally the lowest scored area. They feel least prepared to do research and this has been stable for years, so we feel like this is an area we need to do better on.

#### Any advice for medical students?

When opportunities present themselves to you, think critically about them. It's always good to think about whether or not the skills you are going to develop are ones that will be helpful and more marketable for the future. Also, maintain work-life balance. I learned that it is very easy to lose sight of everything that was important to you in the past. But if you are not a well-rounded or happy person, you're not going to be the best doctor you can be. So try to maintain those relationships and make those phone calls because it can make a big difference.

#### INTERVIEW WITH DR. CHRISTOPHER CUNNIFF

#### Amanda Garfinkel, Weill Cornell Medical College, Class of 2018

Dr. Chris Cunniff, Weill Cornell Medical College's newly appointed Chief of the Division of Medical Genetics within the Department of Pediatrics, didn't always know he would become a physician. Raised in a small Alabama town, he applied to college in April of his senior year and, as a student at the University of Alabama, became the first member of his family to pursue a college education. Medicine, he said, "was sort of a dream I never thought could happen for me."

His vibrant interests in the humanities led him to major in English and Creative Writing, but he was surprised to find himself enjoying and excelling in his science courses as well. He recalls people telling him, "you're smart, Chris; maybe you want to become a doctor."

He remained at the University of Alabama to pursue his medical education. Though he entered with the intention to pursue a career in psychiatry, he found himself intrigued by the field of pediatrics, in which he could fill a different sort of counseling role. "In pediatrics," he said, "the health of your patient is being interpreted by somebody else—a parent. Instead of dealing with a patient, you're dealing with a system." He found a home within the subspecialty of medical genetics because he loved the unique sort of mystery solving he identified within the field; "genetics are exciting because there's always a puzzle to it and the diagnoses are rare, so I get to put some of my pattern recognition to use," he explained.

Dr. Cunniff was also drawn to the field by the complex, emotional situations that a pediatric geneticist is charged to navigate. Reflecting on his experiences, Dr. Cunniff said: "Even though I deal with topics that people consider to be sad—children with disabilities, defects, severe illnesses—I believe that I'm the right person for the job of explaining it to people in a way that makes it as manageable as possible. I've spent a lot of time trying to figure out ways to support people and to deliver difficult news." He believes that most of the techniques involved with this delicate skill arise from "things that your mother taught you"—simply being nice, using a person's name, smiling when appropriate or touching them on the shoulder—but he stresses how important it has been for him to keep reminding himself of these basic lessons.

While he takes great pride in his ability to comfort patients and families through difficult moments and conversations, he understands that sometimes, "parents just need to be angry with me." In those situations, he is willing to "accept the responsibility of being the focus of a parent's anger" because "maybe that will preserve their relationships with their spouse or child or primary care doctor—with people that they really need more than they need me, who they may only see once or a few times."

This emotionally delicate aspect of pediatric genetics has been "terrifically gratifying" for Dr. Cunniff. "I'm part of some of the huge dramas of somebody's life; I've got this incredible privilege to be part of what may be the most life-changing event they've ever experienced," he said.

While Dr. Cunniff has invested tremendous time, intellect, and emotion into his work with individual patients, he has also served in numerous administrative and leadership roles that have allowed him to contribute more broadly to the growth of his field and to the advancement of medical services and education. In roles including but not limited to Associate Director of the Arkansas Reproductive Health Monitoring System, Medical Director of the Genetic Counselor Training Program at the University of Arizona, and Director of Faculty Instructional Development at the University of Arizona College of Medicine, Dr. Cunniff learned many invaluable lessons about how to run an effective meeting, how to supervise people, and how to motivate people.

He identifies his work with the American College of Medical Genetics and Genomics as his favorite administrative role thus far. He was one of the Board's Founding Fellows when it was created in 1993. More recently, in 2009, when this organization created a specific board certification exam for the subspecialty of Medical Genetics, Dr. Cunniff lead the development of the written portion of the exam. This role sparked him to

think deeply about medical education. As he considered how to design a test that could assess doctors as "competent," he was forced to face the corresponding question of how to best educate doctors to be competent. The whole process, Dr. Cunniff stated, "led me to look at doctors and students and myself"; creating the test was "a microcosm that allowed me to see the broader picture of what it is that doctors need to do and learn and what it is my responsibility to teach."

With these reflections and experiences in mind, Dr. Cunniff was driven to take on new responsibilities, challenges, and opportunities by joining the Weill Cornell Medical College faculty in May of 2014. He is thrilled to be working alongside a number of research partners to build a robust translational genetics program here at WCMC. Research into this program comprises about 30 percent of his time, while the remainder of his time includes clinical work with individual patients, administrative duties in the Pediatric Division of Medical Genetics, and educational responsibilities in the medical college, including an appointment on the WCMC Admissions Committee. He is particularly excited to be a member of an educators' affinity group here, through which he has a space to continue thinking critically about the advancement of medical education, and he is greatly looking forward to contributing to the genetics component of the new curriculum as it continues to grow and develop in the coming years.

When asked, in conclusion, what advice he hopes to share with medical students, he replied: "I really want medical students to think that there are three types of knowledge that they are going to bring to the table in taking care of patients, and I want them to really consciously think of these three pieces as they go through their medical education." The first type of knowledge medical students need to build is "foundational knowledge." "Cranial nerves will always be cranial nerves," he said, and even when the amount of information seems overwhelming, building this foundational toolkit is important in the pursuit of any medical career or specialty. The second type of knowledge is what he calls "transactional knowledge," which incorporates the acknowledgement that what is practiced today will likely not be practiced tomorrow. "Learn what you need to know to practice today," he said, "but hold it lightly— continue to be the scientist that's skeptical. Use it as you have to, but always think to yourself, "maybe there's a better way." The last type of knowledge he finds critical for medical students to acquire relates to the topic of emerging information as medicine moves forward. "If you're going to be ultimately successful at solving problems," he explained, "you're going to need to know how to bring the newest information in, where to go and get it, and how to apply it."

The Pediatrics Interest Group warmly welcomes Dr. Cunniff to WCMC and is excited to have him as part of the faculty.

#### INTERVIEW WITH DR. ANNE MOSCONA

#### Michelle Lee, Weill Cornell Medical College, Class of 2018

Originally from Chicago, Dr. Anne Moscona's two passions growing up were science and music. Raised by parents in academic and basic science faculty, Dr. Moscona decided to pursue science and medicine in her early years at Harvard University and studied biochemistry and molecular biology. As an undergraduate researcher in Dr. Judah Folkman's laboratory at Harvard, she realized a passion for understanding fundamental disease mechanisms as well as a growing interest in primary care and healthcare in the developing world.

As a medical student at Columbia University College of Physicians and Surgeons, Moscona became heavily involved in community medicine and outreach, especially abroad. Her experiences working in jungle villages in Peru proved to be transformative and cemented a passion for advancing healthcare and care for children in developing and remote regions. Moscona completed her residency and fellowship at Mount Sinai Medical Center and continued to return to Peru multiple times to work towards improving access to vaccines, controlling infectious disease, and training native healthcare workers in basic care for children.

Moscona was drawn to pediatrics because of the many unknowns in child development, the potential to impact one's life through changes in the early years, and desire to improve understanding of how to care for children. As a resident, Dr. Anne Moscona decided to embark on a research career while also working on the clinical side of pediatrics. With a NIH-funded physician-scientist grant, now called a clinical Dr. Moscona was able to gain the rigorous grounding to start a medical research career in basic virology and pediatric respiratory viruses. For the last twenty years, she has conducted basic research on respiratory viruses that cause serious childhood diseases and on newly emerging paramyxoviruses that affect humans.

Dr. Anne Moscona is currently Attending Pediatrician at New York Presbyterian Hospital/Weill Cornell Medical Center, Professor of Pediatrics and Microbiology and Immunology, and Vice Chair for Research of Pediatrics at Weill Cornell. She joined Weill Cornell following 23 years at Mount Sinai where she was a Professor of Pediatrics, served as Division Chief of Pediatric Infectious Diseases and Vice Chair of Pediatrics for Research, Director of the Microbiology and Immunology course in the medical school and Associate Director of the MD/PhD program in the Graduate School of Biological Sciences.

Dr. Anne Moscona considers her pediatrician and scientist sides to be equally important to her and always tries see the links between the two sides and make them inseparable as possible. Some of the most rewarding aspects of her job are training other clinicians and researchers in further understanding viruses as well as helping families and children.

In her spare time, Dr. Anne Moscona continues her passion for music and plays piano and flute. She enjoys traveling and spending time with her family and two sons, who also enjoy music. This past summer, she traveled with her sons to Peru where she retraced her steps and hiked from village to village, revisiting many of the places precious to her.

#### **MY EXPERIENCE WITH CORNELL KIDS**

#### Khalil Anchouche, Weill Cornell Medical College, Class of 2017

Peering into the classroom, I saw thirty elementary school kids engaged in a cacophony of conversations. Loud and unapologetic, they were engrossed in their own universes, completely carefree. Their teacher, seated at the back, waited patiently for the class to begin, evidently thankful for the break from her teaching duties. Today, we were going to talk to the group about the digestive system. The challenge: bring to life an enormously complex and fascinating set of physiological processes, and hopefully spark the children's interest in science and medicine – all in under an hour. It wasn't going to be easy, but we had snacks.

How do you make science interesting? It seems like a simple, unassuming question, but in actuality is a rather difficult thread to unravel. Do you tell a story? Or do you string together facts? How do people really learn best anyway? Profound pedagogical deliberations aside, we were conflicted. We compromised, then, by settling on the idea that what we wanted to accomplish was show the class the brilliance underlying the organization and design of our organs, and the way in which amazing things can happen when all the parts of the system work together in harmony.

"When you eat a hamburger, where does the food go? How do we break it down?" A sea of hands erupted, and surveying their responses, I was impressed by the extent of their knowledge. "...the stomach, where it's digested, and then the intestines. There's a big one and a small one, but I don't remember which comes first. And then the food comes out when you go to the bathroom." The class broke out in uncontrollable laughter. They had this stuff down, essentially at the level of first-year medical students. We debriefed them on the relevant anatomy, crafting a number of "intricate" diagrams on the white boards, before jumping into the heart of the lesson.

As we described the journey of the aforementioned hamburger through the GI tract, we repeatedly emphasized what makes each organ so tremendously unique and amazing – and necessary for the successful completion of the digestive process. They were awed, for example, when we mentioned that the extreme acidity of the stomach continuously dissolves its inner lining, and that new cells replace the dying ones on a daily basis. "Why does that happen?" they wondered, puzzled. This didn't seem to make much sense, and so we spent a few minutes touching upon the stomach's role in protein breakdown. They were similarly incredulous when we revealed to them that the surface area of the small intestines, where nutrient absorption occurs, is over 2,700 square feet! "How does it fit in the body then?" they responded. Our discussion of the pancreas, liver and gallbladder was equally interactive.

They left, likely, with more questions than answers but at the end of the hour, I knew for a fact that they had learned more than they had bargained for. Unknowingly, they had come to appreciate what it is that makes the study of medicine so wonderful and challenging. In talking to them, I was reminded of that as well.

#### **RAISING THE BATON**

#### Pouya Jamshidi<sup>\*</sup>, Weill Cornell Medical College, Class of 2016

I am standing in front of the orchestra; there is complete silence. I look into the musicians' eyes and without a moment of hesitation I raise my baton to signal the start of Beethoven's 5th. I hear the famous 4 notes: *da da da daa*.

From the very first note of a concert, orchestra and the conductor commit to an exciting, demanding, and ultimately gratifying journey, wherein the destination is merely possible through mutual respect and trust. After all a conductor cannot make the sound without the musicians. During rehearsals, conductors determine the defining features of the piece, from the exact tempo of the music to the intonation and phrasing of a melody. They stand in front of the orchestra and move their arms in the air, creating the space for the players individually and the group as a whole to display their talent, share their emotions, and ultimately take the orchestra beyond technique into the domain of true musicality. Similarly, physicians do not heal their patients alone, but rather engage them and their family members to create an atmosphere of trust, where a therapeutic process can flourish. Although musicians largely practice alone – as physicians spend countless hours studying to conquer the fundamentals of human disease – rehearsal or concert is a shared experience, as is a visit to a physician's office.

My experience during the Pediatric Clerkship, where family-centered care was a core value is a perfect illustration of this collaboration between physicians and patients/families. In almost every encounter, communication occurs in a triad or larger group. The physician has to negotiate with the patient, parent(s) and any other family members in the room. Both information gathering and patient counseling need to take into account multiple voices, perspectives and values. The process of trust-building and give-and-take parallels how the conductor pulls together an ensemble of musicians to reach a common goal, beautiful music.

In addition to having an in-depth knowledge of the piece and understanding its attributes, conductors' success largely depends on the ability to cultivate the musicians' mastery of their instrument and to capture their imagination for something greater. "I have played my instrument for longer time than you have been alive", a seasoned Trumpet player once told me as he pulled me aside after my second rehearsal with the orchestra as a young conductor. "I know my child better than anyone else" is something that a parent would often declare. While pediatricians' knowledge of human disease is a requirement for pursuing a treatment plan, parents' intimate familiarity and strong bond with their child is an invaluable resource for advancing to successful outcome. Establishing connection with patients and their family members, listening to their problems and adjusting the care appropriately, isn't significantly different from the way a conductor can reflect on the mood of both the orchestra and the audience and adjust the performance, or play just the right encore.

Physicians step onto the conductors' podium when we enter the examination room. What makes the practice of Pediatrics uniquely similar to leading an ensemble is the fact that in addition to providing care for the sick child, the pediatrician is often invested in building a meaningful rapport with the family – fostering the essential parts for performing the melody of medicine—improving the human condition. It is truly exhilarating and deeply rewarding for me to be part of that ensemble, where individuals unite their collective egos into one entity to create something greater than sum of the members.

<sup>\*</sup>At the age of 19, Pouya served as the assistant conductor of the Tehran Philharmonic Orchestra in his native Iran. In 2008, he founded and conducted an international music ensemble called "Ensemble con Brio", when he was an undergraduate student at UC San Diego.

#### **REFLECTIONS ON FEMALE GENITAL MUTILATION IN TANZANIA**

Megan McGeehan, Weill Cornell Medical College, Class of 2017

I spent this summer in Mwanza, Tanzania at Weill Bugando Medical Center. This experience was filled with endless learning opportunities, from the cases we saw in the clinic to problems we encountered during field work. The most impactful experiences, however, were often tied to interactions that we had with fellow medical students during rotations; many times, I learned as much from students' personal experiences growing up in Tanzania as I did from a patient's medical history.

One day in clinic, a Tanzanian student was talking about her background, and she mentioned that she was a member of a tribe that expects their women to be circumcised. The student was able to escape circumcision with the help of her mother. This brief conversation made me immediately want to find out more about female genital mutilation (FGM) in Tanzania.

Kilimanjaro

Lindi

Mbeya

Nationally, FGM rates in Tanzania are 15%. They vary geographically, and differences can likely be explained by the cultural traditions of the tribes that settle in certain areas. Rates are the highest in the Manyara region, at 71%, and in Dodoma, at 64% (1). In a recent study in the Kilimanjaro region, prevalence rates were found to be 17%, slightly higher than the national average (2). These regions are located in north eastern Tanzania, as you can see on the map to the right.

The large majority of cases, at 97%, are Type I FGM, which is defined by the WHO as a clitoridectomy (2). Type II FGM involves the removal of the clitoris, labia minoria, and sometimes the labia majora. Only 1% of cases in Tanzania are type III FGM, which involves sewing the vagina shut (1).

The issue of FGM is of particular concern for the field of pediatrics. The median age at which a girl is circumcised in Tanzania is only 10 years old, and the majority of those who will be circumcised are cut by age 15 (2). Despite this young median age, nationwide, women are being cut earlier and earlier; in 2005, 28% of girls were cut before 1 year of age, but in 2010, 34% were cut before 1 year of age (1). Laws do exist in Tanzania against genital cutting, particularly the Sex Offenses National Provisions Act of 1998, which outlaws FGM for women under age 18 (3). However, it does not protect those over the age of 18. Additionally, the law has proven difficult to enforce, due to the challenges of patrolling rural areas, communities being unaware of the law, and women being reluctant to speak out against community members (3).

Though the age at circumcision is getting earlier and earlier, nationally, prevalence rates are declining. Rates of FGM dropped from 18% in 1996 to 15% in 2010 (1). Additionally, 76% of women in the Kilimanjaro study who had undergone FGM themselves intend to not perform FGM on their children, and younger women (<25) were less likely to circumcise their daughter than older women (>35) (2). Women who would circumcise their daughters cited perpetuation of tradition and the opportunity to teach about marriage and life as the most common reasons. Women who would not circumcise their daughters cited medical complications as the major reason, with 73% of women also citing that the "time has passed" (2). Of note, rural women are more likely than urban women to have a circumcised daughter, and poorer women are more likely than wealthier women to have a circumcised daughter (1).

The declining rates of FGM could be partially due to to increased awareness. Overall, 82% of women have heard of FGM. Almost all women in the Northern, Central, and Eastern zones have heard of FGM; this is attributed to the populations of Maasai and Chagga tribes in the North, and the Gogo and Nyaturu tribes in the Central zone, where FGM is common, as well as Dar es Salaam's location in the East, which is more urban and contains more advocacy action (1). Wealthier and more educated women are also more likely to have heard of FGM (1). Of the

women that have heard of FGM, 92% think that it should be discontinued. Only 6% believe that it should be continued, and 2% were unsure (1).

The issue of FGM was particularly compelling to me while living in Tanzania because of the lack of discussion surrounding the issue. In the hospital setting, I never once encountered a discussion of FGM except for that one student's brief story; this was shocking to me in light of the high prevalence and numerous medical complications surrounding female circumcision. On reflecting on the types of public health campaigns that I heard of while living in the country, including those for HIV/AIDS, male circumcision, malaria, TB, and schistosomiasis, I was surprised that I had not encountered campaigns aimed at decreasing rates of FGM. On expressing these thoughts to one of my supervisors, he mentioned that perhaps FGM takes a back seat when so much publicity, energy, and money is spent on more "well known" infectious disease issues that are more obvious to the public eye, such as HIV/AIDS, TB, and malaria, and FGM remains a more hidden, long-term problem. He also mentioned that male prominence in the country may play a role, often leading to women's health issues taking a back seat. While culturally embedded issues may be the hardest to change, declining rates and increased awareness of FGM can give us hope for the future.

If any students are interested in the Weill Bugando program, please feel free to contact me with any questions!

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## RESEARCH ABSTRACTS MEDICAL, GRADUATE, AND MD/ PHD STUDENTS

#### PREDICTORS OF MORTALITY AMONG PEDIATRIC BURN PATIENTS IN SUB-SAHARAN AFRICA

<u>Khatiya Chelidze (Class of 2017)<sup>1</sup>, Christopher Lim (Class of 2017)<sup>1</sup>,</u> R. Peck, MD, DTM&H<sup>2</sup>, G.Giiti, MD, MMed<sup>2</sup>, N Leahy, RN, MPH<sup>2</sup>, A. Rabbitts, MS, RN<sup>2</sup>, R. Yurt, MD, FACS<sup>2</sup>, J. Gallagher, MD, FACS<sup>1,2</sup>, Katrina Mitchell, MD<sup>1,2</sup>

<sup>1</sup>Surgery, Weill Cornell Medical College-New York Presbyterian Hospital, New York, NY/10065, United States,<sup>2</sup>Surgery, Weill Bugando Medical Center, Mwanza, Tanzania

**Background**: Little is known about the outcomes of pediatric burn patients in sub-Saharan Africa, particularly in more rural settings. In March 2013, our East African pediatric burn unit established an electronic registry of all patients. In this analysis, we aimed to determine overall mortality rates and predictors of mortality.

**Methods**: The secure electronic database of all admissions was reviewed for age, gender, weight, %TBSA, body part burned, cause/place of injury, length of stay (LOS), nutritional status, surgery performed, reason for discharge, and mortality. Stata v 13.0 was used for data analysis. Categorical variables were presented as proportions (percentages) and compared using Fisher's exact test. Continuous variables were presented as medians [interquartile ranges] and compared using Wilcoxon's rank sum test. Univariable and multivariable models were analyzed using logistic regression to determine the variables associated with mortality; the magnitude of risk was measured by the odds ratio (OR) at 95% confidence interval (95% CI). The final logistic regression model for prediction of mortality was built using forward selection (inclusion criteria of p<0.20).

**Results**: A total of 211 cases (59.7% male) admitted from March 2013 – June 2014 were reviewed. The median age, %TBSA and LOS were 2.0 years [1.3-3.3], 8.0% [5.0-13.4], and 8.5 days [4-14]. The overall mortality rate was 15/211 (7.1%). Most injuries were unintentional (93.8%) scalds (85.3%) occurring in the home (98.1%). Seventeen children underwent surgery. Mortality was 15/211 (7.1%).

In the univariable analysis, factors significantly associated with mortality were higher %TBSA and burns classified as "major" (p<0.01 for both). Facial burns were significantly associated with mortality in the univariable analysis, but were not significant after adjusting for TBSA (p=0.54). Underweight was not associated with mortality. The final multivariable model for prediction of mortality included 4 variables: %TBSA (OR=1.31 for 1% increase in %TBSA; 95% CI: 1.17-1.46), age (OR=0.20; 0.07-0.63), female gender (OR=3.49; 0.75-16.17), and weight (1.36 for 1kg increase in weight; 0.91-2.03).

**Conclusions**: This study characterizes mortality among patients in a sub-Saharan Africa pediatric burn unit in a rural setting. The majority of pediatric burns were unintentional scalds occurring in the home. %TBSA and lower age were the strongest predictors of mortality. Overall mortality was 7.1%. These data are applicable to improving outcomes for patients in this sub-Saharan African burn unit and similar settings of its kind.

#### PREDICTING FREQUENT EMERGENCY DEPARTMENT (ED) VISITS AMONG CHILDREN WITH ASTHMA USING ELECTRONIC HEALTH RECORD DATA

Lala Tanmoy Das<sup>1,2</sup>, Erika L Abramson, MD, MS<sup>3,5</sup>, and Zachary M Grinspan, MD, MS<sup>3,4</sup>

<sup>1</sup>Center for Healthcare Informatics and Policy, Weill Cornell Graduate School of Medical Sciences, New York; <sup>2</sup> Greenberg Division of Cardiology, Weill Cornell Medical College, New York; <sup>3</sup> Department of Healthcare Policy and Research, Division of Health Policy and Economics, Center for Healthcare Informatics and Policy, Weill Cornell Medical College, New York; <sup>4</sup> Department of Pediatrics, Division of Child Neurology, Weill Cornell Medical College, New York; <sup>5</sup> Department of Pediatrics, Weill Cornell Medical College, New York

**Background**: Asthma is one of the most common chronic conditions among children and is the third leading cause of pediatric hospitalization among patients under age 15. Asthma-related unnecessary ED visits are common and expensive to the health system. One of the proposed cost savings measures is for hospitals to hire care managers who can coordinate care for individuals with chronic diseases such as asthma by informing them about general preventative care practices, reconciling medications, and answering questions. It is unclear if electronic health record (EHR) data can be used to predict which patients will frequently use the ED for asthma, who can then be enrolled in care management programs.

**Objective**: To generate a list of possible predictors of frequent ED use that can subsequently be extracted from the NYP-WCMC's EHR for use in predictive modeling.

**Design**: We interviewed 5 physicians affiliated with NYP-WCMC to help identify potential predictors of ED use based on clinical experience. Three of them were general pediatricians, one was a pediatric pulmonologist, and one was in pediatric emergency medicine. Predictors were categorized under five broad categories; namely, Demographics, Medications, Clinical Data, Comorbidities, and Prior Health Service Utilization. Subsequently, list of predictors was reviewed with content experts (ZG & EA).

**Results**: Sixty potential predictors were identified from the interviews and classified under 9 broad subcategories. The sub-categories were as follows: Individual Characteristics, Disease Factors, Family Characteristics, Zip Code Characteristics, Environmental Factors, Medication History, Comorbidities, Ambulatory & ED Service Utilization, Inpatient Admission Characteristics. Examples of predictors include age, gender, insurance status, asthma severity, estimated median household income based on zip code, exposure to secondhand smoke, allergens at home, albuterol use, total number of ED visits, and total inpatient admissions.

**Conclusions**: A list of 60 potential predictors was compiled from physician interviews. In our ongoing work, these predictors will be operationalized and data elements will be extracted from NYP-WCMC's outpatient and inpatient EHR systems to create a database. Once the database is created and validated, we will evaluate and compare the performance of machine-learning algorithms to predict which cohorts of pediatric asthma patients will use the ED four or more times in the following year.

#### FIRST REPORT OF COCHLEAR IMPLANTATION IN A PATIENT WITH NARROW DUPLICATED INTERNAL AUDITORY CANALS

Wesley L. Davison (Class of 2016)<sup>2</sup>, Myles F. Melton<sup>2</sup>, Michelle L. Kraskin<sup>3</sup> and Kevin D. Brown<sup>1</sup>

<sup>1</sup>Department of Otolaryngology-Head and Neck Surgery, Weill Cornell Medical College and New York Presbyterian Hospital, New York, NY, USA; <sup>2</sup>Weill Cornell Medical College, New York, NY, USA; <sup>3</sup>Hearing and Speech Center, Weill Cornell Medical College, New York, NY, USA

**Objective:** To document the course and outcome of pediatric CI in a patient with duplicated IACs and Oculo-Auriculo-Vertebral (OAV) syndrome.

**Setting:** Tertiary care hospital

Patient: A 4-year old female with hearing loss

Intervention: Left Cochlear Implant

**Results:** Left CI implantation performed with perimodiolar electrode was uneventful. An extended round window insertion was performed. At her 3 week activation the patient was able to repeat /ba/ /m/ /sh/ and /ah/ 3/3 times with visual cues. At 1 month responses were in the mild range with a 25dB SAT.

**Conclusion:** This is the first ever reported cochlear implantation in a patient with duplicated IACs. Even though CI is possibly contraindicated in the presence of narrow IACs, this patient was successfully implanted and has shown marked improvement in hearing abilities.

#### EVALUATING COMPLIANCE WITH OXYGEN SATURATION TARGETS IN THE NICU

Rebecca DeMaria (Class of 2017)<sup>1</sup>, Vivien L. Yap, MD<sup>1, 2</sup>

<sup>1</sup> Weill Cornell Medical College, New York, NY; <sup>2</sup>Department of Pediatrics, Division of Newborn Medicine, New York Presbyterian-Weill Cornell Medical College, New York, NY

**Background**: Some of the consequences of premature birth, such as retinopathy of prematurity (ROP), are linked to the management of supplemental oxygen therapy. While the optimal oxygen saturation (SpO<sub>2</sub>) range to maintain very premature infants to minimize the risk of complications while optimizing benefit has been unclear, the NICU at New York Presbyterian (NYP) Weill Cornell Medical Center (WCMC) had target SpO<sub>2</sub> guidelines for infants on supplemental oxygen at 88-92% for infants <32 weeks gestational age and 90-95% for infants  $\geq$ 32 weeks gestational age. Recent trials have indicated that lower levels of SpO<sub>2</sub> (85-89%) in very preterm infants may be associated with increased mortality, while higher levels (90-95%) are associated with increased risk of ROP. These trials have prompted a review of current care practices in our NICU, in order to inform new SpO<sub>2</sub> guidelines. The objective of this retrospective review is to document achieved SpO<sub>2</sub> levels and compare these with the intended SpO<sub>2</sub> range as described in the guidelines.

**Methods:** De-identified SpO<sub>2</sub> and FiO<sub>2</sub> data from hourly oxygen monitoring were extracted from the medical record for all preterm and term infants admitted to the NICU in 2013. Data for infants receiving oxygen support (FiO<sub>2</sub> > 0.21) were compared to NYP-WCMC NICU SpO<sub>2</sub> guidelines. Gestational age was not available, so target ranges were combined to 88-95%.

**Results:** Extraction of hourly SpO<sub>2</sub> readings for infants receiving oxygen support (FiO<sub>2</sub> > 0.21) yielded 17,469 data points. The mean FiO<sub>2</sub> for infants on supplemental oxygen was 0.42. The mean SpO<sub>2</sub> was 94% (SD 5.9%), and the median SpO<sub>2</sub> was 95%. Oxygen saturation was below 88% in 9% of the recorded levels, between 88-95% in 50% of the recorded levels, and above 95% in 41% of the recorded levels.

**Conclusions:** Infants receiving supplemental oxygen in the NYP-WCMC NICU in 2013 had  $SpO_2$  levels in the targeted range in only half of recorded values. Most noncompliance was above the target range, with the median  $SpO_2$  at the upper limit of the target range. Limitations of this review include the lack of clinical information that may have altered the intended  $SpO_2$  range for some patients. Future directions include new target  $SpO_2$  guidelines based on current evidence, and changes in care practices. We will review similar data in 2015 following implementation of new guidelines.

#### EVALUATION OF CED TREATMENT RESPONSE IN PATIENTS WITH DIFFUSE INTRINSIC PONTINE GLIOMAS USING MR SPECTROSCOPY

Daniela Guisado, B.S. (Class of 2017)<sup>1,2\*</sup>, Ranjodh Singh, B.Phil.<sup>1</sup>, Shlomo Minkowitz, M.D.<sup>1,4</sup>, Mark Souweidane, M.D.<sup>3,5</sup>, A. John Tsiouris, M.D.<sup>4</sup>, Robert Young, M.D.<sup>1,4</sup>, Kyung Peck, Ph.D<sup>1,2</sup>, Sunitha Thakur, Ph.D<sup>1,2</sup>, Sofia Haque, M.D.<sup>1,4</sup>

<sup>1</sup>Department of Radiology, <sup>2</sup>Department of Medical Physics, <sup>3</sup>Department of Neurosurgery Memorial Sloan-Kettering Cancer Center, New York, NY 10065; <sup>4</sup>Department of Radiology, <sup>5</sup>Department of Neurosurgery Weill Cornell Medical College, New York, NY 10021

**Background:** Diffuse intrinsic pontine gliomas (DIPG) are inoperable, high-grade gliomas that account for 75-80% of pediatric brainstem tumors. The current standard of care for DIPG is standard radiation therapy and despite numerous efforts, median survival is <1yr from time of diagnosis. The relatively intact blood-brain barrier (BBB) presents a major hurdle in drug delivery to DIPG. Convection-enhanced delivery (CED) is a local drug delivery technique that bypasses the BBB and allows for enhanced and uniform drug distribution, optimized therapeutic indices, and greatly reduces systemic toxicity to patients. In the current study, magnetic resonance spectroscopy (MRS) was used to evaluate local treatment response in non-progressive DIPG patients undergoing CED of radioactive antibody <sup>124</sup>I-8H9.

**Methods:** We retrospectively analyzed the MRS data for patients diagnosed with DIPG who received <sup>124</sup>I-8H9 CED dose level 4 (1mCi). Patients were diagnosed with DIPG based on clinical symptoms and imaging results, and received standard radiation therapy at least 9 weeks prior to CED treatment. MRS imaging was obtained at 3 or more time points: before treatment (preCED), one day after treatment (postCED1) and 1 month after treatment (postCED2). Several regions of interest were chosen to analyze MRS data including the CED injection site, tumor, and normal tissue voxels.

**Results:** The MRS data for these regions of were analyzed and the Choline/NAA metabolite ratio was determined to be the most informative parameter and was compared across the time course of treatment to evaluate tumor response. MRS results were consistent with clinical outcomes in dose level 4 patients, and can provide additional information on local treatment response.

**Conclusions:** Future studies will expand this analysis to previous dose levels to differentiate tumor response from the CED treatment from the ongoing effect of radiation therapy, and to explore other imaging modalities.

#### EFFECTIVENESS OF A SIMULATION CURRICULUM ON CLINICAL EXCECUTION: A PILOT STUDY

Ilana Harwayne-Gidansky<sup>1</sup>, Jenifer Garnett<sup>1</sup>, Son McLaren<sup>1</sup>, Kristen Critelli<sup>1</sup>, <u>James Wang (Class of 2016)<sup>1</sup></u>, Kevin Ching<sup>1</sup>

#### <sup>1</sup>Department of Pediatrics, Weill Cornell Medical College, New York Presbyterian Hospital, Komansky Children's Center, New York, United States

**Background**: Procedural skills acquired through simulation training have been shown to transfer into clinical practice. Evidence for the transfer of medical decision-making skills is limited. Pediatric residents are expected to learn and utilize a clinical prediction rule derived and validated by PECARN (Pediatric Emergency Care Applied Research Network) to determine whether children with head trauma need neuroimaging to identify a traumatic brain injury. Although straightforward to learn, the quick decisions made to image children with head trauma in a busy pediatric emergency department (PED) may rely on an imprecise recollection of this rule – potentially subjecting children to unnecessary radiation from CT scans.

We predicted that when evaluating real children in a PED, interns who had participated in a high-fidelity immersive simulation exercise illustrating the use of this PECARN rule would implement this rule more accurately than residents who had not participated in this simulation. Our primary outcome was the accuracy of interns when using the PECARN rule to evaluate real children in a PED.

**Design/Methods**: Single center, blinded prospective randomized-controlled pilot trial implemented for the 2013-14 academic year. All interns completed a written pretest and were randomized to participate in a PECARN head trauma simulation or an unrelated simulation control of acute intracranial hypertension. For the next 12 months, any implementation of this rule by interns or senior residents in the PED was compared using a standardized observation tool.

**Results**: Senior residents were able to correctly identify 57% of the PECARN criteria when evaluating children with head trauma while interns in the intervention group were able to correctly identify 64% of the PECARN criteria, compared with 43% in the control group (p=0.028).

**Conclusion**: Although not statistically significant, our preliminary proof of concept data suggests that a single simulation exercise may be superior to routine bedside teaching when used to train interns on how to correctly implement a validated clinical decision rule about neuroimaging in head trauma. This study suggests that we may improve the efficiency of information delivery through simulation, and has promising implications for future larger studies.

#### NOVEL DE NOVO MUTATIONS IN KIF1A AS CAUSE OF COMPLICATED HEREDITARY SPASTIC PARAPLEGIA

Leslie Hotchkiss, MS (Class of 2016)<sup>1,2</sup>; Sandra Donkervoort MS, CGC<sup>1</sup>; Meganne Leach, CRNP<sup>1,3</sup>; Payam Mohassel, MD<sup>1</sup>; Nathaniel Bradley, BS<sup>1</sup>; David Nguyen, BS<sup>1</sup>; Ying Hu, MS<sup>1</sup>; Juliana Gurgel-Giannetti, MD<sup>4</sup>; Carsten Bönnemann, MD<sup>1</sup>

<sup>1</sup>National Institutes of Heath, <sup>2</sup> Weill Cornell Medical College, <sup>3</sup> Children's National Medical Center, <sup>4</sup> Universidade Federal de Minas Gerais, Brazil

**Introduction:** Hereditary spastic paraplegias (HSPs) are a clinically and genetically heterogeneous group of disorders characterized by lower extremity spasticity and weakness. Complicated HSP can present with more extensive central nervous system involvement. To date, 55 spastic paraplegia genes (*SPGs*) have been identified and new gene discoveries are rapidly emerging (Lo Giudice et al., 2014). *KIF1A* encodes for kinesin family 1A (KIF1A) which plays a role in axonal transport in neurons. Recently, the first *de novo* mutations in *KIF1A* were identified in patients with early onset HSP characterized by cognitive impairment, spastic paraparesis, axonal neuropathy, optic nerve atrophy and cerebellar atrophy (Lee et al., 2014).

#### Case descriptions:

*Patient 1* is a 14-year-old male from Saudi Arabia with global developmental delay, cerebellar atrophy, visual impairment and mixed findings of spasticity and hypotonia. First concerns arose at age 6 months when he was unable to roll over and was noticed to have poor head control. He made slow gains in motor function, but never achieved ambulation. He was severely cognitively impaired. He was spastic in the lower extremities, but hypotonic and areflexic in the upper extremities. At 16 years old, he developed seizures. Magnetic resonance imaging (MRI) of the brain at 11 years old showed cerebellar atrophy, thinning of the posterior corpus collosum and small optic chiasm and tracts. N\*\*rve conduction studies (NCS) were consistent with a distal neuropathy with decreased sensory response. Additionally, he had some minor scoliosis and multiple contractures of elbows, wrists, fingers, and knees.

*Patient 2* is a 6-year-old boy from Brazil with global developmental delay, a progressive cerebellar atrophy, optic nerve atrophy, spastic paraplegia, axial hypotonia, and distal neuropathy. He was born with bilateral clubfoot deformities. At 6 months of age, he was unable to roll over or sit and had poor head control. He never achieved ambulation. He had severe cognitive impairment and delayed language development. At age 1 year he was diagnosed with optic nerve atrophy. He had truncal hypotonia with distal spasticity, particularly in his hands. He was hyperrflexic and hypertonic in the lower extremities. He also has mild kyphosis and contractures in the ankles with continued clubfoot deformity. MRI at 1 years of age was reportedly normal, but at 5 years of age his MRI showed cerebellar atrophy. NCS at age 1 reportedly suggested an axonal sensory-motor polyneuropathy. Exome sequencing identified novel heterozygous missense mutationas in *KIF1A* at c.902G>A (p.R307Q) for patient 1 and at c.843>T (p.G199R) for patient 2. These mutations were confirmed by Sanger sequencing. Parental segregation was negative.

**Discussion:** Here we report 2 novel *KIF1A* mutations resulting in complicated HSP characterized by (1) cognitive impairment, (2) non-ambulation, (3) language delay/non-verbal, (4) optic nerve atrophy, (5) peripheral neuropathy, and (6) cerebellar atrophy on MRI. This phenotype is most consistent with the report of *de novo KIF1A* mutations, whereas earlier reported homozygous mutations produced less severe phenotypes. This is the first confirmatory report of *de novo KIF1A* mutations causing disease. Both patients were found to have missense mutations in the motor domain of *KIF1A* (amino acids 1-361) (Lee et al., 2014). KIF1A's function relies on efficient ATP hydrolysis, which produces conformational change, for movement, and a strong interaction with the microtubule scaffolding via three microtubule domains. The mutations in these two patients fall within the "hotspots" of the motor domain that carry out theses essential functions are most likely to be disease causing.

#### NECESSITY OF THERAPEUTIC PROPHYLAXIS IN FETAL/NEONATAL ALLOIMMUNE THROMBOCYTOPENIA ANTENATALLY IN FIRST PREGNANCIES

### Jenny C Jin (Class of 2016)<sup>1</sup>, Polina Ferd<sup>1</sup>, Karen Manotas<sup>1</sup>, Madhavi Lakkaraja<sup>1</sup>, Julia Gabor<sup>1</sup>, Megan Wissert, RN-BC<sup>1</sup>, Richard L. Berkowitz, M.D.<sup>2</sup>, Janice G McFarland, MD<sup>3</sup> and James B Bussel, M.D.<sup>1</sup>

<sup>1</sup>Department of Pediatric Hematology/Oncology, New York Presbyterian Hospital/Weill Cornell Medical College, NY; <sup>2</sup>Department of Obstetrics and Gynecology, Columbia University Medical Center, NY; <sup>3</sup>Platelet and Neutrophil Immunology Laboratory, Blood Center of Wisconsin, WI

**Background:** Fetal/Neonatal Alloimmune Thrombocytopenia (FNAIT) is a potentially life-threatening condition caused by a parental human platelet antigen incompatibility with resulting maternal alloantibodies against fetal platelets; studies have shown that this occurs in 1 in 1,000 live births. Diagnosis based on laboratory testing usually follows the birth of the first affected (thrombocytopenic) neonate. Mothers of FNAIT fetuses can be treated during subsequent affected pregnancies with intravenous immunoglobulin (IVIG) with or without prednisone to increase fetal platelet counts and avoid the most serious complication: intracranial hemorrhage (ICH) occurring in 10-20% of affected children, 50-75% of which occur in utero. FNAIT is analogous to hemolytic disease of the newborn (HDN); however, it has been thought that up to 60% of cases of HPA-1a sensitization occur during the first pregnancy. Recently, a large screening study in Norway suggested that in 75% of cases of FNAIT, sensitization occurs at delivery of the first HPA-1a newborn. This more closely resembles HDN than had been previously thought. Currently HDN management is achieved by universal screening and prophylaxis with Rh<sub>o</sub>(D) Immune Globulin for Rh-negative women primarily at delivery but also at 26-28 weeks. This study attempted to determine what fraction of severely affected FNAIT women had a first affected pregnancy, thus necessitating antenatal prophylaxis.

**Methods:** The birth order of FNAIT-affected pregnancies among 62 available of 138 mothers who had been enrolled in antenatal treatment studies was determined through phone and mailed surveys and retrospective chart reviews. Miscarriages, elective terminations, amniocentesis procedures, and order of affected pregnancies for each mother were recorded to determine if the first affected child was indeed the first pregnancy for each mother.

**Results:** (Table 1) Pregnancies affected with FNAIT were the true first pregnancies of 57% (35 mothers) of 62 surveyed mothers; these 35 mothers did not have elective terminations, miscarriages, or unaffected children before their first FNAIT-affected pregnancy. Nineteen mothers (31%) had miscarriages or terminations before their first FNAIT-affected pregnancy and 8 (12%) had apparently unaffected pregnancies before their first FNAIT-affected pregnancy and 8 (12%) had apparently unaffected pregnancies before their first FNAIT-affected pregnancy. 76 mothers were lost to follow up, declined to participate, or thus far did not complete surveys.

**Conclusions:** Over 50% of the surveyed FNAIT mothers enrolled in our antenatal treatment study had FNAIT first-affected pregnancies, meaning these mothers were sensitized by the time of delivery of their first child. Thus, even with universal screening, prophylaxis would have a high failure rate if instituted only at birth. Antenatal and peripartum prophylaxis during the first pregnancy appear to be necessary for HPA-1a women at risk for FNAIT to avoid fetal ICH and antenatal intravenous immunoglobulin and prednisone treatment of a subsequent affected pregnancy. Universal screening of primigravida females appears necessary to identify mothers at risk of FNAIT who may benefit from prophylaxis. Efficacy and safety of prophylaxis, including dosage, procedure, and benefits remain to be determined.

Ť	Mothers (n)
True first affected child	35
Miscarriage or termination before first affected child	19
First affected child was not first pregnancy; no miscarriages or abortions	8

#### Table 1: FNAIT-Affected Pregnancies

#### DELIRIUM IN CRITICALLY ILL CHILDREN

#### Abigail Kerson (Class of 2017)<sup>1</sup>, and Chani Traube, MD<sup>1</sup>

#### <sup>1</sup>Pediatric Critical Care, Weill Cornell Medical College-New York Presbyterian Hospital, New York, NY/10065, United States

**Background**: Delirium is acute neurologic dysfunction, associated with severe illness or its treatment. It has long been recognized in the adult intensive care unit population where it is associated with increased mortality and length of hospital stay. Preliminary studies suggest substantially increased morbidity in critically ill children, although data has been limited by the lack of prospective universal screening in the pediatric ICU (PICU). With the recent advent of pediatric delirium screening tools, we can investigate the extent of this problem.

**Design/Methods**: This is a prospective study of children admitted to the New York Hospital Weill-Cornell Medical Center PICU in the summer of 2014. Daily data was collected from the electronic medical record of consecutive PICU admissions over the course of 8 weeks. Every child was routinely screened for delirium twice daily using the Cornell Assessment of Pediatric Delirium (CAPD), a rapid observational screening tool scored by the bedside nurse. Children who screened positive were diagnosed with delirium. Children with developmental delay and a positive delirium screen required an assessment by a pediatric intensivist or pediatric psychiatrist to confirm the diagnosis. A child was considered delirious until they had two sequential negative CAPD scores (24 hours delirium-free).

**Results**: One hundred and forty four subjects were included in the data collection, which comprised 818 patient days. Of the 144 subjects, 28 were excluded due to absence of documentation of delirium screening. Incidence of delirium in the remaining 116 subjects was 28.4% (33 subjects were ever delirious during their PICU stay). In the subjects that were ever delirious, the average time in the PICU until the development of delirium was 3.7 days with a standard error of .55 days. Thirty of the 116 subjects were developmentally delayed, and 86 were not. The incidence of delirium in developmentally delayed children was 53.3%, as compared to 19.8% in typically developed children (p<.003)

**Conclusions**: This prospective longitudinal study shows a cross-section of delirium in a single PICU over a 2 month period. The data show that delirium may be prevalent in critically ill children, and developmentally delayed children are at higher risk. Further investigation is necessary to determine the scope of this problem, identify its associated risk factors, and target possible avenues for therapeutic intervention.

#### BIRTH WEIGHT OF NEONATES WITH FETAL AND NEONATAL ALLOIMMUNE THROMBOCYTOPENIA (FNAIT)

#### Madhavi Lakkaraja<sup>1</sup>, Jane Selegan<sup>1</sup>, <u>Jenny Jin<sup>1</sup> (Class of 2016)</u>, Karen Manotas<sup>1</sup>, Polina Ferd<sup>1</sup>, Julia Gabor<sup>1</sup>, Cheryl Vinograd<sup>1</sup>, Megan Wissert<sup>1</sup>, Richard L. Berkowitz<sup>2</sup>, Janice G. McFarland<sup>3</sup>, James B. Bussel<sup>1</sup>

<sup>1</sup>Department of Pediatric Hematology/Oncology, New York Presbyterian Hospital/Weill Cornell Medical College, New York, NY, United States; <sup>2</sup>Department of Obstetrics and Gynecology, Columbia University Medical Center, New York, NY, United States; <sup>3</sup>Platelet and Neutrophil Immunology Laboratory, Blood Center of Wisconsin, Milwaukee, WI, United States

**Background:** Incompatibility between parental platelet antigens, especially HPA-1a, leads to fetal and neonatal alloimmune thrombocytopenia (FNAIT). Antenatal treatment of FNAIT involves treating the pregnant mother with intravenous immunoglobulin (IVIG) +/- steroids. Antenatal treatment is stratified depending on whether the previous affected sibling had an intracranial hemorrhage (ICH). A Norwegian study reported lower than expected neonatal birth weight in affected males with FNAIT identified by universal screening. Villitis and inhibition of angiogenesis have been suggested as potential mechanisms for low birth weight in FNAIT-affected children since the  $\beta$ 3 integrin is expressed by syncytiotrophoblasts.

**Objectives**: (1) To compare birth weights of FNAIT neonates whose mothers received antenatal treatment to untreated neonates (first-affected siblings) (2) To assess the role of gender in birth weight in FNAIT.

**Methods:** This is a retrospective chart review of 118 women who gave birth to 126 fetuses enrolled in an ongoing study trial ClinicalTrials.gov number NCT00194987. Mothers who discontinued the study and neonates with missing birth weight were excluded. The "treated child" was had received antenatal treatment for FNAIT and the "untreated first-affected sibling" was not known to have FNAIT during pregnancy. For each neonate, difference between the birth weight and the expected weight appropriate for gestational age was obtained. This difference was compared between treated children and untreated siblings, and between males and females in each group. Data was summarized using means, medians and statistical analysis was performed using Wilcoxon rank sum test.

**Results:** Birth weights of 119 treated children and 86 untreated first-affected siblings were analyzed. Treated children weighed more than untreated first-affected siblings. Among treated fetuses, there was no difference in the birth weights between males and females. However, unexpectedly among untreated (first-affected) siblings males had a significantly higher birth weight than females. There was no correlation between birth weight and either birth platelet count of the neonate or maternal IgG levels.

**Conclusions**: Antenatal treatment of FNAIT may increase the birth weight of the affected fetus. The inconsistency in gender-related findings between our results and those of the Norwegian study indicates these findings are uncertain. Birth weight was lower in untreated children suggesting that antenatal treatment may negate the disease effect leading to improvement in birth weights of FNAIT-affected children. The mechanism of lower birth weight and of its improvement in treated fetuses needs to be studied.

#### CLINICAL INVESTIGATION OF FACTORS AFFECTING THE DEVELOPMENT AND OUTCOMES OF HEAD AND NECK SQUAMOUS CELL CARCINOMA IN FANCONI ANEMIA PATIENTS

<u>Krupa R. Patel BA (Class of 2017)</u><sup>1</sup>, Agata Smogorzewska MD PhD<sup>2</sup>, Arleen D. Auerbach PhD<sup>2</sup>, Jennifer Kennedy MS<sup>2</sup>, Francis P. Lach BS<sup>2</sup>, Erica Sanborn MS<sup>2</sup>, Marc A. Cohen MD<sup>1</sup>, William I. Kuhel MD<sup>1</sup>, David I. Kutler MD<sup>1</sup>

#### <sup>1</sup>Weill Cornell Medical College, <sup>2</sup>The Rockefeller University

**Background:** Fanconi anemia (FA) is a rare DNA repair disorder characterized by progressive bone marrow failure and increased susceptibility to hematologic and epithelial malignancies. Improvements in hematopoietic stem cell transplant (HSCT) outcomes for FA patients have resulted in a rising percentage of patients surviving into adulthood and a simultaneously increasing number of FA patients afflicted with head and neck squamous cell carcinoma (HNSCC). This study investigates the relationship between genetic and treatment-related factors and the development and outcomes of HNSCC in FA patients.

**Methods:** The International Fanconi Anemia Registry (IFAR) at the Rockefeller University is a prospectively collected database of genetic and clinical information for FA patients. A total of 35 patients met the inclusion criteria including a diagnosis of FA and HNSCC. The Kaplan-Meier method and log rank test was used to evaluate differences in the age of HNSCC onset between patient subgroups and to estimate overall, cause-specific and disease-free survival.

**Results:** We studied 35 FA patients (22 female, 13 male) with HNSCC. The median age for development of HNSCC was 30.1 (range 14 to 48) years. The most common cancer site was the oral cavity (26/35) followed by the larynx (6/35). Thirteen patients (37%) had received a HSCT for hematologic symptoms prior to HNSCC diagnosis. Twenty-seven (77%) patients died during the study period and the median lifespan for this cohort was 36.4 years. Most patients (74%) had no history of tobacco use or alcohol consumption. Kaplan-Meier analysis of survival until HNSCC onset indicated a statistical difference between patients with a history of HSCT and non-HSCT patients (p=0.012). The median age of HNSCC onset for non-HSCT patients was 34.8 years whereas that for HSCT patients was 26.1 years. Patients who received irradiation as a part of the HSCT conditioning regimen had a median age of HNSCC onset of 24.6 years. Most patients (83%) underwent surgical resection of the primary tumor. Twelve patients received adjuvant radiation therapy with mixed outcomes and the addition of conventional chemotherapy to radiation resulted in significant complications. The five-year cause-specific, disease-free, and overall survival rates were 55%, 47%, and 42%, respectively. Gender, complementation group, site of primary tumor, radiation therapy for HNSCC and HPV status had no significant effect on overall survival.

**Conclusion:** A history of HSCT, especially with a conditioning regimen including irradiation, is associated with an earlier onset of aggressive HNSCC in FA patients. This data supports rigorous screening of FA patients for HNSCC after HSCT. FA patients are able to tolerate complex surgical resections for HNSCC treatment and radiation therapy may be appropriate to treat high-risk disease with close monitoring. Further studies are needed to establish HNSCC prevention, screening and treatment guidelines for FA patients.

#### TP53 & IKZF2/3 MUTATIONS IN HYPODIPLOID ACUTE LYMPHOBLASTIC LEUKEMIA

Samir N Patel (Class of 2016)<sup>1,2</sup>, Linda Holmfeldt<sup>1</sup>, Debbie Payne-Turner<sup>1</sup>, and Charles G. Mullighan<sup>1</sup>

<sup>1</sup>Department of Pathology, St Jude Children's Research Hospital, Memphis, TN, US <sup>2</sup>Weill Cornell Medical College of Cornell University, New York, NY, US

**Background:** Every year, nearly 4000 new cases of pediatric acute lymphoblastic leukemia (ALL) are diagnosed in the United States. B lineage ALL (B-ALL) accounts for 85 to 90% of pediatric ALL, and within this cohort, hypodiploid ALL is a subtype that is characterized by multiple whole chromosomal losses and poor outcomes relative to most other ALL subtypes. Besides the whole chromosomal losses associated with hypodiploid ALL, very little genetic information is known about this malignancy despite its high risk relative to other leukemias.

**Methods:** Samples of cryopreserved lymphoblasts, from 41 children with ALL treated at St. Jude Children's Research Hospital and the Children's Oncology Group, were obtained with informed consent at the time of diagnosis. Whole genome sequencing was performed for tumor and normal DNA on 20 children using Illumina's Genome Analyzer.

A combination of the CONSERTING and CREST algorithms was used to detect structural variations (SVs) from the next generation sequencing data, and all putative SVs were validated at the DNA, RNA, and protein level.

**Results:** During the analysis of the 41 next generational sequencing hypodiploid ALL cases, 35 (85%) of the low hypodiploid ALL cases contained *TP53* mutations. These alterations were a combination of frameshifts, missense, nonsense, deletions and insertions. 42% of all mutations were located in the germline. Of the 33 mutations, 27 (82%) were located within the DNA binding domain of *TP53*. 5/6 (83%) of adult patients with hypodiploid ALL harbored *TP53* mutations. 13 cases contained non-somatic mutations (frameshift, nonsense and missense mutations). Next generational sequencing data revealed that within the low hypodiploid cohort, *IKZF2* was altered in 52.9% of cases, whereas *IKZF3* alterations were present in 13.2% of the near haploid cases. Alterations of *IKZF1*, the founding member of the IKAROS gene family, were rare in this cohort despite *IKZF1* alterations being a hallmark of high-risk ALL. Additional work looked at the functional consequences of lkzf2 and lkzf3 on Helios and Aiolos expression, respectively. Ikzf2 with shRNA at mRNA position 449 reduced Aiolos expression by over 88%. Ikzf3 with shRNA at mRNA position 1586 reduced Aiolos expression by over 75%.

**Conclusion:** Prior to these findings, the genomic basis of hypodiploid ALL was unknown. The alterations within the *IKAROS* gene family and *TP53* suggest that the subtypes of hypodiploid ALL, designated by modal chromosomal number, have additional genetically distinct lesions. These findings will be crucial in implementing targeted drug therapies to these hypodiploid ALL subtypes by targeting the specific lesion signaling pathways.

#### CHILDREN WITH SOCIAL-EMOTIONAL AND BEHAVIORAL HEALTH (SEBH) CONCERNS: CAREGIVER PERSPECTIVES ON BARRIERS TO CHILD'S CARE

Tanya Saraiya, BS, Kim Schoenfeld, BA, Eleanor Bathory, MD, Eliza Wierzbinska, MA, BA and Cori Green, MD, MS

<sup>1</sup>Pediatrics/ Family and Social Medicine, Montefiore Medical Center/ Albert Einstein College of Medicine, Bronx, New York, United States and <sup>2</sup>General Academic Pediatrics, Weill Cornell Medical College/New York Presbyterian, New York, New York, United States

**Background:** As pediatric mental health (MH) diagnoses become more prevalent, effective screening and treatment for early childhood SEBH problems can positively impact SEBH and longer term MH outcomes for children. However, barriers to providing SEBH care are frequently cited and both providers and patients report gaps in SEBH care. In order to improve pediatric SEBH it is necessary to understand caregiver perceptions of potential barriers to managing their child's SEBH care.

**Objective:** To assess whether there are associations between parental reported SEBH concerns, parental empowerment in managing their child's behavior, perceived access to care, and MH stigma.

**Design/Methods:** Cross-sectional study using surveys administered over 4 months to a convenience sample of caregivers of children (6 mo- 5 yo) assessed demographics and SEBH variables. Caregiver identified SEBH concern (+SEBH vs. –SEBH) was the predictor variable. Outcome Variables Included: (1) empowerment in managing their child's behavior (7-item likert scale; high empowerment < 10), (2) ease of access to care (report experience of delay/difficulty getting services; adapted from national survey), and (3) MH stigma (validated 2-question likert scale; high stigma > 6). Chi-squared and 2-sample t-test analyses were performed.

**Results:** 249 caregivers were surveyed: 88% mothers, 46% Latino, 33% < HS graduate. 21% caregivers reported SEBH concern. Caregivers of children with SEBH concerns reported feeling less empowered to manage their child's behavior (13.4 vs. 11.3; p=0.006) and more difficulty/delay getting needed services for their child (32.0% vs. 18.3% p=0.04) than caregivers of children without SEBH concerns. Perceived stigma associated with MH was relatively low across the sample (mean score=3.6/10) and was similar (no statistically significant difference) for caregivers of children with and without SEBH concerns.

**Conclusions:** Caregivers of children with SEBH had a lower sense of empowerment and reported increased difficulty gaining access to care. MH stigma scores were low suggesting this is not a barrier for SEBH care. These conclusions strengthen the argument that primary care providers should be recognizing SEBH problems and helping parents manage these problems. Screening programs and co-located models of care may be interventions that can help improve pediatric SEBH care.

#### BRIDGING THE CHILD OBESITY CULTURE GAP IN THE CHINESE-AMERICAN POPULATION WITH A NOVEL MEAL-PLANNING VISUAL AID

James X. Wang<sup>1</sup> (Class of 2016), Betty Lung<sup>2</sup>, Debra Katz-Feigenbaum, MPH, RD, CDN<sup>1</sup>, Maura D. Frank, MD<sup>1</sup>

<sup>1</sup>NewYork-Presbyterian/Weill Cornell Medical Center and <sup>2</sup>Hunter College, The City University of New York

**Background:** Despite growing evidence that Asian-Americans develop obesity co-morbidities at BMIs as low as 24, few studies explore child obesity in this population. Many New York City (NYC) pediatricians use *MyPlatePlanner* (MyPP), a meal-planning visual aid (MVA), to facilitate discussions of healthy diet and to manage child obesity. MyPP may not be effective for Chinese-American families due to cultural differences in dining style.

**Objective:** To survey parents and providers on using *MyTablePlanner* (MyTP), a culturally sensitive MVA for Chinese-American families, and on child obesity challenges in this population.

**Design/Methods:** Parent respondents (N = 35) recruited from 3 pediatric offices in NYC Chinatowns were self-reported as Chinese with a child aged 4-11. Surveys, MyPP, and MyTP were given in the respondent's preferred language. Provider survey respondents (N = 36, 94% pediatricians, 6% NP/RD) were from 5 outpatient pediatric clinics (2 hospital-based, 3 community) in NYC Chinatown and non-Chinatown areas. Data were analyzed using Fisher's exact test.

**Results:** 73% of parents would use MyTP, with 45% of whom preferring it over MyPP. Parents who learn about child obesity solely by word-of-mouth and media perceive MyTP as easier to use 50% more often than those also informed by physicians (p < 0.05). For providers, 86% would stock MVAs for different cultures if available, most of whom predicting MyTP will improve compliance (55%) and effectiveness (62%). Low compliance and loss to follow-up are population-specific challenges for 95% (p < 0.001) and 47% (p < 0.05) of Asian community providers (ACP), compared to 18% and 12% of non-ACPs. All providers rate child obesity as a significant issue, yet 82% of non-ACPs rate it as relatively less so for East Asian patients compared to 11% of ACPs (p < 0.001). "Grandmother" represents 29% of primary meal preparers (PMP). Households preferring Chinese over American foods have 50% less physician-PMP contact relative to those with equal preference (p < 0.05).

**Conclusions:** MyTP is a preferable MVA for many providers and Chinese-American families, especially those medically underserved or culturally preserved. More efforts are needed to increase compliance and follow-up in Asian-American patients as well as provider awareness of co-morbidity risks in this population. The creation of other culturally sensitive MVAs is encouraged.

#### PARENTAL PERCEPTIONS OF SOCIAL EMOTIONAL BEHAVIORAL HEALTH CARE

Eliza Wierzbinska, BA, MA, Tanya Saraiya, BS, Kim Schoenfeld, BA, Eleanor Bathory, MD, and Cori Green, MD, MS

<sup>1</sup>Pediatrics/Family & Social Medicine, Montefiore Medical Center, Bronx, New York, United States and <sup>2</sup>General Academic Pediatrics, Weill Cornell Medical Center/New York Presbyterian Hospital, New York, NY, United States

**Background:** Pediatricians need to provide guidance on early social-emotional and behavioral health (SEBH) with early recognition of concerns.

**Objective:** Assess parental experiences and perspectives' on their child's SEBH care in a practice with colocated psychologists

**Design/Methods:** Cross-sectional study using surveys that were administered to a convenience sample of caregivers of children (6 mo- 5 yo) in an urban academic institute. The survey assessed demographics, travel time, parental opinions on their child's behavior, satisfaction with their child's SEBH care, and preferences for location of SEBH care. Satisfaction with on-site MH services was assessed using 7 questions adapted from a toolkit measuring integrated care. Associations between travel time and location of SEBH care was assessed using multivariate analyses.

**Results:** 249 caregivers were surveyed: 88% mothers, 46% Latino, 33% < HS graduate. 41% of caregivers reported that their pediatrician inquired about their child's SEBH, 6.3 % still had questions, and 83% thought their pediatrician spent right amount of time discussing SEBH. 52% of parents travelled 30- 60 minutes to clinic, 34% less than 30 minutes, and 14% longer than an hour. Of the 21% caregivers that reported a SEBH, 14.2% were referred to a psychologist, and 12.6% saw a psychologist. 79% believed the visit was helpful, and 65% said their child's behavior improved since seeing the psychologist between 1-5 visits (a majority with only 1 visit). [Table 1] 66% of parents preferred to receive SEBH care in the pediatric office which was not associated with travel time.

**Conclusions:** Pediatricians did not routinely inquire about SEBH, yet parents were satisfied with the care provided suggesting the need for more education on the importance of early SEBH. Parents were satisfied with this model of care.

#### Table 1.

% Parents Reported Agree or Strongly Agree	
Convenient location	74%
Easy access to SEBH services	87%
Did not wait long for appointment	68%
Psychologists provided patient-centered care	80%
I was comfortable going to clinic for my child's SEBH	87%
I now know how to manage my child's SEBH	73%

# PEDIATRIC INTEREST GROUPS and FIELD PROGRAMS

# CAMP PHOENIX



Every year, almost one million American children are burned. Fortunately, advancements in trauma and resuscitative care have improved the treatment and survival of these young patients. Despite these medical and surgical advances, the psychosocial care of pediatric burn victims continues long after discharge. These children often return home with scars as permanent reminders of their trauma and the aftermath of surviving a serious burn usually includes considerable stress, diminished self-esteem, and difficulty creating positive social relationships. Camp Phoenix, the first burn camp in the United States run by medical students, was founded in 2000 by Paul Mullan, a 2004 Graduate of Weill Cornell Medical College. Since then, Camp Phoenix has provided a safe environment for pediatric burn survivors and their siblings to interact with their peers and share their experiences.

Camp Phoenix sponsors three one-day events and one overnight camping trip each year. Past events have been held at the Intrepid Museum, the Bronx Zoo, Lucky Strikes Bowling, Sony Wonder Technology Lab, Chelsea Piers, New York Knicks games, the Museum of Natural History and NYC Firehouses, amongst many others. We have worked with over 250 children at these events, with an average of 30 campers and 25 volunteer counselors at each event. Camp Phoenix activities are designed to build confidence, emphasize teamwork, initiate friendship, and maximize fun.

Last June, a group of almost 30 campers and volunteer counselors spent an incredible three days at Camp Kinder in Hopewell Junction, NY. The overnight camping trip is always especially memorable. Campers participate in activities such as canoeing, tie-dye, and hiking. For many of our campers, this is their first time away from home and outside of an urban setting. Campers are divided into cabins, where they work together and quickly develop their sense of community and camaraderie. They create cabin names and cheers and group enthusiasm is rewarded as the cabins participate in one of Camp Phoenix's favorite traditions, the Messy Olympics. Campers compete for cabin pride in games such as the Human Ice Cream Sundae.

In addition to helping the campers and their families, Camp Phoenix offers a unique educational experience for the medical students involved. Our volunteers serve as mentors for children with a range of medical and psychosocial issues, allowing them to hone their skills as leaders, role models, and caretakers. Positive experiences at the day events and overnight camp weekend have inspired many volunteers to develop interest in Pediatrics and Burn Surgery.

Camp Phoenix aims to give future physicians opportunities outside of the classroom to better appreciate the art of compassionate and empathetic care for complex patients. Started in 2011, our shadowing program allows medical students to spend time with the pediatric team in the burn unit. We expect to enhance this shadowing program further with the 2013 implementation of burn surgery shadowing. These experiences will help students learn about the inpatient and surgical experiences of our campers and relevant psychosocial issues. These experiences are meant to educate all interested students about what our campers went through during the rehabilitation phase of their burn care and to have any questions and concerns fully addressed by experts.

#### Faculty Advisor:

T. Jirasevijinda, MD thj2002@med.cornell.edu

#### Student Leadership:

Jaime Bernstein (jlb2016@med.cornell.edu) Sarah Littlehale (sal2031@med.cornell.edu)

### CHEMISTRY FOR KIDS

CHRISTOPHER ROBINSON AND MARIA SACTA

Chemistry for Kids exists to give underprivileged young students the opportunity to experience science in fun and exciting ways. We hope that through our interactive experiments, we can provide young students an avenue to explore their interests in science, and inspire them to become future scientists and doctors. Overall, our main priority is to show students that science can be fun!

#### Student Leadership:

Christopher Robinson (clr2006@med.cornell.edu) Maria Sacta (mas2095@med.cornell.edu)

### CORNELL KIDS

KHALIL ANCHOUCHE AND ANDREW RIVERA

Cornell Kids is an interactive science learning and mentoring project in which sixth and seventh grade students from the East Harlem School in Manhattan are taught about the basic functioning of the body. Teaching sessions are held throughout the Spring semester and are lead by medical students of the Student National Medical Association. Students are taught about the circulatory, respiratory, gastrointestinal, and nervous systems. Students also participate in an Anatomy lab where they can apply what they learned in class and hear about the needs and value of medical research. After each session, students review the content and integrate their new knowledge with common diseases and conditions in their communities.

Medical students can get involved with this program by volunteering to lead a teaching session in the Spring.

#### Student Leadership:

Khalil Anchouche (kha2015@med.cornell.edu) Andrew Rivera (anr2052@med.cornell.edu)

### THE HEADS UP! PEDIATRIC LITERACY PROGRAM

A PROJECT OF THE WEILL CORNELL MEDICAL COLLEGE DEPARTMENT OF PEDIATRICS, DIVISION OF CHILD DEVELOPMENT

Economic disadvantage and limited parental education mean that children born into poverty are susceptible to delays in language development. These children routinely lag behind their peers before pre-school or kindergarten even begin.<sup>1</sup> In most cases, this gap continues to widen in elementary and middle school as children with poorer educational foundations fall further below school standards. Weakness in language and reading skills can lead to poorer educational and health outcomes, such as school failure, low self-esteem, troubled behavior, and substance abuse.<sup>2</sup> In contrast, recent studies have shown that reading aloud to children from early on in life has positive effects on children's language and pre-literacy skills.<sup>3</sup>

In an effort to improve early literacy, the Heads Up! Pediatric Literacy program has initiated a mild intervention mediated by pediatric primary care physicians. Doctors are the professional constituent with the most access to children and parents before school begins. By having physicians alert parents to the need to read to their young children—and by giving an age-appropriate book as part of the physical exam—we make the promotion of early language and literacy development a standard part of primary pediatric care.

Beyond encouraging language development and school readiness, books can also be used for assessment in the exam room. Books can help physicains see whether a four month-old reaches for objects or if a child who moves to accept a book has a normal gait. At some sites, including WCMC, trained volunteers help children select more books and conduct parent outreach in the waiting room.

Heads Up! targets pediatric clinics that serve needy populations. At all of our 12 clinic sites—pediatric outpatient clinics affiliated with WCMC, Lincoln Hospital, St. Barnabas Hospital, Methodist Hospital, and New York Hospital Queens—at least 85% of patients qualify for Medicaid. In 2010-2011, Heads Up! distributed 40,391 brand-new books and corresponding literacy guidance to nearly 20,000 children.

Sadly, this program has been placed in jeopardy due to loss of funding. Reading is Fundamental, Inc., which previously provided over 75% of our book funding, was recently cut from the federal budget. Without our main financial support, we are in danger of having to cut back on book distributions. Because we believe deeply in the mission of promoting child literacy, we are working hard to keep this program going as strong as ever.

#### **Program Contact Information:**

Mary J. Ward, PhD 646-962-6327 mjward@med.cornell.edu

## HEALTH FOR LIFE

REBECCA DEMARIA AND DANIELLE LALONE

Health for Life is a program run by the NYPH Department of Pediatrics that works with overweight children. A team of pediatricians, physical therapists, social workers, nutritionists, and medical student volunteers help children and teens ages ~9 - 18 learn about how to lead a healthier life. The 10-week program has 2 major components: exercise and nutrition. During the exercise sessions, participants discover fun new ways to incorporate physical activity into their lives. As part of this, all participants receive pedometers that they carry around for the duration of the program. The nutrition sessions focus on learning about which foods are healthy and easy ways to make everyday food healthier. The exercise portion includes lessons in yoga, boxing, and other fun exercises to motivate the children.

Medical students get to form relationships with the children as well as the parents. In addition, students get to be role models and have a great time!

Faculty Advisor: Maura Frank, MD mdfrank@med.cornell.edu

#### Student Leadership:

Rebecca DeMaria (rdd2001@med.cornell.edu) Danielle LaLone (dkl2001@med.cornell.edu)

# HEALTH PROFESSIONS RECRUITMENT & EXPOSURE PROGRAM (HPREP)

DANIELA GUISADO, GBAMBELE KONE, CHARLES MCTAVISH, ELIZABETH O'CALLAHAN, AND ANDREW RIVERA

The Health Professions Recruitment & Exposure Program (HPREP) is part of the Pipeline Mentoring Institute of the Student National Medical Association (SNMA). HPREP aims to expose high school students from underrepresented minorities to science, medicine and the health professions. The over-arching goal is to encourage minority students to pursue a career in medicine. During the three month after school program, the students attend a variety of lectures, participate in an anatomy lab dissection, receive assistance on their college application and essay, write a research paper on a topic of their choice and build a lasting relationship with a medical student mentor. We typically accept 80 high school students every year and engage 40 medical students from across all classes to be mentors and role models for the high-schoolers. This program began here at Cornell and has subsequently spread to many other medical colleges around the country.

#### Student Leadership:

Daniela Guisado (dig2016@med.cornell.edu) Gbambele Kone (gjk2003@med.cornell.edu) Charles McTavish (ctm2004@med.cornell.edu) Elizabeth O'Callahan (emo2004@med.cornell.edu) Andrew Rivera (anr2052@med.cornell.edu)

#### Faculty Advisors:

Dr. Carol Capello (cfc2002@med.cornell.edu) Dr. Jane Chang (jac9009@med.cornell.edu)

## KIDS IN CHRONIC CARE SUPPORT (KICS)



DANIELA GUISADO AND MAYA MADHAVAN

KICS is a student-run program with the New York Presbyterian department of Pediatric Hematology/Oncology that creates one on one matches between Weill Cornell medical students and children or adolescents currently receiving therapy. The focus of the program is to provide support for the children and their families; it gives the kids an opportunity to form a close, consistent relationship with someone outside of their treatment team. KICS leadership personally matches students with patients interested in having a buddy. Once a match is made, the student makes the initial contact with the patient during a clinic visit. After this, buddies can spend time together whenever it is best for both; this can be during hospital visits or even outside of the hospital.

For kids, the hospital can be an intimidating place associated with pain, discomfort, and, of course, the terrible effects of chemotherapy. Medical students can help make their treatment experience a little better. Especially in pediatrics, the diagnosis of cancer can have a major impact not only on the patient but also on the patient's family. For parents, KICS can take some pressure off of the situation and give them a needed break. KICS provides medical students with the opportunity to follow a patient case longitudinally and also to delve into the impact of chronic illness on patients and their families.

Past members of the program have had positive experiences with KICS:

"At first I thought, he's on chemo, I'm going to feel bad for him. But although his illness was always in the background that wasn't all there was to him, and you can lose sight of that when you're a doctor. You can forget the humanistic side, putting a person in the context of their life."

*"It's nice for the kids to have someone who's relatively young; not their parent or a sibling, just somebody who wants to hang out with them. It distracts them from their treatment. We're medical students, but we're not there for any medical purpose... We just want to talk to them and have a little fun."* 

"In the first two years you spend so much time learning basic sciences, it can be a real drag. Being able to take yourself out of that, to put a face to what you're doing, really motivates you."

#### Student Leadership:

Daniela Guisado (dig2016@med.cornell.edu) Maya Madhavan (mam2166@med.cornell.edu)

#### Faculty Advisor:

Dr. Alexander Aledo (aaledo@med.cornell.edu) Dr. Zoltan Antal (zoa9003@med.cornell.edu)

## Komansky Center Initiatives Family Advisory Council



The Komansky Center Family Advisory Council (FAC) is a group of dedicated parents and family members of pediatric patients who are committed to working with Komansky Center hospital staff and administration to provide family-centered care to all patients. Our vision is to achieve a level of care where patient and family involvement is expected and welcomed by all. Among the Council's many current initiatives are:

#### Family Education and Orientation Workgroup

The goal of the Workgroup is to improve care while at the hospital by helping patients' families and Hospital staff communicate more effectively. The *Family Education and Orientation Workgroup* tries to identify ways to 1) Orient family members to the Hospital with written and verbal communication tools; 2) Enhance communication skills of new and current Hospital staff members; 3) Improve communication between Hospital staff and families and, d) Revise preoperative procedures for outpatient surgeries.

#### Family Experience Workgroup

A child's stay in the hospital is often very stressful for his or her family. *The Family Experience Workgroup* is committed to creating a pleasant environment for patients and their families. Workgroup members identify different ways to improve and expand the infrastructure and recreational services currently offered at the Hospital. Recent activities have included distribution of gifts during the holidays, participation in the Thanksgiving festivities hosted by Child Life, and engaging local school children to create holiday cards for hospitalized children.

#### Family Support Workgroup

By sharing experiences, families can help each other through a tumultuous and traumatic time. The *Family Support Workgroup* is committed to identifying ways to provide support to families and to managing that support systematically. *Family Support* is focused on three areas: 1) The development of a resource center for families; 2) the creation of a parent-to-parent directory; and 3) the development of a mentoring program so that current families can seek advice from families who have "graduated" from the hospital.

#### Family Faculty Program

The *Family Faculty Program* works with hospital staff and administration to incorporate FAC parents in orienting and educating new residents on the topic of family-centered care. FAC parents help residents learn by sharing their own stories within the healthcare system. The *Family Faculty Program* hopes to expand their activities to student education.

#### **Program Coordinator**

Mariela Guerra (mag9152@nyp.org)

### MOTIVATING ACTION THROUGH COMMUNITY HEALTH OUTREACH (MACHO)

ELIZABETH MACINTYRE AND BRITTNEY SHULMAN



Overview: Motivating Action through Community Health Outreach (MAChO) is a Weill Cornell Medical College student-led, community-centered response to the alarmingly increasing rate of childhood obesity, particularly within minority and socioeconomically disadvantaged communities. The program has a two-pronged approach to improving health within disadvantaged communities: the Youth Wellness and Youth Leadership programs. These programs are aimed at motivating young children to lead healthier lives and engaging high school students on health-oriented community projects and leadership training.

Mission: The goal of MAChO is to empower youth with the knowledge and practical tools to take control of their health and find solutions health challenges facing their communities. Our focus is on proper nutrition, fitness, personal development and leadership. We aim to accomplish this goal by:

*Motivating Action* by *building a community of empowered youth* through dissemination of information that inspires the adoption of healthy living habits,

*Motivating Action* by providing youth with a platform and the mentorship necessary to become agents of healthy change in their community

Motivating Action by pursuing a holistic, adaptive, and individualized approach towards addressing poor nutrition and sedentary lifestyles, and

Motivating Action by partnering with community organizations to build a supportive network of empowered individuals and families.

History: MAChO was established in the fall of 2009 by a handful of Weill Cornell Medical College students who recognized the desperate need for education and resources to fight the obesity epidemic. By pairing with Settlement Health, a nonprofit community health center in East Harlem, MAChO initiated the Youth Wellness Program, a pilot-phase program to teach kids how to make healthy nutrition and fitness choices within their community. The pilot program met once a week for ten weeks. In 2010-2011, the program was expanded to a full-year curriculum that met once a week during the school year and every day in the summer. The scope of MAChO was broadened to include a pilot mentoring program, educational field trips, and assessments to track knowledge and fitness progress. The lessons from the first three years have served as a foundation for the revamped Youth Wellness Program and new initiatives for the 2012-2013 year. In 2012, the MAChO Youth Leadership Program launched with the goal of engaging local high school students who will grow to be leaders in their community and within MAChO.

Program: The structure of the curriculum consists of one weekly after-school session coupled with one weekend session on mentoring and personal development. For the after-school program, our volunteer graduate and undergraduate teachers lead the nutrition, physiology and exercise classes under the leadership of a site coordinator. The mentoring session includes our personal development curriculum, where we use a group-mentoring model: mentor teams are composed of college students who serve as mentors to our middle school and elementary school participants. The mentor teams work on group projects to be presented at the end of each semester based on a specific theme surrounding health.

Day 1			Day 3		
Nutrition	Exercise	Snack	Exercise	Group Project	P.D. Curriculum

For the 2013-2014 year, we run our Youth Wellness Program sessions at St Marks the Evangelist School in Harlem and the Silberman School of Social Work at Hunter College. The Youth Leadership Program meets bimonthly at the Silberman School of Social Work. This fall we enrolled a total of 60 participants, ages 7-13 and 14-18.

Members: MAChO volunteers include students from WCMC, Cornell University, Columbia University, Hunter College, City College and Queens College. We are overseen by faculty advisors from New York-Presbyterian Hospital Pediatrics department, WCMC, Hunter College, and Hunter School of Public Health and Social Work. Our community health outreach partners include the Boys' Club of New York, Settlement Health, Harlem Center for Healthy Living, and Choosing Healthy and Active Lifestyle for Kids (CHALK)

#### Contact:

445 East 69<sup>th</sup> St. #208 New York, NY 10021 347-746-2461 machoprogram@gmail.com

#### **Program Faculty Mentors:**

Dr. Curtis Cole, MD Chief Information Officer at Weill Cornell Associate Attending Physician New York Presbyterian Hospital - Cornell ccole@med.cornell.edu

Dr. Melanie Wilson-Taylor, MD Assistant Attending Pediatrician and Professor of Pediatrics New York Presbyterian Hospital - Cornell mtw2001@med.cornell.edu

Dr. May May Leung, PhD Assistant Professor at CUNY School of Public Health at Hunter College mm.leung@hunter.cuny.edu

#### Student Leadership:

Elizabeth MacIntyre (etm2002@med.cornell.edu) Brittney Shulman (brs7006@med.cornell.edu

## SCIENCE AND MEDICINE ENHANCEMENT PROGRAM (SMEP)

ROLAKE ALABI

The Science and Medicine Enhancement Program (SMEP) provides middle school students with hands-on opportunities to learn about health and disease through a multi-subject approach. SMEP students are from the Science and Medicine Middle School, a school serving students from the Carnasie and East Flatbush communities in Brooklyn, NY. In a series of sessions held at the Weill Cornell Medical College in Manhattan, NY, groups of 3-4 students led by medical students work on hands-on group and individual activities designed to reinforce basic concepts in basic biology, physiology, pathophysiology, and healthy living related to the diseases and health conditions that most affect the students; communities. During the program, students also engage with material through reading, research, persuasive writing, presentations and clinical skill sessions. The program culminates with students presenting and leading demonstration tables related to various curricular topics at a Community Health Fair held at the Science and Medicine Middle School.

#### Student Leadership:

Rolake Alabi (roa2019@med.cornell.edu)

# WEILL CORNELL YOUTH SCHOLARS PROGRAM (WCYSP)

ANDREW HILLMAN

The main purpose of the Weill Cornell Youth Scholars Program (WCYSP) is to expose underprivileged, underrepresented students, especially from inner city high schools, to the vast educational resources and opportunities at Weill Cornell Medical College and NewYork Presbyterian Hospital. Many of these high schools have exceptionally high drop out rates that coincide with low percentages of graduates that go on to attend a four-year university. By developing early experiences in medicine, students can develop appropriate attitudes towards their education, interpersonal skills, and more importantly, confidence in themselves to succeed academically. The WCYSP curriculum is designed to educate, inspire, and groom participants for personal and professional success. We seek to address some of the weaknesses that prevent many inner-city students from performing well at the college level through an innovative format that emphasizes critical reading and writing. Students attend lectures, given by WCMC students, in physiology, anatomy, and basic science. Our daily Problem Based Learning (PBL) sessions provide a forum for youth scholars to interact with one another and build their teamwork abilities. All high school students that completed the program reported that it had a significant impact on their personal motivation to pursue a career in science or medicine and are more motivated to take more challenging courses in high school. Moreover, all of the students who graduated from the program matriculated in college and most who are now in college plan on majoring in science and/or pursing a pre-med track. Weill Cornell medical students, residents and attending physicians serve as mentors and teachers in the program. Volunteer teachers can choose one or more topics and will be given lecture notes and powerpoint slides that are already prepared for each topic. Alternatively, volunteer teachers may use their own teaching materials for their particular topic. The program runs for one month every July from Tuesday to Friday. Typically. each lecturer will give one or two one-hour lectures, but can choose to volunteer more of their time. We also recruit new leadership every year to plan the next summer's program under the guidance of leaders from the previous year.

#### Student Leadership:

Andrew Hillman (anh2024@med.cornell.edu)

## MENTORING AND RESEARCH OPPORTUNITIES IN PEDIATRICS

## **Faculty Mentors and Advisors**

#### Erika Abramson, MD, MS

General Academic Pediatrics Department of Pediatrics, Weill Cornell Medical College 212-746-3929 err9009@med.cornell.edu

#### Oleh Akchurin, MD

Assistant Professor of Pediatrics Division of Nephrology Weill Cornell Medical College 646-962-4324 oma9005@med.cornell.edu

#### Alexander Aledo, MD

Pediatric Hematology/Oncology Department of Pediatrics, Weill Cornell Medical College 212-746-3400 aaledo@med.cornell.edu

#### Zoltan Antal, MD

Pediatric Endocrinology Department of Pediatrics, Weill Cornell Medical College zoa9003@med.cornell.edu

#### Adele Boskey, Ph.D.

Musculoskeletal Integrity Program Hospital for Special Surgery 535 E 71<sup>st</sup> St. Room 628 212-606-1453 boskey@hss.edu

#### Susan Bostwick, MD

General Academic Pediatrics Department of Pediatrics, Weill Cornell Medical College 212-746-3522 sbbostwi@med.cornell.edu

#### Farid Boulad, MD

Department of Pediatrics Memorial Sloan-Kettering Cancer Center 212-639-6684 bouladf@mskcc.org

#### James Bussel, MD

Pediatric Hematology/Oncology Department of Pediatrics, Weill Cornell Medical College 212-746-3474 jbussel@med.cornell.edu

#### BJ Casey, Ph.D.

Sackler Institute Department of Psychiatry, Weill Cornell Medical College Suite F-1332 bjc2002@med.cornell.edu

#### Sheila Carroll, MD

Pediatric Cardiology Department of Pediatrics, Weill Cornell Medical College sjc7002@med.cornell.edu

#### Marisa Censani, MD

Pediatric Endocrinology Department of Pediatrics, Weill Cornell Medical College 212-746-3462 mac9232@med.cornell.edu

#### Margaret Crow, MD

Department of Rheumatology Hospital for Special Surgery 535 E. 70<sup>th</sup> St.. Room R200 212-606-1397 crowm@hss.edu

#### **Christopher Cunniff, MD**

Department of Pediatrics Division of Medical Genetics Weill Cornell Medical College 646-962-2205 cmc9039@med.cornell.edu

#### Susanna Cunningham-Rundles, Ph.D.

Pediatric Hematology/Oncology/GI Department of Pediatrics, Weill Cornell Medical College 212-746-3414 scrundle@med.cornell.edu

#### Jessica Davis, MD

Pediatric Genetics Department of Pediatrics, Weill Cornell Medical College 212-746-1496 jgdavis@med.cornell.edu

#### Jeffrey Dayton, MD

Pediatric Cardiology Department of Pediatrics, Weill Cornell Medical College 212-746-3561 jed9031@med.cornell.edu

Diane Felson, Ph.D.

Pediatric Urology Department of Pediatrics, Weill Cornell Medical College 212-746-5796 dfelson@med.cornell.edu

#### Patrick Flynn, MD

Pediatric Cardiology Department of Pediatrics, Weill Cornell Medical College 212-746-3561 paflynn@med.cornell.edu

#### Maura D. Frank, MD

Department of Pediatrics, Weill Cornell Medical College 212-746-3353 mdfrank@med.cornell.edu

#### Sara Gardenghi, PhD

Pediatric Hematology/Oncology Department of Pediatrics, Weill Cornell Medical College sag2010@med.cornell.edu

#### Patricia J. Giardina, MD

Pediatric Hematology/Oncology Department of Pediatrics, Weill Cornell Medical College 212-746-3415 pjgiardi@med.cornell.edu

#### Allison Gorman, MD

General Academic Pediatrics Department of Pediatrics, Weill Cornell Medical College agg9003@med.cornell.edu

#### Daniel W. Green, MS, MD Hospital for Special Surgery 212-606-1631

greendw@hss.edu

**Cori Green, MD** General Academic Pediatrics Department of Pediatrics, Weill Cornell Medical College 212-746-0921 cmg9004@med.cornell.edu

#### Bruce Greenwald, MD

Pediatric Critical Care Medicine Department of Pediatrics, Weill Cornell Medical College 212-746-305 bmgreen@med.cornell.edu

#### Alan Groves, MD

Neonatology Department of Pediatrics, Weill Cornell Medical College 212-746-3530 alg2049@med.cornell.edu

#### Duncan Hau, MD

General Academic Pediatrics Department of Pediatrics Weill Cornell Medical College 212-312-5000 dkh2001@med.cornell.edu

#### Joy D. Howell, MD

Pediatric Critical Care Medicine Department of Pediatrics Weill Cornell Medical College 212-746-3272 jdh2002@med.cornell.edu

### Lisa Cooper Hudgins, M.D.

The Rogosin Institute Weill Cornell Medical College 212-327-7744 hudgins@mail.rockefeller.edu

#### Lisa Ipp, MD

General Academic Pediatrics Department of Pediatrics, Weill Cornell Medical College 212-746-3372 Isi9001@med.cornell.edu

#### Thanakorn Jirasevijinda, MD

General Academic Pediatrics Department of Pediatrics, Weill Cornell Medical College 212-746-3131 thj2002@med.cornell.edu

#### Barry Kosofsky, MD

Department of Pediatrics Department of Neurology Neurobiology Laboratory 212-746-3321 bar2009@med.cornell.edu

#### Nicole Kucine, MD

Pediatric Hematology/Oncology Department of Pediatrics, Weill Cornell Medical College 212-746-3873 nik9015@med.cornell.edu

#### Juhi Kumar, MD, MPS

Pediatric Nephrology Department of Pediatrics, Weill Cornell Medical College 646-962-2037 juk2013@med.cornell.edu

#### Thomas J.A. Lehman, M.D.

Pediatric Rheumatology Hospital for Special Surgery 212-606-1158 lehmant@hss.edu

#### David C. Lyden, MD, PhD

Pediatric Hematology/Oncology Children's Cancer & Blood Foundation Labs Department of Pediatrics, Weill Cornell Medical College 413 East 69<sup>th</sup> St, Rm 1228, Belfer Building dcl2001@med.cornell.edu

#### Catharine McGuinn, MD

Pediatric Hematology/Oncology Department of Pediatrics, Weill Cornell Medical College 212-746-3400 cam9061@med.cornell.edu

#### Jordan Metzl, MD Hospital for Special Surgery

212-606-1678 metzlj@hss.edu

#### William Beau Mitchell, MD

Pediatric Hematology/Oncology Department of Pediatrics, Weill Cornell Medical College 212-746-3400

#### Anne Moscona, M.D.

Friedman Research Laboratories Department of Pediatrics, Weill Cornell Medical College 515 East 71st St., 6th Floor 212-746-4523 anm2047@med.cornell.edu

#### **Richard O'Reilly, MD**

Department of Pediatrics Memorial Sloan-Kettering Cancer Center 212-639-5957 oreillyr@mskcc.org

#### Snezana Nena Osorio, MD

General Academic Pediatrics Department of Pediatrics, Weill Cornell Medical College 212-746-3457 snm2001@med.cornell.edu

#### Jeffrey Perlman, MD

Neonatology Department of Pediatrics, Weill Cornell Medical College 212-746-3530 jmp2007@med.cornell.edu

#### Shari Platt, MD

Emergency Medicine Department of Pediatrics, Weill Cornell Medical College 212-746-3431 slp9001@med.cornell.edu

#### Dix Poppas, MD

Pediatric Urology Department of Pediatrics, Weill Cornell 212-746-5337 dpoppas@med.cornell.edu

#### Matteo Porotto, Ph.D.

Friedman Research Labs Department of Pediatrics, Weill Cornell Medical College 515 East 71st St., 6th Floor 212-746-4141 map2028@med.cornell.edu

#### Cathleen L. Raggio. MD

Hospital for Special Surgery 535 E. 70th St. 212-606-1339 raggioc@hss.edu

#### Stefano Rivella, Ph.D.

Pediatric Hematology/Oncology Children's Cancer & Blood Foundation Laboratories Department of Pediatrics, Weill Cornell 515 East 71st St., Room 7th Floor 212-746-4941 str@2010@med.cornell.edu

#### Christine M. Salvatore, MD

Division of Infectious Disease Department of Pediatrics, Weill Cornell Medical College 646-962-6845 chs2032@med.cornell.edu

#### Sujit Sheth, MD

Pediatric Hematology/Oncology Department of Pediatrics, Weill Cornell Medical College 212-746-3400 shethsu@med.cornell.edu

#### Leonard G. Steinberg, M.D.

Pediatric Cardiology Weill Cornell Medical College 212-746-3561 Igs9003@med.cornell.edu

#### Anne Stone, MD

Pediatric Allergy, Immunology, Pulmonology Department of Pediatrics Weill Cornell Medical College 646-962-3410 ans9079@med.cornell.edu

#### Heidi Stuhlmann, PhD

Department of Cell & Developmental Biology Department of Pediatrics Weill Cornell Medical College 212-746-6156 hes2011@med.cornell.edu

#### Robbyn E. Sockolow, MD

Division of Gastroenterology Department of Pediatrics, Weill Cornell Medical College ros2023@med.cornell.edu

#### Aliza Solomon, DO

Division of Gastroenterology Department of Pediatrics, Weill Cornell Medical College als9047@med.cornell.edu

#### Chani Traube, MD

Critical Care Medicine Department of Pediatrics, Weill Cornell Medical College 212-746-3056 chr9008@med.cornell.edu

#### Sima Toussi, MD

Division of Infectious Disease Department of Pediatrics, Weill Cornell Medical College 212-746-7379 sst2002@med.cornell.edu

#### Susan Vannucci, PhD

Neonatology Department of Pediatrics, Weill Cornell Medical College 212-746 1446 suv2003@med.cornell.edu

#### Maria Vogiatzi, MD

Pediatric Endocrinology Department of Pediatrics, Weill Cornell Medical College 212-746-3462, 212-746-3486 mvogiatz@med.cornell.edu

#### Mary Jo Ward, Ph.D.

Child Development Department of Pediatrics, Weill Cornell Medical College 212-746-3582 mjward@med.cornell.edu

#### Roger Widmann, M.D.

Division of Pediatric Orthopaedic Surgery Hospital for Special Surgery 212-606-1325 widmannr@hss.edu

#### Melanie Wilson-Taylor, MD

General Academic Pediatrics Department of Pediatrics, Weill Cornell Medical College (212) 746-3303 mtw2001@med.cornell.edu

#### Stefan Worgall, MD, PhD

Pediatric Allergy, Immunology, Pulmonology Friedman Research Laboratories Department of Pediatrics, Weill Cornell Medical College 212-746-5353 stw2006@med.cornell.edu

## RESEARCH OPPORTUNITIES IN PEDIATRICS

#### Erika Abramson, MD, MS

General Academic Pediatrics Department of Pediatrics, Department of Healthcare Policy and Research Weill Cornell Medical College 212-746-3929 err9009@med.cornell.edu

Field(s) of Interest: Pediatric hospitalist and outpatient medicine, health services research, healthcare safety and quality research

Research Title: Health services research, healthcare safety and quality research

<u>Project Description</u>: I am working on a health services research qualitative study assessing the implementation of various health information exchange interventions designed to improve patient care in the Bronx.

I am also working, with several residents and a medical student, on a project assessing the outcome for infants born to mothers who are GBS positive but treated with clindamycin and gentamycin, a popular but understudied treatment regimen.

<u>Students' Role in the Projects</u>: For the qualitative project, students would have the opportunity to participate in 1:1 interviews with study subjects. They would learn qualitative data analysis techniques and have the opportunity to participate in research team meetings where we discuss study design, data collection, analysis and manuscript writing.

For the newborn project, students would have the opportunity to conduct retrospective chart reviews and participate in data analysis and manuscript writing.

Preferred Experience: None required

Oleh Akchurin, MD Assistant Professor of Pediatrics Division of Nephrology Weill Cornell Medical College 646-962-4324 oma9005@med.cornell.edu

<u>Field(s) of interest</u>: Pediatric nephrology. Might be also considered by students interested in pediatric endocrinology, rheumatology, immunology and general pediatrics.

<u>Research Title</u>: The role of inflammation in growth impairment and modulation of pro-inflammatory activity by growth hormone therapy in children with chronic kidney disease

<u>Project Description</u>: This project has retrospective and prospective arms. Medical students may be particularly interested in the retrospective arm. Study is already IRB-approved.

<u>Students' Role in the Project</u>: Data abstraction, processing and analysis, presentation the results at scientific meetings.

<u>Preferred Background/ Experience:</u> Excel skills will help. If the student can work with SPSS or similar packages (Stata, SAS, R) it would be fantastic but <u>not</u> required. If there are any students with experience in NHANES, I have some potential projects with NHANES also.

#### Adele Boskey, Ph.D. Musculoskeletal Integrity Program Hospital for Special Surgery; 212-606-1453 boskeya@hss.edu

Field(s) of Interest: Mineralization, matrix formation, bone development and repair.

<u>Research Title</u>: Mineral analysis in bones of animals with developmental abnormalities

<u>Project Description</u>: The goals of one of the major project in this laboratory is the determination of how matrix proteins regulate biomineralization. As such we study the effects of these proteins in solution, in culture, and when they are ablated or over expressed in transgenic animals. The project would be based on one of the models currently under investigation, where the student would do the histology, and work on the infra red imaging analysis of the bones of animals of different ages.

<u>Students' Role in the Project</u>: Infrared and microCT analyses of bones and teeth of a specific KO or TG animal. Student will learn about the ablated protein and perform IR Imaging and microCT

Preferred Background/ Experience: Student should have computer skills

James B Bussel, MD Department of Pediatrics Weill Medical College of Cornell 212-746-3474 jbussel@med.cornell.edu

Field(s) of Interest: Hematology/ Oncology

- Antenatal Management of Fetal Alloimmune Thrombocytopenia
- Experimental treatments of Refractory ITP

<u>Project Description</u>: Diagnosis, counseling, and entry into a multi-center randomized clinical trial. We design and coordinate this study, which is intended to prevent intracranial hemorrhage from immune thrombocytopenia in fetuses and neonates by administering treatment to mothers while they are pregnant who have a platelet antigen incompatibility with their husbands.

We have also started work on a prophylaxis project to prevent women at risk from developing Alloimmune thrombocytopenia.

Children and adults with difficult to treat ITP are enrolled on treatment protocols of various agents including thrombopoietic agents, anti-CD20 including augmented versions with high dose steroids and/or anti-T cell agents, IV gammaglobulin, and inhibitors of syk and other novel agents. All of the studies have collaborative laboratory studies) connected with them.

#### Students' Role in the Project:

A) Helping to collect data. This entails contacting other centers to ensure that the various components of the trial are sent to us: consents and IRB paperwork; infusion related data, lab work (maternal data and fetal

sonos), and follow up information on the neonates and infants. It also involves reviewing charts and potentially contacting patients.

B) Helping to analyze the data that has been collected.

C). Design and contribute to special projects related to AIT study eg an investigation of autoimmunity in the mothers who have alloimmuen thrombocytopenia

1. Monitor the individual ITP patients to ensure that their visits and studies occur as per protocol and that the appropriate information is collected.

2. Help to develop new studies connected with individual protocol agents and/or help to develop novel studies of new agents .

3. Ongoing analysis of data to determine progress with protocols.

4. Facilitate laboratory studies by pulling freezer specimens to be batched and sent off

Preferred Background/ Experience: None (clinical not laboratory research)

#### BJ Casey, PhD

Sackler Institute, Department of Psychiatry Weill Medical College, Suite F-1332 bjc2002@med.cornell.edu

Field(s) of Interest: Developmental cognitive neuroscience

Research Title: Research in Developmental Psychobiology

#### Project Description:

Work on developmental brain imaging studies using functional magnetic resonance imaging and fiber tracking with diffusion tensor imaging to examine limbic forebrain regions implicated in addiction and impulsivity.

Work on attention and reading training interventions and how they impact behavior and neural systems testing pre and post-training effects with functional magnetic resonance imaging and diffusion tensor imaging. This work is relevant for the disorders of ADHD and reading disorders.

Work on developmental brain imaging studies using functional magnetic resonance imaging and fiber tracking with diffusion tensor imaging to examine limbic forebrain regions implicated in addiction and impulsivity.

Work on attention and reading training interventions and how they impact behavior and neural systems testing pre and post-training effects with functional magnetic resonance imaging and diffusion tensor imaging. This work is relevant for the disorders of ADHD and reading disorders.

<u>Students' Role in the Project</u>: Students would be provided with background reading, IRB and HIPAA training, image analysis, behavioral testing, programming and scientific discussions. Typically students are exposed to every aspect of the study and depending on contributions in the lab can be a co-author on a paper or conference presentation and as such get writing experience too. The student is jointly mentored by a team of investigators including pre and post-doctoral fellows and a faculty PI.

<u>Preferred Background/Experience:</u> Yes, some general computer experience would be very helpful.

#### Marisa Censani, MD

Pediatric Endocrinology Department of Pediatrics, Weill Cornell Medical College 212-746-346**2** mac9232@med.cornell.edu

<u>Field(s) of Interest</u>: Obesity and insulin resistance, bone and mineral metabolism, growth, thyroid disorders, and diabetes.

Research Title: TItles are pending. Topics include obesity and bone mineral metabolism

Project Description: Pending; please contact Dr. Censani

Preferred Background/ Experience: None

Margaret Crow, MD Hospital for Special Surgery Department of Rheumatology 535 East 70<sup>th</sup> Street, Room R-200 212-606-1397 crowm@hss.edu

Field(s) of Interest: Autoimmune Disease; Immunoregulation

Research Title: Regulation of the Immune Response in Autoimmune Disease

<u>Project Description</u>: The laboratory studies the human immune system in healthy individuals and patients with systemic lupus erythematosus to better understand the triggers and mediators of autoimmunity and inflammation in that disease. Students are welcome to participate in ongoing laboratory projects, or initiate their own projects, that use cell culture, flow cytometry, real-time PCR, cell transfection, protein analysis, and other approaches to study mechanisms of autoimmunity.

Preferred Background/ Experience: None, although laboratory experience helps.

#### Susanna Cunningham-Rundles, PhD

Department of Pediatrics Weill Medical College of Cornell University Cellular Immunology Laboratory scrundle@med.cornell.edu

<u>Field of Interest:</u> Cellular Immunology, host response to pathogens, development of immune response, cytokine regulation

Research Titles:

- 1. Role of beta glucans in immune response and hematopoiesis
- 2. Development of Neonatal Immune Response

#### Project Descriptions:

<u>Role of beta glucans in immune response and hematopoiesis:</u> The overall objective of our studies in collaboration with the MSKCC Research Center for Botanical Immunomodulators (NIH P50) is to identify botanicals with potential bioactivity for enhancement of immune function and reconstitution of hematopoietic and immune function after cancer chemotherapy, and to investigate botanicals that have adjuvant activity for cancer immunotherapy.

<u>Development of neonatal immune response</u>: The increased susceptibility of neonates to infections stems from the immaturity of the immune system at birth. Hematopoiesis and host defense in the neonate are developmentally immature. Studies focus on the role of microbes both commensals and potential pathogens on neonatal immune response in the regulation of proinflammatory response.

<u>Students' Role in the Project</u>: Student will participate in all aspects of the studies including experimental design and hypothesis testing and will learn relevant technology.

Preferred Background/Experience: Knowledge of basic laboratory skills and sterile technique.

Jessica G. Davis, MD Department of Pediatrics Weill Medical College of Cornell 212-746-1496 jgdavis@med.cornell.edu

Field (s) of Interest: Medical Genetics

<u>Research Title:</u> Student can work on one of two projects, these include study mage in adolescent patients with Marfan syndrome and or parental attitudes re: newborn screening.

<u>Project Description:</u> Both studies will involve the use of questionnaires. We are in the process of developing a questionnaire for IRB approval re: Newborn screening. The aim is to determine what information pregnant women and their partners have about newborn screening and the NYS Screening program in order to determine their needs as well as to develop an educational program about this subject in face of the expanded test panel. The educational program will be aimed at patients but will include a professional component. We plan to develop a questionnaire for adolescents with Marfan syndrome to learn more about their views on their asthenia appearance and life activities.

<u>Students' Role in Project:</u> Students can help develop and modify the questionnaires. Student will learn interview techniques.

Preferred background/Experience: None

#### Sara Gardenghi, PhD

Hematology-Oncology Children's Cancer and Blood Foundation Laboratories Department of Pediatrics Weill Cornell Medical College/ Pediatrics, 515 E 71<sup>st</sup> Street, room S704 212-746-4938 sag2010@med.cornell.edu

Field(s) of Interest: Hematology, disorders of iron metabolism, anemia of inflammation, beta-thalassemia.

Research Title: Investigating the role of cytokines and hepcidin in a mouse model of anemia of inflammation

<u>Project Description</u>: Anemia of inflammation (AI) is the second most common form of anemia, affecting patients with chronic illnesses, such as infections, autoimmune diseases, or cancer. The aim of the project is to better characterize the complex pattern of inflammatory cytokines that is responsible for AI, with the purpose of identifying new therapeutic targets for this condition. It has already been shown that interleukin-6 is increased in AI, activating the production of the iron regulatory hormone hepcidin in the liver. Through hepcidin, IL-6 ultimately affects iron metabolism reducing the availability of iron for erythropoiesis, and thus generating the anemia. However, together with IL-6, other cytokines (e.g. IFN-g, TNF-a) have been shown to alter iron metabolism and erythropoies, with mechanisms that still need to be fully elucidated.

<u>Students' Role in the Project</u>: Student(s) will participate in all aspect of the above-described research, learning numerous techniques. These include: tissue samples collection, tissue iron analysis, flow-cytometry, ELISA, RNA extraction and analysis, quantitative PCR. Students will gain experience in many of the following experimental approaches:

- 1. Use of mouse models lacking the expression of hepcidin (Hamp KO) or specific cytokines (IL-6 KO, IFN- g KO, and TNF-a KO), and generation of double knockout (e.g. IL-6/Hamp KO).
- 2. Induction of AI by injection of heat-killed Brucella abortus antigen (HKBA).
- 3. Study of iron metabolism by expression profile analysis of iron-related genes, and comparison of results to tissue and serum iron levels in HKBA-treated and control mice.
- 4. Study of erythropoiesis with the goal of elucidating the mechanisms responsible for anemia in mice.
- 5. Inactivation of specific cytokines by treatment with cytokine inhibitors to selectively analyze the effect of the numerous cytokines involved in AI.

<u>Preferred Background/Experience</u>: Basic knowledge of molecular biology and laboratory techniques. Literature review skills. Interest and motivation are required.

Diane Felsen, PhD and Dix P Poppas, MD Pediatric Urology Department of Pediatrics The Weill Medical College of Cornell 212-746-5796 dfelsen@med.cornell.edu

Field(s) of Interest: Uretal obstruction- renal histopathology and function

Field(s) of Interest: Uretal obstruction- renal histopathology and function

#### Project Descriptions:

Renal Dysfunction models: Hydronephrosis and polycystic kidney disease: In children, the most commonly detected prenatal anomaly is hydronephrosis, the dilation of the renal collecting system. Our laboratory has had a long-standing interest in the molecular mechanisms of damage to the kidney after obstruction, especially the fibrotic response, in which there is a pathologic accumulation of extracellular matrix proteins, which damage the kidney and reduce its function. One of the first events in the obstructed kidney is the build-up of pressure, which results from obstruction of the ureter. We have previously found that pressure activates important signaling pathways in the generation of Nitric Oxide, a cytokine with an important role in renal. Currently, we are investigating how pressure activates the fibrotic process in various cells in the kidney. These studies will use gene array, proteomic and metabolomic approaches to identify appropriate candidates. These studies will be important to determine if there are pathways which might be amenable to therapeutic intervention to halt or reverse renal damage in obstruction. We are also investigating an in vitro model of polycystic kidney disease. Using embryonic kidneys, we are studying different signaling pathways and examining their role in cAMP-mediated cyst formation.

Design of a Synthetic Bladder Augment Patch: Bladder dysfunction related to small, fibrotic bladders is a significant problem in children, resulting in high bladder storage pressures and low bladder volume. The high pressures that build up impact upon bladder function by inducing fibrosis and on quality of life because of incontinence; if left untreated, high bladder pressure can lead to renal failure and a lifetime of dialysis, or renal transplantation. The conventional surgical approach to increase bladder size is bladder augmentation [ileocystoplasty], which is associated with significant morbidity. In our laboratory, we are interested in designing a synthetic bladder augmentation patch to increase the bladder storage capacity. This approach would reduce much of the current surgical morbidity, and would also eliminate the metabolic complications of ileocystoplasty. Studies are underway to determine the biocompatibility of the synthetic patch to determine its suitability for use in vivo.

Effect of Androgens on Development of Genitourinary Tissue : Congenital Adrenal Hyperplasia is an inherited deficiency of certain enzymes involved in the production of male hormones [such as androgens]. The most common deficiency is 21-hydroxylase, the enzyme involved in cortisol production. The deficiency of 21-hydroxylase not only decreases cortisol, but also stimulates adrenocorticotropic hormone, leading to excess male hormones. In females, the result of this enzyme deficiency is virilization [the appearance of secondary male characters in the female], which begin in utero; these girls are born with genital ambiguity and an enlarged clitoris. The molecular mechanisms controlling androgen's action in the clitoris are unknown. Therefore, we are studying the in vitro expression of androgen and estrogen receptors in surgical waste tissue obtained from CAH patients. These preliminary studies will allow us to understand how androgens act on female genitalia, so that we may be able to design strategies to prevent female genitals from the negative effects of androgen excess in CAH.

<u>Wound Healing</u>: The healing of acute cutaneous wounds requires interactions among cytokines, immune cells, parenchymal cells, and components of the extracellular matrix. This process is dynamic and results in scar formation, which restores functional continuity in the affected area. Compromise of the wound-healing process contributes to significant morbidity and even death. Our laboratory has developed a model in which to study wound healing in full thickness human skin. This model was originally developed using pediatric foreskin and was used in several studies by our laboratory. We have recently expanded the model to use adult tissue and to study aspects of the immunology of wound healing in both adult and pediatric skin. We have further adapted this model for use in studies on squamous cell carcinoma.

<u>Students' Role in the Project:</u> Students will learn basic biochemical and molecular biology techniques including immunostaining, PCR, and western blot analysis. They will use these skills in experiments evaluating the effects of pressure on cells in the urinary tract.

<u>Preferred Background/ Experience</u>: Willingness to learn and work hard and committed interest are prerequisites.

#### Maura D. Frank, MD

Department of Pediatrics The Weill Medical College Helmsley Tower Room 508 212-746-3353 mdfrank@med.cornell.edu

Field(s) of Interest: Obesity

<u>Research Title</u>: Effect of weight management program on weight/BMI, eating and physical activity behaviors, and quality of life.

<u>Project Description</u>: Data entry and management, study recruitment, medical student mentoring program, IRB proposal development.

<u>Students' Role in the Project:</u> Student will learn the basics of research project development, recruitment for research projects, formulation of an abstract.

Preferred Background/ Experience: Knowledge of Excel helpful, student wiil learn EndNote

**Cori Green, MD, MS** General Academic Pediatrics Department of Pediatrics, Weill Cornell Associate Director of Pediatric Undergraduate Medical Education Director, Pediatric Sub-Internship 212-746-0921 cmg9004@med.cornell.edu

<u>Field(s) of interest</u>: Integration of pediatric mental health (MH) care into primary care, mental health screening, co-located models of behavioral health, resident education in pediatric mental health care

<u>Current Project Title:</u> The Need for Community Resources to Best Integrate Mental Health Care into the Primary Medical Home

Principal Investigators: Dr. Cori Green, Dr. Elisa Hampton, Dr. Eleanor Bathory

<u>Project Description:</u> We are conducting a needs assessment of how our clinic is doing with caring for our patients early mental health needs. We are assessing residents and parents through qualitative and quantitative research methods. We are also performing community asset mapping to improve our patients' access to MH resources in the community.

<u>Students' Role in the Projects:</u> Students will be involved in recruitment of resident subjects, analysis of data, and abstract writing.

Preferred Experience: None required

Daniel W. Green, MS, MD Hospital for Special Surgery 535 East 70<sup>th</sup> Street, New York, NY 10021 212-606-1631 greendw@hss.edu

Field(s) of Interest: Pediatric Orthopedic Surgery and Scoliosis

Research Title: Selected clinical projects in pediatric orthopedic surgery

<u>Project Description</u>: Previous projects include: DDH, congenital muscular torticollis, discoid meniscus, scoliosis and kyphosis.

Students' Role in the Project: Literature review, radiograph review, data analysis

Preferred Background/ Experience: None requested

Alan Groves, MD Neonatology Department of Pediatrics, Weill Cornell Medical College 212-746-3530 alg2049@med.cornell.edu

Field(s) of Interest: Heart function in the newborn

Project Description: Upcoming project on hemodynamics in the newborn.

<u>Students' Role in the Project</u>: Possibly help analyze echocardiography and bioimpedance data to assess the impact of clinical interventions on brain blood flow.

#### Barry Kosofsky, MD, PhD

Department of Pediatrics, Division of Neurology The Weill Medical College of Cornell University 525 East 68<sup>th</sup> Street, Room LC-6 212-746-3321 bar2009@med.cornell.edu

<u>Research Title</u>: Identifying biomarkers of mild traumatic brain injury (mTBI)

<u>Project Description</u>: We have a multidisciplinary basic (based at the Burke Research Institute) and clinical (based at NYPH/WCMC) translational research program to identify molecular (signaling pathways), functional (using eye tracking, and neuropsychologic assessments), and structural (using DTI/MRI) changes in the brain following mTBI. Our goal is to identify a set of actionable biomarkers reflective of mTBI as a starting point for designing a clinical trial.

<u>Students' role in the project</u>: Basic and/or Clinical Research Skills, including molecular biology, DTI/MRI, and neuropsychologic analyses.

<u>Preferred Background/ Experience</u>: Bench/wet lab or clinical/dry lab experience preferred (especially familiarity with animal models of mTBI, or prior work in clinical trials).

#### Nicole Kucine, MD

Pediatric Hematology/Oncology Department of Pediatrics 212-746-3873 nik9015@med.cornell.edu

<u>Field(s) of Interest</u>: Sickle Cell Disease, Anemia, Coagulation and Bleeding Disorders, Myeloproliferative Neoplasms, Leukemia, Bone Marrow Failure/Abnormal Hematopoiesis

<u>Potential Research Topics</u>: I am currently conducting research with various pediatric residents as well as on my own, and have some research interests that have not yet been developed. Topics of research that may be eligible for student involvement include:

- 1. Development of a database for Pediatric Myeloproliferative Neoplasms
- 2. Evaluation of thrombosis in pediatric patients (multiple projects under discussion)
- 3. Evaluation of practitioner attitudes and assessment of barriers in providing adequate pain management to children with Sickle Cell Anemia in the Pediatric ER
- 4. Issues surrounding chronic illness and mental health

#### Juhi Kumar, MD, MPH

Pediatric Nephrology Department of Pediatrics Weill Cornell Medical College 646-962-2037 juk2013@med.cornell.edu

Field(s) of Interest: Pediatric renal disease, vitamin D, kidney transport, Focal segmental glomerulosclerosis

Research Projects:

- 1. <u>Vitamin D in children with chronic kidney disease (CKD): prevalence of deficiency and clinical correlates</u>: This is a NIH funded ancillary study to the ongoing multicenter, prospective cohort study of children with CKD (CKiD). This study aims to define the prevalence and correlates of vitamin D deficiency. It will also prospectively evaluate the role of Vitamin D deficiency in growth failure, progression of CKD and cardiovascular outcomes.
- 2. <u>Vitamin D supplementation in children with chronic kidney disease:</u> Current guidelines for vitamin D supplementation in children with CKD are not evidence based and are extrapolated from adults. This study aims to evaluate the adequacy of the current KDOQI recommendations for treating vitamin D deficiency in these children.
- 3. <u>Kidney transplant outcomes:</u> This proposal aims to evaluate the effects of using a steroid free immunosuppression protocol on outcomes such as growth, allograft rejection and cardiovascular profile.
- 4. <u>Gut microbiome in Chronic kidney disease:</u> This proposal aims to examine the gut microbiome in patients with CKD and also determine the impact of the gut microbiome on immune function.

#### Thomas J.A. Lehman, MD

Hospital for Special Surgery Pediatric Rheumatology 535 E. 70<sup>th</sup> St. 212-606-1158 lehmant@hss.edu

Field(s) of Interest: Pediatric rheumatic diseases

Project Description: Students have been involved in a variety of clinical research projects over the past years.

Students' Role in the Project: Chart review, data tabulation. We also teach basic aspects of clinical pediatric rheumatology/history.

Preferred Background/ Experience: None requested

David C. Lyden, MD, PhD Stavros S. Niarchos Professor Children's Cancer and Blood Foundation Labs 413 East 69<sup>th</sup> Street Rm 1228, Belfer Building dcl2001@med.cornell.edu

Field(s) of Interest: Cancer Metastasis

Research Title: Tumor exosomes determine pre-metastatic niche formation and organotropism

<u>Project Description</u>: Tumor micropartilces known as exosomes are released into the circulation and fuse with specific cells at distant sites establishing a pre-metastatic niche in cancer patients. Tumor exosomes transfer exosomal tumor contents (proteins, miRNA and DNA) into normal cells and "educate" these cells to a pro-metastatic phenotype.

<u>Students' Role in the Project</u>: The student will be responsible for determining the key factors associated with exosomes that support their role in organotropism.

Preferred Background/ Experience: None requested

Catharine McGuinn, MD Pediatric Hematology/Oncology Department of Pediatrics, Weill Cornell Medical College 212-746-3400 cam9061@med.cornell.edu

Field(s) of Interest: Benign Hematology, Thrombosis, Coagulation, Thrombocytopenia

Research Title: Quality Improvement/ Outcomes in Pediatric Hematology Population

<u>Description of Project(s)</u>: To be decided in conjunction with research team. Prospective survey or retrospective chart review format. Ideas include looking at sickle cell pain management pathway, anti-coagulation adherence, etc.

Students' Role in the Project: Flexible. Would be developed as project expanded

Preferred Background/ Experience: None requested Enthusiasm is important.

W Beau Mitchell, MD Pediatric Hematology/Oncology Department of Pediatrics, Weill Cornell Medical College Laboratory Address: New York Blood Center Platelet Biology 212-570-3280 E-mail Address: bmitchell@nybloodcenter.org

Fields of Interest: Clinical and laboratory aspects of bleeding, clotting, and platelet biology

<u>Research Project 1</u>: Bleeding complications in patients with connective tissue disorders. <u>Description of Project</u>: We have seen a series of patients with connective tissue disorders who present with bleeding. This project will be a retrospective chart review to compile and analyze the bleeding characteristics of this population. Given the large sample size this project should provide unique information about bleeding in connective tissue disorders.

Students' Role in the Project: Chart review. Assistance with IRB process, scientific writing.

Preferred Background/ Experience: None

Research Project 2: A novel mutation resulting in an unusual type 2A von Willebrand Disease

<u>Description of Project:</u> We have identified a family with a novel mutation causing severe type 2A VWD. The mutation completely eliminates some aspects of von Willebrand factor function, but leaves others intact. Review of these patients' laboratory and clinical findings in concert with what is known of the VWF structure will likely reveal novel information about VWF structure and function.

<u>Students' Role in the Project</u>: Chart review. Assist with IRB submission. Review of literature. Scientific writing. Work with 3D structure imaging software.

Preferred Background/ Experience: None, although it would help if adept at computers.

<u>Research Project 3</u>: Morphology of platelets during thrombopoiesis

<u>Description of Project:</u> We are producing platelets from stem cells derived from umbilical cord blood cells. One critical question is whether the produced platelets are "normal". To determine this we are analyzing the platelets in several different ways. One of these ways is by morphology. We use both light and fluorescence microscopy to study the platelets as they are being produced in culture. This project will establish a baseline morphology by which to judge the effects of changes in the production techniques.

<u>Students' Role in the Project:</u> This will be primarily a visual cataloguing of microscopy images. The student will learn to use our imaging software and microscopes.

Preferred Background/ Experience: None, but will have to take the NYBC volunteer orientation.

Anne Moscona, MD Matteo Porotto, PhD Pediatrics and Microbiology/Immunology 515 East 71<sup>st</sup>, 6<sup>th</sup> floor 212-746-4523 anm2047@med.cornell.edu

<u>Field(s) of Interest</u>: Infectious diseases – Virology (specifically respiratory viruses and viral agents of bioterrorism).

<u>Research Title</u>: Molecular pathogenesis of human paramyxoviruses: parainfluenza virus type 3, and Hendra virus.

<u>Project Description(s)</u> The laboratory's research centers on molecular pathogenesis of human paramyxoviruses: parainfluenza virus type 3, and also the emerging pathogen Hendra virus. Parainfluenza virus is an important cause of lower respiratory tract infections in children, including croup and bronchiolitis, and there are currently no vaccines or antiviral agents for these diseases. Hendra virus is a highly fatal paramyxovirus is a potential agent of bioterrorism. We are interested in how viruses enter cells by fusing with the cells' envelope, and in how we might interfere with entry.

<u>Molecular basis for human parainfluenza virus 3 infection.</u> This laboratory has identified the role of the parainfluenza virus receptor-binding protein hemagglutinin-neuraminidase (HN) in the virus-induced fusion process whereby all paramyxoviruses enter host cells. HN's receptor binding is the critical first step towards HN's role in fusion promotion, and leads to activating or "triggering" of the fusion protein (F) to mediate fusion. Our parainfluenza projects focus on the molecular mechanisms for HN in the viral life cycle and in lung pathogenesis. Ongoing studies have led to novel antiviral strategies that are being tested, and to understanding mechanisms of resistance to antivirals.

Role of human parainfluenza virus 3 hemagglutinin-neuraminidase in immunopathogenesis of lung disease. The role of HN in pathogenesis of lung disease in vivo is being studied in a cotton rat model. Our group showed that mutations in HN that alter HN-receptor interaction (but do not affect replication) lead to dramatic differences in the disease in the cotton rat lung. We are determining whether HN's receptor affinity, its receptor-cleaving, or its F-triggering activities determine its virulence in the lung. We also are interested in identifying which immune response is altered by HN mutations that lead to enhanced disease.

<u>Triggering of fusion by Hendra virus F protein: the role of G</u>: In the Hendra virus projects, we apply our strategies for the study of paramyxovirus entry and fusion to an emerging and potentially fatal paramyxovirus that is viewed as a potential bioterrorism agent. For Hendra virus, the receptor binding protein (G) is required in order for the F protein to mediate fusion. Hendra G binding to receptor, like parainfluenza virus HN binding to sialic acid, "triggers" F protein to mediate fusion. The study of the mechanism of triggering/activation of F protein in Hendra virus should lead to strategies for interfering with this key step in viral entry.

<u>Triggering Hendra F to fuse: peptides to interfere with fusion</u>: Taking advantage of our finding that peptides that correspond to regions of HPIV3 F are superior to those derived from Hendra F at inhibiting Hendra fusion, we will determine whether inhibitory F-peptides bind to Hendra F, as would be predicted if the peptides are preventing the formation of the 6-helix bundle (6HB) believed to be required for fusion, or if inhibitory F-peptides bind to G and interfere with G triggering of F.

Innovative approaches to developing therapeutic and diagnostic reagents for Hendra virus. Insertion of F into the target cell membrane leads to fusion of the viral envelope with the plasma membrane and release of the nucleocapsid into the cytoplasm. Efficiency of F-triggering by G influences the extent of fusion, and provides a range of strategies for preventing viral entry. Based upon our studies of the paramyxovirus F-triggering

process, peptides corresponding to heptad repeat regions of F can be used to prevent F from reaching its fusion-active state. It may also be possible to induce F to trigger "prematurely", thus becoming incapacitated before it reaches its target. Finally, molecules that inhibit receptor binding may prevent receptor interaction and all downstream events. Targeting several stages of the entry process simultaneously may provide synergism.

<u>Students' Role in the Project:</u> Virology, molecular biology, biochemistry, structural analysis, immunology

Preferred Background/Experience.

Some lab experience preferred but not required. Interest and motivation are required.

Snezana Nena Osorio,MD General Academic Pediatrics Department of Pediatrics, Weill Cornell 212-746-3457 snm2001@med.cornell.edu

Field(s) of Interest: Obesity and Medical Communication skills

Title of Research Project: Medical Communication Skills and Exploratory cancer project

<u>Project Descriptions</u>: The project includes goals for empirical evaluation of the family-centered care program. There are two projects on Medical Communication Skills. A Third project is focused on the natural history of pre-malignancy and the metastatic niche.

<u>The first project</u> will assess parent satisfaction with patient care before and after the introduction of Family-Centered Rounds. Parent perceptions of clinical care will be assessed among all families of hospitalized pediatric service patients upon admission. Time series analyses will be used to evaluate changes in patient satisfaction in response to the new strategy for bedside rounds.

<u>The second project</u> is a study to evaluate the impact of Family Centered Rounds on medical commun-ications skills among pediatric residents. The study was designed with the directors of pediatric resident training at KCCH to introduce standardized scales to assess resident communication skills by parents, nurses, and supervising physicians. I will use the existing data from education files without identifiers from two time periods to assess the utility of a new clinical program of family-centered rounds. I am also working on developing communication curriculum for pediatric residents.

<u>The third project</u> is collaborative with Dr. David Lyden. This is an investigation of profiles of angiogenic and metastatic parameters in children with and without cancer. Emerging evidence suggests that bone marrow-derived, hematopoietic stem progenitor cells (HPC's) and endothelial progenitor cells (EPC's) contribute to tissue vascularization during both embryonic and postnatal physiological processes. Identification of cellular mediators and tissue-specific chemokines, which facilitate selective recruitment of bone marrow-derived stem and progenitor cells to specific organs, may provide insight into the mechanisms by which the pre-metastatic niche develops in patients with pediatrics malignancies. In this study, we seek to compare peripheral levels of circulating chemokines and progenitor cells in healthy pediatric controls to those of age-matched patients with pediatric malignancies. My role in this project involves recruiting and profiling blood samples from children who do not have cancer. Obtaining this information will be crucial in defining norms for the measures to be gathered among cancer patients. We will analyze blood samples gathered as "extra" blood when children already are undergoing blood testing. From these samples, we will measure plasma levels of growth factor and chemokine profile. This profile includes VGEF (vascular endothelial growth factor), PIGF (Placental

derived growth factor ), FGF (fibroblast derived growth factor ), and SDF-1 (stromal derived growth factor). We have obtained IRB approval for this project, which also is approved by the WCMC CTSC.

I am also expanding my research to study angiogenesis and vasculogenesis among children who are overweight, as it is well known that the population of obese pediatric patients faces a future that includes elevated risk for cancers and cardiovascular problems. Research in adults has demonstrated that angiogenic factors are elevated in overweight and obese individuals. Furthermore, previous research demonstrated that coupling of adipogenesis and angiogenesis is essential for differentiation of adypocytes in obesity and that vascular growth factor (VGEF) is a key mediator. We will explore what genetic variants are responsible for this effect and possible predict which subtypes of obesity are more prone to cancer. This research can be enhanced by studying other family members with obesity and malignancies to determine the genetic factors involved in the pre-metastatic setting.

<u>Students' Role in the Project</u>: 1. Medical Communication skills and patient satisfaction projects will provide the student with opportunities to learn how to develop questionnaire as an assessment tool, IRB process, how to analyze data. The third project is now centering on obesity-data collection, data entry, data analysis in collaboration with Dr. Lyden's lab.

Preferred Background/ Experience: None

Jeffrey Perlman, MD Department of Pediatrics, Division of Neonatology Weill Medical College jmp2007@med.cornell.edu 212-746-3530

Field(s) of Interest: Neonatology, Brain development, Resuscitation, Global health

<u>Title of Research Project</u>: Evaluation of the Ergonomics of Chest Compressions in a Neonatal Manikin Model

<u>Project Description</u>: Evaluate the influence of compression rates on the depth of compressions including decay over time as well as the potential influence of surface location and gender as well as the impact of ventilations

<u>Students' Role in the Project:</u> Assist in the evaluations of data following a session and help to develop strategies to enhance CPR in the neonatal period

Preferred Background/ Experience: None

Cathleen L. Raggio, MD Hospital for Special Surgery 212-606-1339 raggioc@hss.edu

Project Description: Pediatric, clinical and lab research. Spine Osteogenesis Imperfecta, Skeletal Dysplasia

Students' Role in the Project: Patient interaction, dissection, x-ray review, computer work

Preferred Background/ Experience: Good work ethic and enthusiasm

#### **Stefano Rivella, PhD** Pediatric-Hematology/Oncology 515 East 71<sup>st</sup> Street

212-746-4941 str2010@med.cornell.edu

#### Research Title:

1) Development of new strategies to cure beta-thalassemia

2) The role of iron in cancer and anemia of inflammation

#### Project(s) Description: Gene therapy of beta-thalassemia

Abnormal erythropoiesis and iron metabolism in beta-thalassemia The role of iron metabolism in cancer and anemia of inflammation

The projects include:

- Design and generation of retro or lentiviral vector harboring genes involved in abnormal hematopoiesis and iron disorders
- Design and generation of retro or lentiviral vector with genomic elements to regulate gene expression
- Test of the system in vitro
- Test of the system *in vivo* (infection of hematopoietic stem cells, embryonic stem cells, bone marrow transplantation and/or generation of transgenic animals)
- Generation of new tumor models and their correlation with inflammation, anemia and tumor progression

#### Students' Role in the Project:

Microbiology: bacteria transformation, plasmid DNA preparation

Molecular Biology: generation of recombinant DNA vectors, Southern blot analysis

Tissue Culture: maintenance and expansion of primary and secondary tissue culture cell lines; retroviral production, viral transduction

Mouse handling: analysis of hematopoietic parameters (CBC), facs, iron, gene and protein analyses, gene transfer in the bone marrow and liver, tumor induction and analysis

<u>Preferred Background/ Experience</u>: Basic and good knowledge of molecular biology and laboratory techniques; good skills in reviewing and summarizing scientific literature. The subjects that the

candidate will review include: retrovirus, RNA interference, tetracycline controlled gene expression system, mouse embryonic stem cells. Good organization skills; computer literate.

#### Heidi Stuhlmann, PhD

Developmental Biology Department of Cell & Developmental Biology (primary) Department of Pediatrics (secondary) 212-746-6156 hes2011@med.cornell.edu

Research Title: Placental Development and Placentopathies

The placenta serves as the site of contact for the maternal and embryonic circulatory systems to enable nutrient and gas exchange. It contains two primary functional cell types, trophoblast and endothelial cells.

Proper placental development requires invasion and differentiation of trophoblast cells, as well as coordinated maternal vascular remodeling and fetal vasculogenesis. Any disruption in these processes can result in placental pathologies, including preeclampsia (PE). PE is a leading cause of maternal and fetal morbidity and mortality worldwide, and the only resolutive treatment is delivery of the baby and placenta. Although the pathophysiology of PE remains largely unknown, inadequate trophoblast cell invasion, endothelial cell dysfunction, dysregulated uteroplacental vascularization, and an imbalance of pro- and anti-angiogenic growth factors have been implicated in the disease.

We are using mouse models and human placentas to investigate the role of EGFL7 during normal and pathological placental development. EGFL7 is a secreted factor that was previously thought to be endothelial-restricted in its expression. However, our recent studies revealed that Egfl7 is expressed in the placenta by the endothelium of both the maternal and fetal vasculature, as well as in a previously unknown site, the trophoblast cell lineage. Our results showed a significant down-regulation of Egfl7 expression in human PE placentas at term and in compromised placentas of a mouse model of PE prior to the onset of the characteristic maternal signs of PE (Lacko et al., 2014. Novel expression of EGFL7 in placental trophoblast and endothelial cells and its implication in preeclampsia. Mech. Dev. 133:163-176).

Preliminary studies from our lab, using gain- and loss-of-function mouse models, point to specific roles for EGFL7 during placental development. We also have a keen interest to understand its role in human placentas and preeclampsia. Specifically, we plan to investigate if EGFL7 protein van be detected in the serum during pregnancy, and if EGFL7 is an early indicator for the onset of preeclampsia.

<u>Students' Role in the Project</u>: The student would get "hands-on" lab experience. Initially, the student would work together with a research scientist in the lab to learn and master the required techniques, and later work more independently. The laboratory techniques could involve:

ELISA assay; extraction of protein from tissue sample; protein gel electrophoresis; western blot analysis; extraction of DNA from tissue samples; PCR amplification, DNA gel electrophoresis, preparation of sample for DNA sequence analysis; dissection of mouse embryos; embedding and sectioning, immunostaining/ immunofluorescence analysis

<u>Preferred Background/ Experience</u>: Basic lab skills, knowledge in molecular and developmental biology, strong interest in research

Sima Toussi, MD Division of Infectious Disease Department of Pediatrics, Weill Cornell 212-746-7379 sst2002@med.cornell.edu

Field(s) of Interest: Pediatric Infectious Diseases

Research Title 1: Clostridium difficile colonization in infants and young children

<u>Project Description</u>: *Clostridium difficile* can cause diarrhea and severe illness in children and adults. *C. difficile* infection is likely under-recognized in the young pediatric population. Infants and young children are often not evaluated for *C. difficile* infection because it is thought to colonize their gut. However, it is unknown how commonly it colonizes the stool of young children. The rates published are extremely wide ranging and reported as being anywhere from 10-100% during the first year of life. The objective of this study is to describe the prevalence of *C. difficile* colonization in infants and young children and to assess possible risk factors.

<u>Students' Role in the Project</u>: The student's role will be the recruitment of study subjects in the inpatient and outpatient settings. This would involve learning how to consent and enroll patients with one of the co-investigator's and then eventually doing this independently. Part of the student's role will also be entry of the information into the database.

Preferred Background/ Experience: Interest in participating in clinical research.

**Chani Traube, MD** Pediatric Critical Care Medicine Department of Pediatrics, Weill Cornell 212-746-3056, chr9008@med.cornell.edu

Field(s) of Interest: Pediatric Critical Care Medicine; Pediatric Neuro-Intensive Care

<u>Research Title</u>: A Prospective Longitudinal Assessment of Pediatric Delirium, Associated Risk Factors and Short Term Outcomes in Pediatric ICU Patients

Project Description:

The pediatric critical care community has just begun to explore delirium in its population, but an emerging literature indicates a prevalence greater than 20%, with associated short- and long-term morbidity. With an estimated 200,000 children admitted to intensive care in the US annually, more than 40,000 children are likely affected each year. At Cornell, we have implemented universal delirium screening in the PICU as standard of care. This study is designed to define the natural history of pediatric delirium, identify associated risk factors, and assess the impact of delirium on long-term cognition, behavior, and psychological health.

<u>Students' Role in the Project</u>: Students will have the opportunity to join a multidisciplinary team engaged in several projects regarding pediatric critical illness and delirium. They will participate in research study design, data collection, and manuscript writing. Students will learn how to obtain informed consent, conduct chart reviews, analyze data, and perform follow-up phone calls using surveys to detect whether a patient has experienced long-term effects from delirium.

<u>Preferred Background/ Experience</u>: None required. Interested students should be friendly, comfortable interacting with children and their families, and demonstrate organizational skills and attention to detail. Research is ongoing, with active clinical trials in progress, others pending IRB approval, and others in planning stage.

Susan J. Vannucci, PhD Neonatology Department of Pediatrics, Weill Cornell 212-746 1446 suv2003@med.cornell.edu

<u>Field(s) of Interest</u>: Developmental Brain Injury/Hypoxic Ischemic Encephalopathy/Hypoglycemia/Neonatal Seizures

Research Title: Hypoxia-Ischemia in the Immature Brain

<u>Project Description</u>: Hypoxic-Ischemic (HI) brain damage resulting from asphyxia in the neonatal period is a major cause of death of premature and term infants and responsible for permanent neurologic handicap in the survivors. We have developed an animal model to study this injury in the newborn rat and utilize this model in

both preterm and term-equivalent rodents. HIE is a major cause of seizures yet there is continued debate as to whether these seizures contribute to or merely reflect the severity of brain damage. We have recently extended our HIE model to include the detection of behavioral and electrographic seizures to test several of these relevant questions. A second project using this model will continue to look at the role of mast cells in promoting inflammation and cell death following HI in the immature brain.

<u>Students' Role in the Project</u>: The student can assist in performing the surgeries to induce the hypoxiaischemia, as well as in the recording of the video EEG. It is important that the student is comfortable working with animals and in survival surgeries as well as in euthanasia of the animals to study the effects on brain development and injury. In addition, the student could participate in the study of the role of mast cells in mediating the inflammatory cascade as well as potentially contributing to the tissue repair.

#### Maria Vogiatzi, MD

Metabolic Bone Disease Pediatric Endocrinology Department of Pediatrics 212-746-3462 or 212-746-3486 mvogiatz@med.cornell.edu

#### Research Topics:

*Studies of osteoporosis in thalassemia:* Thalassemia is a congenital hemolytic anemia that is associated with high rates of osteoporosis. The etiology of osteoporosis in this disease is poorly understood. Our project examines the etiology of bone disease in thalassemia, by doing both clinical and animal studies. We use a mouse of model of thalassemia to determine the effect of certain medical interventions (such as PTH and bisphosphonates). The methodology that is used includes imaging, such as micro-CT, histology for assessment of bone remodeling and other basic science techniques.

*Effect of erythropoiesis on mesenchymal differentiation:* In this project, we use the thalassemia mouse as of model to study the effect of hematopoiesis on bone remodeling. Our results so far support the hypothesis that hematopoietic progenitors affect mesenchymal differentiation leading to decreased osteogenesis. In addition, we have identified that this process involves erythropoietin (EPO). Cell cultures and co cultures, other basic science techniques and other mice models are used to examine the interactions of EPO and autocrine/paracrine factors on bone remodeling.

The role of iron in the development of osteoporosis: This project involves studies in iron overloaded mice, and the effect of iron overload on bone remodeling. The methodology that is used includes imaging, such as micro-CT, and histology for assessment of bone remodeling. Cell cultures and other basic science techniques are used to determine the role of iron on mineralization and the osteoclast.

*Inflammatory response to iron excess:* Iron excess has been thought to lead to increased oxidative stress. Our animal data support the presence of ROS and an inflammatory response. Our lab is in the processing of delineating the molecular mechanism by which iron excess triggers an inflammatory cascade. This is done by performing animal experiments using techniques such as flow cytometry.

Studies of diabetes in iron overload and thalassemia: Iron excess is associated with a number of endocrinopathies including diabetes. This project determines the development of insulin resistance and diabetes in our murine diabetes model as well the role of oxidative stress and inflammation in this process. In addition, we are conducting clinical studies that examine glucose abnormalities in iron overloaded patients with thalassemia by using continuous glucose monitoring by glucose sensors.

*Studies of vitamin D supplementation on calcium excretion in thalassemia*. This project studies the effect of various vitamin D doses on serum vitamin D concentrations and calcium excretion in regularly transfused patients with thalassemia. The study is supported by Cooley's Anemia Foundation grant.

<u>Students' Role in the Projects</u>: The student can be exposed to imaging techniques such as microCT, bone histology and dynamic histomorphometry for assessment of bone remodeling and basic science techniques including cell cultures and flow cytometry. The student will also have the opportunity to participate in clinical research in the area of diabetes and osteoporosis.

<u>Preferred Background/ Experience</u>: The student must be familiar with basic laboratory procedures. Biology majors preferred

Mary Jo Ward, PhD Division of Child Development Department of Pediatrics The Weill Medical College of Cornell University mjward@med.cornell.edu

Field(s) of Interest: Development: infants, children, mother-child interaction

Research Title: Infant feeding skills, parental feeding practices, and growth disorders

<u>Project Description</u>: We will evaluate the effectiveness of an intervention delivered to the parents of infants from birth to 6 months of age. The study will include 75 families in a standard care group and 75 in an intervention group. The first group (standard care group) will receive routine well-child care on the schedule recommended by the American Academy of Pediatrics. The second (intervention group) will receive routine well-child care plus an intervention focused on teaching parents about age-appropriate infant nutrition and infant feeding skills. Group assignment will be made on the basis of historical cohort membership: the standard care group will be enrolled first and the intervention group enrolled approximately 3 months later. Subjects in both groups will be followed for 6 months. Outcome measures include parent feeding practices, infant diet, infant feeding skills, and infant overweight. Measures will address cultural and familial biases in favor of overweight children.

The following hypotheses will be tested:

- Compared to parents in the standard care group, more parents in the intervention group will report feeding only single-grain infant cereal and Stage 1 fruits and vegetables to their 6 month-olds. In contrast, more parents in the standard care group will report feeding Stage 2 and 3 foods, snacks, juice, and table foods.
- At 6 months, the rate of infant weight for length above the 75th percentile will be higher in the standard care versus intervention group.
- Parents in the intervention group will be more likely to report receiving accurate information about infant feeding and nutrition from their pediatricians than parents in the standard care group.
- More infants in the intervention than standard care group will use a cup for drinking and fewer will have been fed solid food in a baby bottle.

<u>Student's role in the project</u>: Students will be trained to conduct standardized interviews, to gather anthropometric data on adults and children, and to monitor delivery of the intervention, according to the research protocol.

<u>Preferred Background/ Experience</u>: Skills in interacting with adults from varied cultural backgrounds, interest in infant growth and development and primary care intervention models.

#### Stefan Worgall, MD, PhD

Pediatrics / Genetic Medicine 515 E 71 St, S-600B 212-746-4875 stw2006@med.cornell.edu

Field(s) of Interest: Cystic fibrosis / host defense in lung / gene therapy

<u>Research Titles</u>: Lung antigen presenting cells in cystic fibrosis Respiratory syncytial virus vaccine using capsid-modified adenovirus vectors

#### Project Descriptions:

1. Cystic fibrosis lung disease is characterized by exaggerated inflammation and increased susceptibility to infections. Although the CFTR protein is primarily thought to be expressed by epithelial cells we and others have studied the expression of CFTR in non-epithelial cells, in particular antigen presenting cells in the lung. This project studies the abnormalities of lung dendritic cells derived from CF knock-out mice. Our data so far indicates that abnormal CFTR expression lung macrophages and dendritic cells is related to abnormalities in innate immune responses. These findings are important in understanding lung disease in CF and also to identify new targets for therapy of this severe disease.

2. Infections with RSV are one of the major causes for viral lower respiratory tract illness, especially in young children. Our laboratory has been working on the development of genetic vaccines for pulmonary pathogens. This project aims to analyze the immunological properties of a novel anti-RSV vaccine using a capsid-modified adenovirus vector. Protection against RSV could be achieved with an efficient vaccination strategy inducing neutralizing humoral immunity as well as a Th1-dominant cellular response. Adenovirus gene transfer vectors can be used to evoke robust systemic and mucosal immunity against an immunogen expressed as a transgene and Ad functions as a potent adjuvants. The Ad modifications include the addition of a RGD motif to the fiber knob, a modification known to enhance infection of antigen presenting cells and to increase Th1-type immune response, as well as the addition of RSV epitopes into the Ad capsid. These modified vectors will be assessed to induce immunity and protection against RSV in adult and neonatal mouse models. The study will evaluate if a modified Ad vector expressing the RSV F protein engineered to increase activation and infectivity of antigen presenting cells could be useful as a RSV vaccine.

<u>Students' Role in the Project:</u> Design of new and continuation of the present experiments. Student will be involved in cell culture studies and flow cytometry analysis of lung dendritic cells (project 1) and adenovirus vector construction and immunological analyses (project 2).

## **PROGRAM MATCHING**

## CLASS OF 2014 PEDIATRIC RESIDENCY MATCHES

#### NAME

Saifeldeen Alzoobaee Melissa Cain Crystal Castaneda Rula Green Gladden Alyssa Marchman Eunee Park Miheer Sane Alexandra Satty Dennis Spencer Sphoorti Umarjee Colleen Wichser Maria Zhadina Sisi Zheng Theresa Zhou Dara Zyburo

#### PROGRAM

Pediatrics Pediatrics

#### INSTITUTION

NYP Hosp-Weill Cornell Med Ctr-NY Einstein/Montefiore Med Ctr-NY Univ of Chicago Med Ctr-IL NYP Hosp-Weill Cornell Med Ctr-NY NYP Hosp-Columbia Univ Med Ctr-NY Georgetown Univ Hosp-DC UC San Francisco-CA NYP Hosp-Weill Cornell Med Ctr-NY Stanford Univ Progs-CA Baystate Med Ctr-MA Childrens National Med Ctr-DC North Shore-LIJ Health Sys-NY Childrens Hosp-Philadelphia-PA Icahn SOM at Mount Sinai-NY NYP Hosp-Weill Cornell Med Ctr-NY

#### **CITY, STATE**

New York, NY Bronx, NY Chicago, IL New York, NY New York, NY Washington, DC San Francisco, CA New York, NY Palo Alto, CA Springfield, MA Washington, DC Great Neck, NY Philadelphia, PA New York, NY New York, NY

## DEPARTMENT OF PEDIATRICS RESIDENCY PROGRAM GRADUATES CLASS OF 2014

#### PHYSICIAN

Alex Byron Julie Augenstein Sarah Blank Dawnnica Eastman Lindsay Edwards James Enos Lisa Fahey Bridget Godwin Nicole Grocky Ruchika Karnik Violetta Kivovich Peter Leimena Sharon Lewin Ayala Miller Sean O'Brien Kimberly O'Hara Katharine Rae Alvin Teodoro Jessica Wang Corey Wasserman

#### POSITION

**Preventative Medicine Residency Emergency Medicine Fellowship** Chief Resident General Pediatrician Cardiology Fellowship Cardiology Fellowship Gastroenterology Fellowship Gastroenterology Fellowship **General Pediatrician** Cardiology Fellowship Chief Resident **General Pediatrician** Neonatology Fellowship **General Pediatrician** Pediatric Emergency Attending **Chief Resident** Hematology/Oncology Faculty **General Pediatrician General Pediatrician** 

#### INSTITUTION

NYC DOH Seattle Children's Hospital NYPH - Weill Cornell Seaside Pediatrics, Hyannis, MA Texas Children's Hospital Children's National Medical Center Children's Hospital of Philadelphia Children's Hospital of Philadelphia Heights Pediatrics, New York, NY Mount Sinai MSKCC North Carolina NYPH - Weill Cornell Global Pediatrics, New York, NY Elmhurst Hospital NYPH - Weill Cornell MSKCC Columbia University Dept. of Pediatrics Manhattan Pediatrics Associates Weill Cornell Medical Associates