

- Staff weather the snow
- Lectures highlight the future of organ transplantation, cell therapy for ocular disease, medical care in the computer age

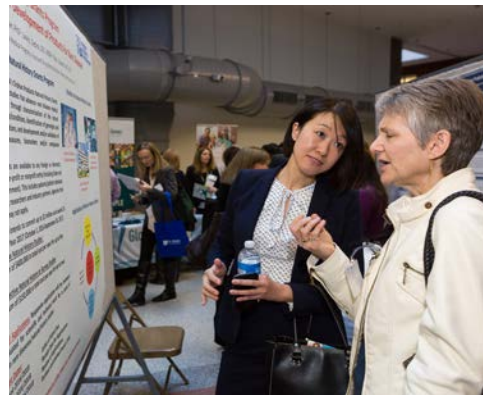


Rare Disease Day features patients' voices, international collaboration

On Feb. 29, Leap Day, the Clinical Center and the NIH National Center for Advancing Translational Sciences (NCATS) hosted the 2016 Rare Disease Day at NIH. The event, which took place worldwide on Feb. 29, is aimed at educating policymakers and the public about rare diseases and their impact on patients' lives.



Martha Rinker (left) speaker from the non-profit National Organization for Rare Disorders (NORD) addresses the audience on Rare Disease Day. The event also included a poster session (right) and exhibits.



few hundred have any treatment. Each rare disease affects fewer than 200,000 Americans. But, these illnesses affect an estimated 25 million people in the U.S. Less than 5 percent of rare diseases have a treatment. One such rare disease is the Ebola virus disease, which caused a severe epidemic in West Africa.

The event drew more than 500 attendees, a record high. Among the distinguished speakers were the co-chairs of the Rare Disease Congressional Caucus. Senator Orrin Hatch (R-UT) spoke via video, and Senator Amy Klobuchar (D-MN) and Representatives Joseph Crowley (D-NY) and Leonard Lance (R-NJ) each spoke in person about their work for legislation that will improve the lives of people with rare diseases.

The event initiated a mutually beneficial dialogue among public and private researchers, patients, patient advocates and policymakers; offered a venue to exchange the latest rare diseases information with stakeholders to advance research and therapeutic efforts; and put a face on rare diseases by sharing stories of patients, their families and their communities.

More than 6,500 rare diseases affect people around the world, of which only a

ity exceeding 11,000 deaths, numbers that tragically eclipse all prior outbreaks combined, the 2014-15 Ebola crisis in West Africa proved how previously rare infectious diseases can flare without warning in new and unexpected ways and command the world's attention," said Dr. Richard T. Davey, Jr., deputy clinical director of National Institute of Allergy and Infectious Diseases.

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Dept. of Transfusion Medicine improves safety of platelet transfusions

On Jan. 14, the Clinical Center Department of Transfusion Medicine (DTM) prepared the first pathogen reduced apheresis platelets at NIH and transfused them to a patient in the Intensive Care Unit. The CC became the first hospital in the U.S. to collect, prepare and transfuse pathogen reduced apheresis platelets.

Approved by the U.S. Food and Drug Administration in December 2014, the INTERCEPT Blood System for Platelets is used to inactivate a broad spectrum of viruses, bacteria and parasites as well as contaminating donor T-cells, a type of white blood cell, in platelet components. Blood components are very safe and are tested for various pathogens like HIV and the hepatitis B and C viruses. The INTERCEPT system may inactivate other microorganisms, such as the Zika virus, parasites and bacteria that



Betsy Furlong, Dr. Mike DeVan, Sherry Sheldon and Dr. Bill Flegel gather near the UV Illuminator, the main hardware component of the INTERCEPT Blood System.

can be difficult to detect.

The process uses amotosalen HCl (a light-activated compound) and ultraviolet (UVA) illumination to photochemically treat platelet components. This treatment procedure also inactivates donor T-cells to lower the risk of transfusion-associated

graft-versus-host disease, eliminating the need to expose the platelets to radiation previously performed for the same purpose.

The DTM INTERCEPT implementation team has been working since March 2015 to bring pathogen reduced apheresis platelets to CC patients. This has been a true team effort, touching almost every section within the department. Apheresis platelets are collected at DTM's satellite facility on Fishers Lane in Rockville, part of the Blood Services Section; primary production steps are performed in the Laboratory Services Section; and platelets are counted in the Cell Processing Section for quality control purposes. Others involved included DTM's Quality Assurance group and IT Support staff.

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Dr. Allan D. Kirk honored as NIH Distinguished Clinical Research Scholar and Educator in Residence

Dr. Allan D. Kirk, a former NIH staff member and an internationally recognized surgeon and expert on transplant immunology, has been named the 2016 Distinguished Clinical Research Scholar and Educator in Residence. As a part of this award, the fourth to be honored, Kirk presented the Clinical Center Grand Rounds lecture Feb. 10 on “Costimulation Blockade for Organ Transplantation.”

Dr. John I. Gallin, director of the NIH Clinical Center, described Kirk as a great teacher and outstanding selection for this honor. Kirk serves as the editor-in-chief for the American Journal of Transplantation, is the David C. Sabiston, Jr. Professor and Chairman of Surgery at Duke University School of Medicine and is surgeon-in-chief of the Duke University Health System. He has mentored over 40 pre- and post-doctoral students during his medical career.

Kirk spoke about organ transplant recipients, who have to take immunosuppressive drugs throughout their life to prevent rejection of their graft. He stated that the survival rate of kidney transplants was now well over 96% in a year, that the rejection rate is about 10 to 15%, and the loss to rejection is almost zero. He went on to discuss how the multiple medications have to be taken daily and that there are still side effects and economic problems related to the current process.

“There’s probably a better way,” he stated.

He then went on to discuss multiple completed and ongoing studies that could one day help transplant patients by allowing them to switch from daily to monthly or bi-monthly medications. While optimistic, he stressed that “we still have a long way to go.”

In the lecture, Kirk described how special the Clinical Center is and how it is a place of continuous flow and insight, from the clinic back to the labs



Dr. John I. Gallin, left, director of the CC, presented a plaque to Dr. Allan D. Kirk, the 2016 Distinguished Clinical Research Scholar and Educator in Residence.

through translational studies. In fact, he said what happens here is so extraordinary and important not only because it takes care of patients or gives new scientific insight; but because the Clinical Center drives biomedical inquiry and policy which influences patient treatment in the U.S. and around the world.

In speaking with some of the medical students during his visit, Kirk stated, “It is important to emphasize that learning how to ask a question and then using that question to guide your career is really the way to focus on being a biomedical investigator.”

“This place does great science as second nature. There has to be this seamless flow of information from the lab to the clinic, from the policymakers to the clinicians, from the translational models to clinical models to basic models,” Kirk said. “This place is mind-numbingly inefficient. Embrace that! Some inefficiency is required; it is the only way you can do what you need to do in science. The drive for efficiency often prevents the required interactions between all the elements of science, and this is one of the few places that preserves that beautiful quirky inefficient productive scientific atmosphere.”

View the videocast: <http://videocast.nih.gov/launch.asp?19482>

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“Not only have we implemented the INTERCEPT pathogen reduction system, but we’ve simultaneously introduced the use of Platelet Additive Solution (PAS) as part of the collection process,” said Dr. Bill Ward, deputy chief of DTM. PAS is a buffered salt solution that replaces approximately 65% of the plasma in the apheresis platelet product. PAS products have recently been approved for use in the U.S. Research suggests that PAS may reduce certain types of transfusion reactions that may be seen in some patients, and improve platelet quality.

In 2015 alone, more than 3,500 apheresis platelet transfusions were administered to over 300 CC patients. The majority of apheresis platelet transfusions are to treat patients undergoing cancer therapy or organ and tissue transplants. The implementation of the pathogen reduction system and the collection of apheresis platelets in PAS further improve the safety of platelet transfusions for patients.

Wear Red Day brings NIH advice for the heart, from the heart



On Feb. 5, the National Heart, Lung, and Blood Institute held National Wear Red Day in the Clinical Center atrium to help raise awareness about heart disease in women. NIH staff members shared their heart-healthy advice and behaviors on a chalkboard to inspire women to care and make a change and inspire those around them to do the same. Above, Dr. Francis Collins, director of the NIH, begins to write “NIHers: Be an ambassador for health!” View a timelapse video: <https://youtu.be/gzblMJxO1cc>

Read more online! Scan the barcode or visit www.cc.nih.gov/about/news/newsletter.html



- By studying affected families at CC, NIH scientists discover genetic cause of rare allergy to vibration
- CC employees donate \$156,567 to CFC
- NHGRI & NIDDK seek study participants

Use a downloaded app on a smartphone or tablet to scan the Quick Response (QR) barcode. You will be directed to the CC News online.

Clinical Center News

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Vision for the Future: cell therapy for ocular disease

On Feb. 11, stem cell biologist Dr. Dennis O. Clegg presented a lecture titled "Vision for the Future: Cell Therapy for Ocular Disease," as part of the National Eye Institute Audacious Goals Initiative Seminar Series in Neuroregeneration.

Clegg, a professor at the University of California Santa Barbara in the Department of Molecular, Cellular, and Developmental Biology, stated that, in terms of cell therapy, "the eye may be a good place to start," as it has many advantages over other tissues. The eye is in some ways better suited for developing a therapy using cells, he went on to say. There are advanced surgical methods to get into the back of the eye, good ways to image the tissues in a non-invasive way and good end point parameters to tell if it's working.

Another major advantage, Clegg stressed, is that, "We don't think we'll need that many cells." For example, with a spinal cord injury, people may use billions of cells injected into the spinal cord, he stated. Unfortunately, they then disappear, and researchers can't follow them. With the eye, Clegg said, "We think we can get away with [only needing] 100,000 cells [to make a difference].... we think that's a real advantage."

Clegg emphasized the lack of treatments for ocular disease, things like retinitis pigmentosa, glaucoma and age-related macular degeneration, which affects roughly 11 million Americans.

"You probably know people who

are affected by these diseases," he said. "There is a real unmet medical need to develop therapies. We might be able to use stem cell therapies to treat these diseases."

Lightheartedly, Clegg pulled up a slide of potential presidential candidates in the 2016 election, and said that, like the current presidential campaign, there are a lot of different approaches and candidate options.

"It's worth it to go out there and try using cells to treat disease!"

Clegg, who is also the founder and co-director at the University's Center for Stem Cell Biology and Engineering and a co-principal investigator of the California Project to Cure Blindness, emphasized the importance of cultivation (how to make the cells), installation (how to deliver the cells) and preservation (how to bring about survival and function of transplant without the cells' growing out of control).

He has served on advisory boards for the California Institute for Regenerative Medicine and the NIH Center for Regenerative Medicine. The Audacious Goals Initiative lecture series explores topics relevant to regenerative neuroscience and medicine, with special emphasis on the visual system. It is a sustained effort by NEI to catalyze research toward new therapies for disorders that affect the retina and its connections to the brain.

Videocast: <http://go.usa.gov/cpmPJ>.
More details: <http://go.usa.gov/cpmmH>.

Professor presents on "The Digital Doctor," patient safety



In January, Dr. Robert Watcher visited the NIH and presented a Contemporary Clinical Medicine: Great Teachers Lecture on "The Digital Doctor: Hope, Hype, and Harm at the Dawn of Medicine's Computer Age." Watcher, a professor and Interim Chair in the Department of Medicine at the University of California, San Francisco, is a well-known leader in academic medicine for his contributions to patient safety and for his role in the development of the "hospitalist" concept in the U.S. Above, Watcher (center standing), met with the Accreditation Council for Graduate Medical Education Clinical Learning Environment Review Committee and Graduate Medical Education Committee faculty and fellows to discuss patient safety education for trainees. He also met Medical Research Scholars Program students, who are engaged in a year of mentored research in the NIH, went on patient care rounds and talked with senior Clinical Center leadership. Videocast: <http://go.usa.gov/cpyd3>

Upcoming Events

View lectures online: <http://videocast.nih.gov>

Pi Day at NIH

March 14, 2016

FAES Terrace and Lipsett Amphitheater
Celebration will increase awareness across the biomedical science community of the role that the quantitative sciences play in biomedical science. Events: PiCo Lightning Talks (10 a.m.); Poster/Demo Session and Networking (11 a.m.); Keynote address by Dr. Carlos Bustamante, Stanford's Department of Biomedical Data Science (1 p.m.); Workshop on Reproducible Research (2:30 p.m.); Pi Day Scholars Event for high school students and teachers (all day). Open to the public. Details: <https://datascience.nih.gov/PiDay2016>.

Clinical Center Grand Rounds Lecture: Combined Antiangiogenic Agents for Hepatocellular Carcinoma; Immune Based Therapies for Gastrointestinal Malignancies

March 23, 2016, 12 noon – 1:00 p.m.

Lipsett Amphitheater

Presented by Austin Duffy, MD, NCI and Tim F. Greten, MD, NCI.

Clinical Center Grand Rounds Lecture: Healthy Volunteer Human Influenza Challenge Model: A Key Piece of The Universal Vaccine Puzzle; Development and Pre-Clinical Evaluation of a Virus-like Particle (VLP) Universal Influenza Vaccine

March 30, 2016, 12 noon – 1:00 p.m.

Lipsett Amphitheater

Presented by Matthew J. Memoli, MD, NIAID and Jeffery K. Taubenberger, MD, NIAID.

NIH "Take Your Child to Work Day"

April 28, 2016, 9:00 a.m. - 4:00 p.m.

Bring children grades 1-12. Email questions to Take-Your-Child-To-Work@nih.gov.

NIH Management Intern Program seeks participants

The NIH Management Intern Program, a way to unlock a new career path, is recruiting April 4-8, 2016. This is a highly competitive, two-year career-development program for current NIH employees. Interns come from a variety of job backgrounds including both scientific and administrative fields. Upon completion of the program, interns transition into an administrative-management career in one of many areas throughout NIH. Eligible employees are invited to apply. Program FAQs and details about eligibility: <http://go.usa.gov/cfdgR>. Learn more by attending any of these information sessions, all of which are held from noon to 1 p.m.:

- March 22, Bldg. 31, 6C, Rm. 16
- March 23, Bldg. 45, NIH Training Center
- March 28, Rockledge 1, Suite 4000, RT1

Clinical Center staff go above and beyond during the blizzard



As winter comes to an end, the NIH Clinical Center won't soon forget weathering the blizzard in late January that brought upwards of 25 inches of snow. While federal offices were closed during and for a few days following the storm, over 250 clinical and support staff slept in Building 10 to provide safe and uninterrupted care for the over 100 inpatients who were hospitalized at the Clinical Center during the storm. In addition to committed clinical and support staff from the Clinical Center and institutes and centers, the partnership of central service colleagues from the NIH Office of Research Services, Office of Research Facilities and Office of Financial Management allowed the Clinical Center to maintain continuous operations. The ACRF cafeteria, patient shuttles, campus utilities, snow removal and patient cashier services provided ongoing services. Staff issued 75 inflatable beds and opened the contingency station to provide cots for employees spending the night. Additionally, colleagues from The Children's Inn at NIH provided sleeping accommodations for staff.



Top photos, from left to right: pediatric patient Lucy Wiese received a surprise when a nurse brought her a bucket of snow to play with inside (go online to view the extensive press coverage of Lucy's snowy fun thanks to nurse Alex Classen including the Washington Post); snowy north entrance to the Clinical Center; food services staff Debbie Shipp, Michelle Taylor, Andre Peters and Anna Boyer; and pediatric nurses. Bottom photos, from left to right: voucher of office staff member Sam Goldsmith; Chauncey Buford, housekeeping supervisor; and Office of Research Facilities snow removal team.

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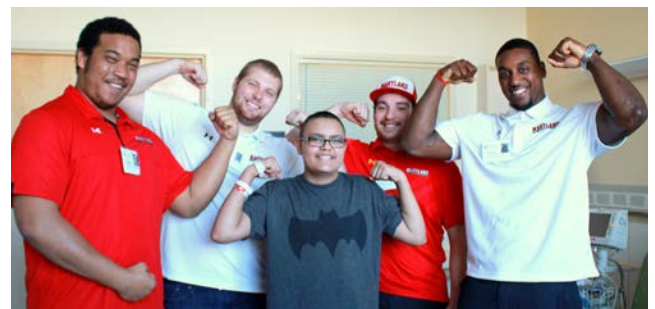
Other rare diseases are studied with the help of the Undiagnosed Diseases Network (UDN), a research study funded by the NIH that brings together clinical and research experts from across the U.S. to solve the most challenging medical mysteries using advanced technologies.

"Millions of Americans who deal daily with rare disorders and thousands of physicians and scientists have dedicated their lives toward improving the lives of these individuals," said Dr. William Gahl, director of the UDN. "On Rare Disease Day, we remember all of these people and thank them for the inspiration they provide us and for their contributions to the future of medicine."

Beatrice Bowie, a NIH patient who has a rare inherited blood disorder called Sickle

Cell Disease, also addressed attendees of Rare Disease Day.

"It has been a roller-coaster ride for those of us afflicted with sickle cell disease, whose lives are hanging in the balance. Fortunately, today there is much more widely disseminated knowledge of sickle cell disease, thanks to the efforts of researchers and care providers here at NIH and elsewhere," she said. "Sickle cell disease is now more commonly recognized, properly diagnosed and treated than before. But due to the mysterious nature of sickle cell disease, there is still so much to be learned to help us, the patients, and to finally find a universal cure for this disease."



Uplifting Athletes visit Darius Gallegos, a pediatric patient, on RDD.

The day also included a visit from Uplifting Athletes, a national nonprofit organization aligning college football with rare diseases and raising them as a national priority through outreach, research, education, and advocacy. Athletes from the University of Maryland visited pediatric patients.