

CAR T-Cell Therapy Moves Closer to FDA Approval

BY ERIC BOCK

Currently, there are few options for patients with relapsed and treatment-resistant blood cancers. A new approach to immunotherapy might give those patients another avenue, said Dr. Carl June at a recent NIH Director’s Lecture in Masur Auditorium.

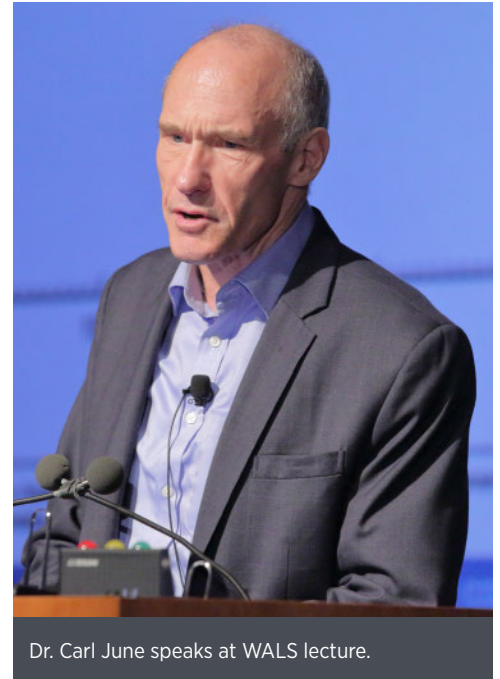
The approach, called chimeric antigen receptor (CAR) T-cell therapy, is expected to be approved next year by the Food and Drug Administration for the treatment of blood cancers such as leukemia and lymphomas, said June, a professor of immunotherapy at the Perelman School of Medicine at the University of Pennsylvania.

The CAR T cells nearing approval are genetically engineered to seek out and destroy leukemia cells. There are several types of leukemia, including chronic lymphocytic leukemia (CLL) and acute lymphoblastic leukemia (ALL).

To prepare CARs for therapy, white blood cells called T cells are taken from a patient. Then they are “genetically modified in the lab and returned to patients, usually after some sort of conditioning, like chemotherapy,” he said. “CARs are synthetic molecules. They don’t exist naturally.”

The gene for a receptor that binds to the CD19 antigen is added to T cells. CD19 is a protein found on the surface of all B cells, a type of white blood cell. June called CD19 a “dream target” because it’s expressed on the surface of every B-cell malignancy, including CLL and ALL. Although healthy B cells

SEE JUNE, PAGE 4



Dr. Carl June speaks at WALS lecture.



Recent forum focuses on partnerships. See p. 12.

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Former HHS secretary Dr. Louis Sullivan

Former HHS Secretary Devotes Life to Diversity

BY DANA TALESNIK

Dr. Louis Wade Sullivan never forgot his roots. Throughout his distinguished career, the former HHS secretary, hematologist, professor and medical school president emeritus made great strides in health policy, medicine and education. Guided by his upbringing, Sullivan also made it his lifelong mission to bring inclusive diversity into our health system.

SEE SULLIVAN, PAGE 6

Five Questions with New NICHD Director

Dr. Diana Bianchi joined NIH as director of the National Institute of Child Health and Human Development on Nov. 7. She comes to NIH from the Floating Hospital for Children and Tufts Medical Center in Boston, where she served as founding executive director of the Mother Infant Research Institute and vice chair for pediatric research.

She recently answered some questions about her goals as she takes on this new role.

Why did you want to become NICHD director?

I always have been passionate about advancing knowledge to help people. Although I really enjoy taking care of patients and families, I realized early on that I could have a much bigger impact on health care through research. I am fortunate enough to have seen one of my basic research projects advance into medical practice. That project—isolating fetal cells from the blood

SEE BIANCHI, PAGE 8

Now Through Dec. 12

NIH Leave Bank Holds Open Enrollment

Fall open enrollment for the NIH Leave Bank is available now through Dec. 12. The membership period will begin on Jan. 8, 2017.

The Leave Bank is a pooled bank of donated annual and restored leave available to eligible members. It acts like insurance for your paycheck and amounts to paid leave for members who have exhausted all of their leave and are affected by a personal or family medical emergency. Said one grateful Leave Bank recipient, "This program was wonderful; I don't know what I would have done without it. I think this program is a great benefit to new employees and to any employee who has not accrued sufficient time for needed leave. I recommend joining to everyone."

The Leave Bank differs from the Voluntary Leave Transfer Program (VLTP) in that the bank is a depository of leave and leave is distributed to members who are approved to be leave recipients.

The VLTP, on the other hand, requires a direct donation from a donor to a recipient. An advantage of the Leave Bank is that eligible members may receive leave from the bank to cover time out of the office without awaiting donations from co-workers.

To elect to become a Leave Bank member, access the Integrated Time and Attendance System during open enrollment and sign up under "Leave Bank Membership."

If you are currently a 2016 Leave Bank member, your membership will automatically continue into 2017, unless you take action in ITAS during open enrollment to opt-out. The membership contribution is one pay period's worth of annual leave accrual. The membership contribution will automatically be waived if you lack sufficient leave to make the membership contribution.

A list of upcoming Leave Bank events may be viewed at <http://hr.od.nih.gov/benefits/leave/vlbp/important.htm>. The events are free and no registration is required. Questions may be directed to the NIH Leave Bank office at (301) 443-8393 or LeaveBank@od.nih.gov.

OALM Makes Gift to Children's Inn

During a recent visit to the Children's Inn at NIH, Diane Frasier, director of the Office of Acquisition and Logistics Management, presented the inn with a check for \$400 from raffle proceeds at a recent OALM function.

Earlier this year, Jennie Lucca, CEO of the inn, spoke at the annual OALM employee awards ceremony. Her talk identified the many ways the inn offers support to the families of children participating in clinical trials, treatment and research at the Clinical Center. Several tours were set up for OALM



On hand for the check presentation are OALM employees (from l) George Martinez, Sharmaine Fagan-Kerr, Keivon Avents, Colby Dawson, Melissa McKerrow, Patricia Kirby, Diane Frasier, Karen Thomas and Children's Inn staff Anna Tate and Julie Herman.

employees to see the inn and learn of its activities, including the work being done to make children and their families comfortable and provide them with a home away from home. The experience

reminded OALM staff of the importance of their contributions to the NIH mission, including helping youngsters aided by the inn.



Cancer Survivor-Congressman Visits NIH

Rep. Mark DeSaulnier (D-CA) visited recently to learn more about NIH, with a special interest in cancer research. He was diagnosed with chronic lymphocytic leukemia (CLL) last year and successfully completed his treatment. His cancer experience sparked his interest in NIH and NCI. He was met by NIH principal deputy director Dr. Lawrence Tabak (right, l) and Clinical Center director Dr. John Gallin. During a 2-hour tour, DeSaulnier learned more about the latest treatment for CLL. He toured NCI's molecular imaging clinic and completed his visit in the company of (above, from l) Dr. Adrian Wiestner, senior investigator, Laboratory of Lymphoid Malignancies, NHLBI; Dr. Inye Ahn, a clinical fellow in the lab; Ethan Van Ness, DeSaulnier's legislative assistant; and Dr. Neal Young, chief, Hematology Branch, NHLBI.



PHOTOS: ERNIE BRANSON

Journalists Learn Fundamentals of Cancer Research

BY ROBERT PINES

“I don’t want you to leave this room as cynics, but as skeptics,” said NCI’s Dr. Barry Kramer while presenting to a room of 14 journalists about cancer screening and guidelines. Participating as part of the first-ever National Cancer Reporting Fellowships, the individuals to whom Kramer spoke represented the lucky few selected to come to the Clinical Center for a 4-day workshop aimed at improving how the media reports on cancer research. The program ran Oct. 24-27.

The National Cancer Reporting Fellowships, a joint effort of NCI and the Association of Health Care Journalists (AHCJ), was developed as an opportunity for journalists to interact directly with the people whose news they report. The fellows represented a variety of affiliations, from independent freelancers to the *Washington Post* health section.

Over the course of the week, they heard from more than 20 experts covering a variety of topics. Sessions were on everything from the Cancer Moonshot and immunotherapy to disparities and genomics.

The program also featured a tour of several Clinical Center labs led by Caryn Steakley, deputy clinical director of the NCI Center for Cancer Research. During this time, the journalists met with Dr. James Gulley about checkpoint inhibitors for vaccines, Dr. Christian Hinrichs about bench-to-bedside research and Dr. Peter Choyke about advanced imaging techniques.



Caryn Steakley of NCI speaks to the fellows during a tour of the Clinical Center.



Fellows of the National Cancer Reporting Fellowships pose with staff from the NCI Office of Communications and Public Liaison.

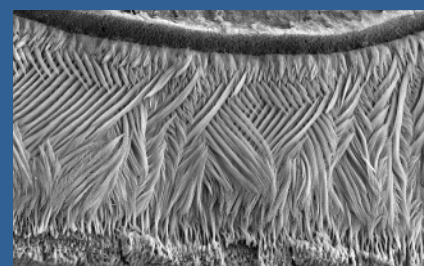
PHOTOS: MARIANA EBERLE-BLAYLOCK

One special session included advocate Jamie Goldfarb, a melanoma survivor who led a discussion on story-building and the proper ways to interview patients for an article. Highlighting the need for empathy and understanding, Goldfarb provided personal anecdotes about her past interactions—positive and negative—with journalists.

“Over the last few days, this fellowship gave me the opportunity to immerse myself in research and data about cancer prevention, disparities and treatment,” said fellow Laura Santhanam of *PBS NewsHour*. “Cancer is one of the top causes of death among Americans, and as a journalist, I must use nuance to report on innovation so the public understands where science and medicine stand in the quest for a cure and what ground is left to cover.”

The event concluded with a session on the future of health care journalism by Len Bruzzese, AHCJ executive director, and Dr. Robert Logan of the National Library of Medicine. “Reporters are hungry for

this kind of detailed topic training,” said Bruzzese. “We’re happy NCI was willing to host the fellowship week and make so many experts available to speak.” **R**



ON THE COVER: Lattice pattern formed by enamel rods protecting a tooth's surface. 2016 CFC Beauty of Science 2nd place entry by NIAMS

IMAGE: OLIVIER DUVERGER & MARIA MORASSO, NIAMS

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June

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are killed during therapy as well, they can be replaced.

Serious side effects of the treatment include cytokine release syndrome (the so-called “cytokine storm”) and tumor lysis syndrome. Symptoms of cytokine release syndrome are high fever and drop in blood pressure. Tumor lysis syndrome is a complication of treating a fast-growing cancer. When cancer cells are destroyed, they break apart and release their contents into the bloodstream. Left untreated, it can cause kidney failure and irregular heart rhythms. For both syndromes, the severity depends on the cancer’s size.

June first began genetically modifying T cells in the mid-1990s, when he was studying HIV/AIDS. Then, he and his colleague Dr. Bruce Levine thought they might be able to modify T cells to kill HIV, the virus that causes AIDS. He gave patients infusions of CAR T cells every 30 to 60 days. Although the modified cells didn’t have an antiviral effect, they proved safe.

“We found in 2012 that, in fact, 36 out of 39 patients we treated still had CARs—out past a decade,” he noted. “We think they persist longer than natural T cells.”

★ ★ ★

“CARs are synthetic molecules. They don’t exist naturally.”

-DR. CARL JUNE

★ ★ ★

He and colleague Dr. David Porter first treated 14 patients with incurable CLL in 2010. That study had an overall response rate of 57 percent. Four patients had a complete response and four more had a partial response. Six patients had no response. Of the patients who had complete responses, “we’ve had no relapses. It’s effective in a subset of patients,” June noted.

In some patients who did relapse, the CAR T cells didn’t proliferate “for reasons we don’t understand,” he said. In other cases, the cells could not find their target because the tumor mutated. If CD19 is no longer on the surface of a B cell, the CAR T cell can no longer find the cancer.

Clinical trials are under way at Penn and other institutions to develop and test CARs



June first began genetically modifying T cells in the mid-1990s, when he was studying HIV/AIDS.

PHOTOS: ERNIE BRANSON

that seek and destroy multiple targets.

Between 2012 and 2016, June and his team treated 60 pediatric and young adult patients with ALL; 93 percent had a complete response.

“The results we’ve seen in ALL have been more dramatic than CLL,” he said.

As of May 2016, June enrolled 368 patients in CAR T-cell clinical trials. Not one had a “genotoxic event,” where damage to cells can lead to mutations or deletions that can later cause cancer.

June expects the FDA will approve CARs for CD19-specific malignancies such as leukemia and lymphoma in 2017. Even though this therapy is nearing approval, there are significant challenges that must be addressed, he cautioned.

At first, CAR T-cell therapy will only be available at high-end “quaternary” cancer centers. Physicians practicing at community hospitals won’t be sufficiently trained in immunotherapy, so they can’t offer it to patients. How T cells are produced will also limit the availability of treatments.

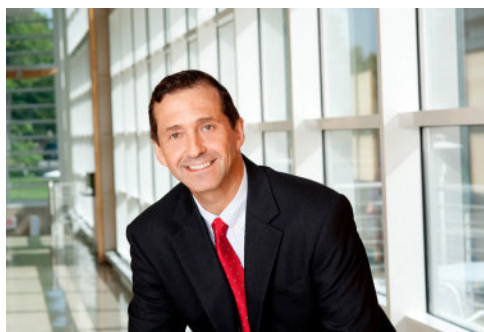
“We need robotic and fully automatic cell culture,” said June. “We have a system that still depends on academic-based manufacturing systems—basically requiring highly trained personnel.”

Since CAR T-cell therapy is personalized, new T cells must be grown from a patient’s own cells. June said it isn’t clear yet whether cord blood or T cells from a healthy donor can be used.

Unfortunately, he has not had the same success in patients with solid tumors because “most tumors have targets on the surface essential for other normal cells.” Complex sugars found only on the surface of tumors, however, might be alternative targets for therapy.

Currently, June’s lab is conducting clinical trials in patients with multiple myeloma, a cancer of plasma cells, and in patients with pancreatic cancer to test whether certain glycans can serve as effective targets.

June concluded by noting that when he first started treating leukemia patients in 2010, there were only three cancer centers with open CAR T-cell trials. Now, there are 110 trials open in the U.S and around the world, evidence for the global emergence of a new therapy. **R**



Mascola To Give NIAID Kinyoun Lecture Dec. 8

Dr. John Mascola, director of the Dale and Betty Bumpers Vaccine Research Center at NIAID, will deliver the 2016 Joseph J. Kinyoun Memorial Lecture on Thursday, Dec. 8 at 3 p.m. in Lipsett Amphitheater, Bldg. 10.

His talk, titled "Structure-Based Vaccine Design and B-cell Ontogeny in the Modern Era of Vaccinology," will include an overview of the challenges facing the development of effective vaccines against viruses, including HIV, respiratory syncytial virus and influenza virus. Mascola will describe how researchers can use structural information about viral proteins and antiviral antibodies to design new vaccines. He also will discuss how an understanding of antibody evolution, termed B-cell ontogeny, can inform approaches to improving vaccines.

Mascola, an internationally recognized expert on HIV immunology and vaccine development, was appointed VRC director in October 2013. In this role, he oversees a basic and translational research program aimed at developing and testing candidate vaccines against HIV, influenza virus, Zika virus and other infectious agents that cause diseases of global importance. He also serves as chief of the Virology Laboratory and chief of the humoral immunology section at the VRC, where his research focuses on structure-based design and testing of novel vaccines for HIV/AIDS and influenza, optimization of immune responses and identification of correlates of protection.

Mascola is a fellow of the American College of Physicians and has been elected to the American Society of Clinical Investigation, the Association of American Physicians and fellowship in the American Academy for Microbiology.

Mascola obtained his medical degree in 1985 and completed training in internal medicine and infectious diseases, followed by a fellowship in retrovirology. He joined the VRC as deputy director in 2000. Prior to joining the VRC, he was head of HIV prevention research in the division of retrovirology at Walter Reed Army Institute of Research.

Since 1979, NIAID has hosted an annual public lecture in honor of Dr. Joseph J. Kinyoun, who in 1887 founded the Laboratory of Hygiene, the forerunner of NIH, and launched a new era of scientific study of infectious diseases.

KUDOS

Four from NIH Named to National Academy of Medicine

Four NIH scientists are among 70 new members named to the National Academy of Medicine. Election to the academy is considered one of the highest honors in the fields of health and medicine and recognizes individuals who have demonstrated outstanding professional achievement and commitment to service.

"These newly elected members are outstanding professionals who care deeply about advancing health and health care in the U.S. and globally," said NAM president Dr. Victor Dzau. "Their expertise will help our organization address pressing health challenges and improve health, science and medicine for the benefit of us all. It is my privilege to welcome these accomplished individuals to the National Academy of Medicine."

Elected from NIH:

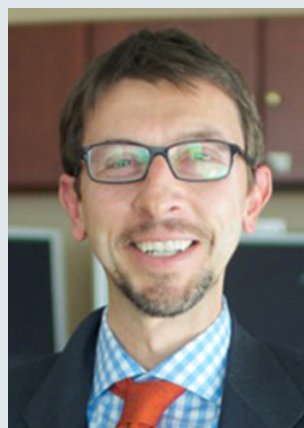
Dr. Karen Faith Berman is senior investigator and chief, section on integrative neuroimaging and the Clinical Brain Disorders Branch, National Institute of Mental Health Intramural Research Program.

Dr. Leslie Glenn Biesecker is senior investigator and chief of the Medical Genomics and Metabolic Genetics Branch, National Human Genome Research Institute.

Dr. Antonello Bonci is senior investigator, Cellular Neurobiology Research Branch, synaptic plasticity section, and scientific director, National Institute on Drug Abuse.

Dr. T. Jake Liang is chief of the Liver Diseases Branch and deputy director of translational research, National Institute of Diabetes and Digestive and Kidney Diseases.

Established originally as the Institute of Medicine in 1970 by the National Academy of Sciences, the National Academy of Medicine addresses critical issues in health, science, medicine and related policy and inspires positive actions across sectors.



New NIH NAM members include (clockwise, from top l) Dr. Karen Berman, Dr. Leslie Biesecker, Dr. T. Jake Liang and Dr. Antonello Bonci.



Sullivan credits parents, mentors and unexpected opportunities for his success.

PHOTOS: LISA HELFERT

Sullivan

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For Sullivan, life was an uphill battle, growing up a black man in the racially segregated rural south. Fortunately, he had a series of mentors and unexpected opportunities that set him on a path to success. Sullivan eloquently recounted his experiences—also told in his recently published memoir *Breaking Ground: My Life in Medicine*—at an NLM History of Medicine lecture Oct. 4 in Lipsett Amphitheater. In the casual town hall setting, the sizeable audience included quite a few fellow graduates of Sullivan's alma mater, Morehouse College.

Sullivan's story begins in Georgia in the 1930s. His father established the first black funeral home in rural Blakely. His mother commuted great distances to schools that would employ black teachers. Sullivan was fortunate to attend better schools in urban areas. His parents were his earliest role models, instilling in him a passion for education.

When it was time to see the doctor, rather than suffer the humiliation of the separate waiting room at the white doctors' offices, Sullivan's parents took him to see Dr. Joseph Griffin, the only black doctor in southwest Georgia, some 40 miles away.

"Dr. Griffin so impressed me that I decided, by age 5, I wanted to be a doctor," said Sullivan.

He later found another role model in Dr. Benjamin Elijah Mays while studying at Morehouse College. "He was [always] telling us that in a segregated society, there may be barriers because of prejudice, but we should not have a barrier because of lack of preparation."

Sullivan graduated from Morehouse in 1954, the year the Brown vs. Board of Education ruling declared school segregation unconstitutional. He then went to study at Boston University School of Medicine, his first experience living and working in a non-segre-

gated environment. It was a positive one, he said, though he was the only black student in his class and 1 of only 3 at the whole medical school, at the time.

For his post-graduate training in 1958, Sullivan was stunned to get accepted to New York Hospital-Cornell Medical Center, which up until then had never had a black intern. The chair of medicine, a burly white physician from Tennessee, personally kept protective watch over him. "Then I saw my own prejudices," Sullivan said. "I had made a judgment of him when I met him, with that southern accent."

One of the most gratifying experiences of his professional career, he said, was presenting a research paper at the American Society for Clinical Investigation's national meeting in 1963. His study linked heavy alcohol consumption with suppressed red blood cell production.

By 1973, Sullivan was a settled professor of medicine at Boston University, and married with three children, when Morehouse College recruited him to help establish a medical school in 1975. The Morehouse School of Medicine would become the only 4-year U.S. medical school organized for black students in the 20th century, joining Howard University School of Medicine and Meharry Medical College, which had been founded in the 19th century.

"The civil rights movement of the '50s, '60s and '70s opened the eyes of the country to just how severe the conditions were for

blacks in the south," said Sullivan. "So the country responded to that in a way that, when I started at Morehouse in 1975, not only were black physicians in Georgia supporting this medical school...but also the white physicians; they went with me when I had meetings with the governor or state legislature."

In 1977, Sullivan was instrumental in founding the Association of Minority Health Professions Schools. "There was really an upwelling of support for activities around the country...to try and address the shortage of black and other minority physicians."

Today, minority physicians (blacks, Latinos and Native Americans combined) represent just 8 percent of our nation's doctors, yet these minorities represent 32 percent of the nation's population. Meanwhile, said Sullivan, a 2011 *Science* paper reported that first-time white investigators applying for RO1 grants from NIH

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"From my perspective, one of the most important measures of an institution is what do its graduates do. Do they change the world?"

-DR. LOUIS SULLIVAN

• • •

had nearly double the success rate of equally qualified black investigators.

"We've made significant progress over the years in increasing the diversity of the health workforce," said Sullivan, "but that progress has been far less than what we had expected."

Advocacy is even more urgent today, he said, because "now we are more polarized as a society." He cited the need for job training, career counseling and more scholarships for minorities.

"We need to strengthen K-12 education," he said. "So many youngsters in inner cities don't get the kind of academic exposure and counseling that they should. They don't have role models."

Another obstacle is student indebtedness. "We are not seeing students from low-income backgrounds coming into the health professions," Sullivan said. "Those who do come through end up with significant debt... that drives them into high-paying specialties or into affluent communities."

In 1989, President George H.W. Bush appointed Sullivan secretary of HHS, where he would develop several initiatives to increase racial, ethnic and gender diversity. He oversaw the formation of NIH's Office of Minority Health, later to become NIMHD. He also oversaw the appointment of NIH's first female director, the late Dr. Bernadine Healy, as well as the first female and Hispanic surgeon general and first female HHS chief of staff.

One important feat under his leadership was getting new labeling on packaged foods to help Americans make healthier choices. Sullivan recounted the arduous process of negotiating the FDA food labeling, which the Department of Agriculture opposed over concerns it would hurt the sales of dairy farmers, the cattle industry and other constituents. When President Bush said he'd arbitrate, Sullivan came equipped with a McDonald's placemat that featured a nutrition label to illustrate his point. Why would a fast-food giant list a food label if it would hurt sales? "Then I knew I had him," Sullivan said. Within days, the President accepted his recommendation.

After his HHS term, Sullivan returned to Morehouse School of Medicine as its president. The medical school's notable graduates include former surgeon general Dr. Regina Benjamin, Meharry College president Dr. Wayne Riley and Dr. Sam Gulube, a South African doctor who returned to his country to found its first nationwide blood banking system.

"From my perspective, one of the most important measures of an institution is what do its graduates do," said Sullivan. "Do they change the world?" Sullivan is yet another distinguished Morehouse College alumnus, continuing that tradition. **R**

Next Protocol Navigation Lecture, Nov. 21 in Lipsett

The IRP Protocol Navigation Training Program Seminar Series will host a lecture on Monday, Nov. 21 from 11 a.m. to noon in Lipsett Amphitheater, Bldg. 10. The program is a trans-NIH effort to develop resources and tools and to provide training for intramural staff and contractors involved in protocol development, writing, coordination and management. Bruce Burnett and Val Bonham from the NIH Office of Research Support and Compliance will present "ORSC-The First 100 Days." For more information contact Marcia Vital, (301) 451-9437, vitalm@mail.nih.gov.



Director of ceremonies Dr. Afaf Meleis (l) and NINR director Dr. Patricia Grady

NINR Marks Anniversary with Symposium

NINR recently hosted the scientific symposium "Advancing Science, Improving Lives: A Window to the Future" at the Washington Hilton in Washington, D.C. NINR director Dr. Patricia Grady opened the event—one of the concluding activities in NINR's year-long 30th-anniversary commemoration—and Dr. Afaf Meleis, professor of nursing and sociology and former dean of nursing at the University of Pennsylvania School of Nursing, served as master of ceremonies. More than 500 scientists, health care professionals and members of the public discussed current and future research in nursing science.

The symposium featured scientific speakers and included panel discussions on the topics of sleep and omics science. Dr. David Dinges of the University of Pennsylvania Perelman School of Medicine moderated the symptom science panel on sleep, joined by Dr. Nancy Redeker of Yale School of Nursing and Dr. Terri Weaver of the University of Illinois at Chicago College of Nursing.

The group discussed a transformation in our

understanding of the role of sleep on human health, including the effect of sleep on the outcomes of chronic conditions, and vice versa. Panelists noted that there are effective treatments for sleep disorders and that continued research will help ensure that those with chronic disease receive these treatments when they need them.

Dr. Yvette Conley of the University of Pittsburgh School of Nursing moderated the omic science panel on precision health. Panelists included Dr. Bernice Coleman of Cedars-Sinai Medical Center and Dr. Jessica Gill of NINR's Division of Intramural Research.

Panelists noted the great strides made in omic sciences, with the first map of the human genome taking over 10 years to complete, compared with the mere days this process requires now. Because of such advances, it's now practical to use omics sciences to identify diagnostic and prognostic biomarkers for disease.

NINR released its new strategic plan at the symposium, *Advancing Science, Improving Lives: A Vision for Nursing Science*, which details the institute's priorities for the conduct and support of future nursing science. The plan, which incorporates feedback from the recent NINR Innovative Questions Initiative, highlights four areas—symptom science, wellness, self-management and end-of-life and palliative care—as well as two cross-cutting areas of emphasis: promoting innovation and developing 21st century nurse scientists. The plan is available at www.ninr.nih.gov/strategicplan.

In closing, Grady noted that nursing science, ultimately, is about people. She encouraged those in attendance to work side by side with representatives of the communities who will benefit from their research. "Be active listeners," she advised. "Their insights, knowledge and perspectives will help ensure the success of your research by informing, guiding and shaping its design and implementation."

NIH Issues New Energy-Saving Freezer Policy

With energy conservation in mind, NIH has set a new policy on management of ultra-low temperature (ULT) freezers, which provide storage for preserving research-related materials. Well-maintained, energy-efficient ULT freezers can play a significant role in reducing NIH cold storage costs and preserving medical research funds.

NIH's new policy requires that institutes and centers purchase energy-efficient ULT freezers when acquiring new units and perform regular preventive maintenance on all ULT freezers in NIH facilities. The Division of Scientific Equipment and Instrumentation Services can help ICs meet both of these requirements. The policy also encourages ICs to use the division's new equipment sales and rental program.

"DSEIS is really a one-stop shop for lab equipment needs, including ULT freezers," notes Anju Vergheese, chief of the Scientific Equipment Rental & Sales Branch. "We can reduce the administrative burden, save staff time and negotiate low costs. We also offer budget-friendly, rent-to-buy agreements that ensure ICs comply with the new NIH policy."

For policy details, see <https://outreach.ors.nih.gov/2016/10/27/new-nih-freezer-policy-are-you-in-compliance/>. For more information, visit <http://dseis.od.nih.gov>, contact Vergheese (sales and rental agreements) at (301) 496-9748, or Jerry Tyus (maintenance and repair) at (301) 451-1753.



Bianchi

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of pregnant women—is now a noninvasive test for screening placental DNA that has been used by millions of women. While it is gratifying to see the clinical benefit of this test, I felt I could have an even greater influence on the lives of children and families by joining NIH and helping to shape a global research agenda.

NICHD's broad mission offers a range of opportunities for multidisciplinary and longitudinal approaches to the research portfolio. I believe that my clinical training in pediatrics, medical genetics (including care of people with physical, intellectual and developmental disabilities) and neonatology, as well as my research expertise in reproductive genetics and genomics and fetal care, align well with the mission.

My concerns about federal research funding and the loss of talented people from the academic pipeline also played a role in my decision to join NICHHD. I am dedicated to the training and mentoring of students,



“The joy of science truly comes from analyzing the data and realizing that you have found something unexpected, but potentially much more interesting.”

—DR. DIANA BIANCHI



residents, postdoctoral fellows and faculty and I wanted the opportunity to work with leaders at NIH, as well as with those in the pediatric, obstetric, gynecologic and rehabilitation communities, to improve prospects for academic investigators looking to advance their careers and make a difference.

Another important factor in my decision was the lure of public service. As the child of immigrants who came to the United States before and after World War II, I wanted to give something back to the country that sheltered my family and provided us with economic and educational opportunities. A lot of that inspiration comes from my grandmother, who after escaping the Nazi invasion of Austria,

settled in New York City and immediately volunteered to drive an ambulance. I want to continue that legacy of service.

What are your goals for NICHHD?

Because science evolves so rapidly, we must be flexible enough to reorder our priorities in response to scientific opportunities and emerging public health needs.

For example, today the congenital defects associated with Zika virus infection are a significant and immediate concern. Researchers funded by NICHHD can and should contribute to an understanding of the basic mechanisms that result in microcephaly and more subtle fetal anomalies, as well as those that affect fertility and sexual transmission of the virus in adults. Furthermore, NICHHD researchers can contribute to better and more accurate ways of detecting the virus and preventing its spread.

Another priority is to increase representation in clinical research of the populations that NICHHD serves. I have had many pregnant women participate in my research and I can affirm that they are very interested in their bodies and their child's development. Expectant mothers are likely to be enthusiastic participants in broader clinical research and children can benefit enormously from the knowledge generated by clinical studies. Lastly, we need to include people with disabilities as participants in research not only to obtain important information about an understudied group of people, but also to send a powerful message of inclusion.

Among other goals, I aim to enhance NICHHD's focus on basic and translational research in reproductive and neonatal genomics, which can help define the roles of genes in infertility, reproductive disorders and birth defects. I am very excited about using big data to develop innovative approaches and solutions to problems in NICHHD's research areas. I also will continue to support NICHHD's ongoing initiatives on medical rehabilitation and on the role of the placenta in maternal and child health,



Bianchi has a lab at NHGRI, to continue her scientific pursuits.

as well as efforts to improve our payline and align research funding with evolving institute priorities.

Being new to NIH, I plan to listen and learn from others. In doing so, I expect to gain a better understanding of how NICHHD-funded scientists collaborate inside and outside of the institute. I want to help build bridges in pursuit of common research goals. I also would like to increase patient and family engagement in NICHHD's activities, including study design and communications.

You have a lab at NIH. What does your group study?

My laboratory at the National Human Genome Research Institute studies prenatal genomics and fetal therapy. Since 2011, more than 2 million screening tests for fetal chromosome abnormalities have been performed using genomic analysis of cell-free DNA that originates from the placenta and circulates in the blood of pregnant women. This technology has profoundly improved prenatal care because it is more accurate and less invasive than prior screening tests. Also, because genomic analysis is far more sensitive than prior tests, it sometimes detects unexpected disease, such as maternal cancer.

My laboratory is particularly interested in the underlying biological mechanisms of unusual or secondary findings that result from whole genome sequencing. One of our goals is to apply DNA sequencing technology to the development of novel biomarkers that identify risks for fetal and placental disease.

In addition, my research group studies gene expression in developing fetuses with trisomy 21, or Down syndrome. For the past 6 years, we have focused on identifying

potential drug candidates that help improve fetal brain development following a prenatal diagnosis of Down syndrome. We are analyzing the effects of therapy in cells and animal models. Several people from my laboratory at Tufts have joined me at NIH and we all look forward to establishing productive collaborations with other investigators here.

What advice do you have for early stage researchers?

First, find a mentor who is willing to support your professional development and to put you on the right path to an independent career.

Second, find a program or department that has transparency and a demonstrated track record of protecting time for research.

Third, learn how to communicate your scientific findings orally and in writing. Practice your “elevator speech,” because you never know when you might need it.

Fourth, be persistent and develop a thick skin. Your papers will be rejected and your grant applications won’t get funded, but if you learn from the experience, you will improve.

Fifth, find great collaborators who you can trust and with whom you have good chemistry. No one is an expert on everything; working with others results in unexpected discoveries and, frankly, team science is a lot more fun.

Finally, always remember that the data are the data. Don’t ever try to force the data into a hypothesis that initially may have been incorrect. The joy of science truly comes from analyzing the data and realizing that you have found something unexpected, but potentially much more interesting.

What do you like to do in your free time?

I have a wonderful family that includes a husband and two adult sons who still enjoy going on vacation with us. We love to travel; over the past few years, we have concentrated on Central and South America, with trips to Panama, Peru, Chile (including Easter Island) and Argentina. We also like to hike, and with a name like “Bianchi” it wouldn’t surprise many to know that I am an avid cyclist! I also appreciate the visual arts not only for their intrinsic beauty, but also for their ability to enhance the creative process. I am excited to explore the museums in the Washington, D.C., area. **R**

New Method for Aortic Valve Replacement Proves Successful in High-Risk Patients

Researchers at NIH have developed a new, less invasive way to perform transcatheter aortic valve replacement (TAVR), a procedure widely used to treat aortic valve stenosis, a lethal heart condition. The new approach, called transcaval access, will make TAVR more available to high-risk patients, especially women, whose femoral arteries are too small or diseased to withstand the standard procedure. The *Journal of the American College of Cardiology* published the findings.

Aortic valve stenosis involves the narrowing of the heart’s aortic valve, which reduces blood flow through the heart. For about 85 percent of patients with this condition, doctors typically perform TAVR through the femoral artery in the leg. But for the other 15 percent, doctors must find a different access route. The most common alternative routes are through the chest, which requires surgery and are associated with significantly more complications.

Transcaval access, which can be performed in awake patients, involves electrifying a small wire so that it crosses between neighboring blood vessels in the abdomen. The technique calls for making large holes in both the abdominal aorta and the inferior vena cava, which physicians previously considered dangerous because of the risk of fatal bleeding.

The new method was developed by researchers at NHLBI and tested in a trial on 100 patients at 20 hospitals across the United States. Researchers said it proved successful in 99 of the patients.

Gene Therapy Shows Promise for Niemann-Pick Disease Type C1

For the first time, NIH researchers have demonstrated in mice that gene therapy may be the best method for correcting the single faulty gene that causes Niemann-Pick disease, type C1 (NPC1). The gene therapy involved inserting a functional copy of the NPC1 gene into mice with the disease; the treated animals were then found to have less severe NPC1 symptoms. The study, led by researchers at NHGRI and NICHD, was published Oct. 26 in the journal *Human Molecular Genetics*.

Niemann-Pick disease is a rare and fatal disorder of the central nervous system (the brain and spinal cord) that has no cure. The disease occurs when a faulty housekeeping gene fails to remove cell waste, like lipids and cholesterol. The accumulation of waste in the spleen, liver and brain causes progressive deterioration in intellectual and motor functions. It also shortens patients’ lives, as people with Niemann-Pick disease typically die in their teens.

The researchers’ goal was to correct the faulty NPC1 gene in as many cells and organs as possible, with a strong focus on the brain. To do this, they

used a non-disease-causing virus called the adeno-associated virus serotype 9 to transfer functioning NPC1 to the cells. The AAV9 containing a functioning NPC1 gene successfully crossed the blood-brain barrier, reaching cells in the brain and elsewhere. Once inside cells, the normal NPC1 gene was then able to make the functional NPC1 protein to correct the cell defects.

With a single injection, mice showed improvements in motor coordination, weight gain and longevity compared to those without this gene therapy.

“Our work in NPC1 mice may help lead to human clinical trials and eventually FDA approval for gene therapy as a treatment for NPC1 disease,” said Dr. Charles Venditti, senior investigator in NHGRI’s Medical Genomics and Metabolic Genetics Branch. “For NPC1 patients, gene therapy could halt progression of the disease, improve the quality of their lives and, hopefully, increase the patient’s life span.”

Skin Patch to Treat Peanut Allergy Shows Benefit in Children

A wearable patch that delivers small amounts of peanut protein through the skin shows promise for treating children and young adults with peanut allergy, with greater benefits for younger children, according to 1-year results from an ongoing clinical trial. The treatment, called epicutaneous immunotherapy or EPIT, was safe and well-tolerated, and nearly all participants used the skin patch daily as directed.

The ongoing trial is sponsored by NIAID. One-year outcomes were published online on Oct. 26 in the *Journal of Allergy and Clinical Immunology*.

“To avoid potentially life-threatening allergic reactions, people with peanut allergy must be vigilant about the foods they eat and the environments they enter, which can be very stressful,” said NIAID director Dr. Anthony Fauci. “One goal of experimental approaches such as epicutaneous immunotherapy is to reduce this burden by training the immune system to tolerate enough peanut to protect against accidental ingestion or exposure.”

“The clinical benefit seen in younger children highlights the promise of this innovative approach to treating peanut allergy,” said Dr. Daniel Rotrosen of NIAID. “Epicutaneous immunotherapy aims to engage the immune system in the skin to train the body to tolerate small amounts of allergen, whereas other recent advances have relied on an oral route that appears difficult for approximately 10 to 15 percent of children and adults to tolerate.”

Nearly all of the study participants followed the EPIT regimen as directed. None reported serious reactions to the patch, although most experienced mild skin reactions, such as itching or rash, at the site of patch application.





Lindner Named Director of CSR Office

The Center for Scientific Review has named Dr. Mark Lindner as new director of its Office of Planning, Analysis and Evaluation. He has been working part-time in this office for the last 5½ years

while serving as scientific review officer for several CSR study sections, including small business applications and, most recently, cognition and perception.

“I am very pleased that Mark will be stepping into this critical position,” said CSR director Dr. Richard Nakamura. “His scientific, analytic and statistical expertise is broad and deep. He also brings remarkable insight into current and needed research to evaluate and improve peer review.”

The Office of Planning, Analysis and Evaluation provides data, analysis and evaluations of the peer review of NIH grant applications for special projects, inquiries and advisory council activities.

Lindner received his Ph.D. in biopsychology from the University of Texas. After postdoctoral training, he spent 15 years managing and coordinating drug discovery research in industry at CytoTherapeutics in Lincoln, R.I.; Neurocrine Biosciences in San Diego; and Bristol-Myers Squibb in Wallingford, Conn. He also did consulting with McArthur and Associates GmbH, in Basel, Switzerland.

Lindner has published numerous papers that discuss how scientific research can be evaluated, conducted and managed to increase scientific productivity and the rate of progress. His interests range from the reductionist focus on molecular mechanisms to the psychology of the scientist and systemic and organizational factors that affect human judgment and decision-making.



NCI’s Keith Brosky

NCI’s Brosky Says Farewell

BY PATRICIA A. FETSCH

After 18 years at NIH, Keith Brosky, who joined the NCI Laboratory of Pathology in 1998 as a cytotechnologist, is hanging up his lab coat. Friends and co-workers helped celebrate the end of an era on Oct. 6.

“A truly vital part of the laboratory,” according to colleagues, Brosky was instrumental in developing new procedures, training residents and fellows and even collaborated with hospitals in Kenya to improve cytology services and access to patient care in that country. In addition, he co-authored numerous publications. Also, because of his direct patient contact, he has seen dramatic improvements in the patients who have been involved in cancer clinical trials over the years.

“I enjoyed working in the cytology laboratory with co-workers who have been like a family to me,” Brosky said. “There is a complete team effort, and technical and support staff are treated with respect and as equals to the staff physicians. The taco parties we had a few times each year were also a plus!”

Dr. Armando Filie, head of the cytopathology section, said, “Keith has a keen knowledge as a cytotechnologist and dedication and professionalism not only with regard to patient care but also in maintaining the lab. Of course, his dry and witty sense of humor could always make even the most

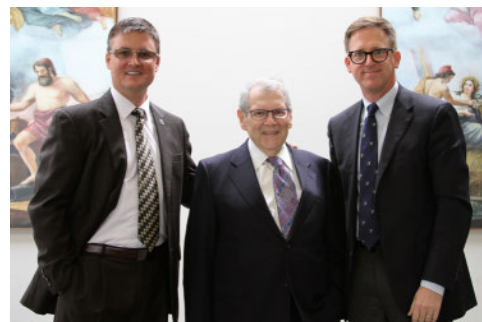
stressful situations less tense. Keith has been a critical part of the cytopathology section.”

Brosky took a great deal of pride in taking on difficult jobs and his presence in the laboratory will be genuinely missed by all who knew him, colleagues said.

“Keith’s technical expertise, professionalism and commitment to patient care set a uniquely high standard that will continue to serve as an inspiration to everyone who has had the pleasure to work with him,” said staff cytopathologist Dr. Mark Roth. “He is truly among the best, with a distinctive blend of humor, joy and efficiency that will be deeply missed and that is unlikely to be replicated.”

Brosky and his wife, Kathleen, who is also retiring at this time from the NRC, will stay in the area for the next year or so, but plan to move to Pennsylvania or Delaware and downsize in the future.

There will also be lots of traveling, with a trip to Italy already scheduled for next year. Brosky will also catch up on some reading, make a few home improvements and potentially foster cats.



NIAMS director Dr. Stephen Katz (c) joins fellow speakers on Capitol Hill—Maj. Mark Sullivan (l) and David Gillies.

Myotonic Dystrophy Briefing Features Katz

NIAMS director Dr. Stephen Katz recently presented on Capitol Hill at the invitation of the Myotonic Dystrophy Foundation. Myotonic dystrophy is a genetic muscle disorder that affects many parts of the body and currently has no cure. Katz joined fellow speakers David Gillies of the Senate appropriations committee and Maj. Mark Sullivan (U.S. Air Force, ret.) in discussing the importance of research and collaboration to improve myotonic dystrophy understanding and patient care. Katz gave an overview of current myotonic dystrophy-related research funded by NIH and described recent advances made in explaining this condition. Gillies summarized the Department of Defense’s Congressionally Directed Medical Research Program, its history, contributions and current outlook. Finally, Sullivan gave a compelling personal account of his stepbrother’s experience living with myotonic dystrophy while serving in the military.

PHOTO: COLLEEN DUNDAS



Hodes Receives Lifetime Achievement Award

NIA director Dr. Richard Hodes (c) was recently honored with the President’s Council on Fitness, Sports & Nutrition’s 2016 Lifetime Achievement Award. He was one of five recipients of this year’s award, which recognizes individuals whose careers have greatly contributed to the advancement or promotion of physical activity, fitness, sports and nutrition-related programs nationwide. The award was presented recently by Dr. Wanda Jones (l), senior advisor, offices of the assistant secretary for preparedness and

response and assistant secretary for health at HHS, and JoHannah Torkelson (r), Oakridge Institute of Science and Education fellow at the President’s Council.

PHOTO: STEPHANIE NATOLI

NIAMS's Gourley Mourned

After living with kidney cancer for 4½ years, Dr. Mark F. Gourley, former director of the NIAMS Rheumatology Fellowship and Training Branch, died on Sept. 17. He was 58 years old.

“Mark faced adversity with bravery, humility and kindness, continuing to be a great teacher even as he dealt with his own illness,” said NIAMS clinical director Dr. Richard Siegel. “He will be greatly missed by the many physicians, nurses, researchers, patients and friends whose lives he touched and enlightened with his wit and wisdom.”

When Gourley was still a student at Tulane University Medical School in New Orleans, he had his first experience at NIH, completing a 9-week immunology rotation. In 1988, after finishing his residency at the University of Washington, he returned to the Bethesda campus as an NIAMS rheumatology fellow.

Dr. Lisa Rider, who trained with Gourley at NIAMS, said, “Mark was a very warm, personable and caring person who always took time to smile and to help everyone—patients and colleagues at every level. He is remembered as an outstanding rheumatologist, a true expert in the clinical care of lupus and myositis and he was beloved by his patients, fellows and colleagues.”

At NIAMS, Gourley began his career as a lupus researcher, eventually conducting the landmark study that established cyclophosphamide as the standard of care in the treatment of lupus nephritis.

In 1996, he left NIH to establish Washington, D.C.'s first lupus clinic at the Washington Hospital Center. He returned to NIH 6 years later as a clinical investigator at NIEHS, where he focused on environmental causes of autoimmune diseases.



Dr. Fred Miller worked with Gourley at NIEHS and throughout his NIH career. “While his clinical and scientific skills are justly celebrated, we will always remember his kind humor, dedication to patient care, generous training of young clinicians and intellectual stimulation of his colleagues,” said Miller.

In 2007, Gourley was recruited back to NIAMS—the institute he would call home—to direct the NIH Rheumatology Fellowship Program and to oversee clinical care at the NIAMS Community Health Center.

Dr. Adam Schifffenbauer, a fellow who trained under Gourley, said, “Mark was always able to get the best out of people. He knew the right way to get someone to excel and was always willing to go well past an extra mile to help others shine.”

In addition to his training role, Gourley continued to contribute to clinical research in myositis and other areas until his retirement in 2013. Dr. Paul Plotz, who worked with Gourley on muscle disease and myositis research, remarked, “He was a superb physician—always anxious to pass on any new knowledge to his colleagues and students and to draw out of students and fellows what they knew.”

Gourley is survived by his wife, Wendy Kisch; children and sons-in-law, Charlie Gourley, Justin Gourley, Lindsey and Tim Miller, and Jamie and Justin Dean; granddaughter, Elise Dean; mother, Phyllis Gourley; and sister and brothers, Carol Stadler, Paul Gourley and Glenn Gourley.

Overweight Volunteers Needed

Are you 30-65 years old, overweight or obese and have pre-diabetes? NIH obesity researchers want to learn how well a medication called roflumilast improves blood sugar levels in overweight and obese adults who are not diabetic but who have high fasting blood sugar levels (“pre-diabetes”). Compensation is provided for participation. For more information, call 1-866-444-2214 (TTY 1-866-411-1010). Refer to study 13-H-0123.

Study Seeks Healthy Older Adults

NIH researchers are seeking healthy volunteers 55-75 years old for a study about the effects of omega-3 oil and blackcurrant supplementation on heart health. Participants will have 4 outpatient visits over 24 weeks and take omega-3 oil and/or blackcurrant supplement. Compensation is provided. For more information, call 1-866-444-2214 (TTY 1-866-411-1010) and refer to study 14-NR-0034.

NHLBI Needs Healthy Adults

NHLBI study team seeks healthy adults for a research study. Researchers are studying cells taken from the lungs of healthy individuals to compare to cells taken from individuals with asthma and other lung diseases. Compensation is provided for participation. For more information, call 1-866-444-2214 (TTY 1-866-411-1010). Refer to study 99-H-0076.

NIAAA Recruits Drinkers

NIAAA invites volunteers, 21-60 years of age, who drink more than 15-20 alcoholic beverages per week to participate in a study researching if a medication reduces drinking. Research participation includes four outpatient visits that consist of alcohol self-administration, brain scans (MRI), blood draws and filling out questionnaires. Compensation may be provided. For more information, call (301) 827-0905. Refer to study 16-AA-0037.

ADHD Genetics Study

Take part in an NIH study seeking to identify the genes that contribute to attention deficit hyperactivity disorder (ADHD). For more information call 1-800-411-1222 (TTY 1-866-411-1010). Refer to study 00-HG-0058.

NIH Mission First, Safety Always Award



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At left, NINDS's Dr. Vicky Whittemore presents a poster on the institute's channels, synapses and circuits cluster at the nonprofit forum's first-ever poster session. At center, forum attendees (from l) Linda Cooper of the Association for Creatine Deficiencies, Monica Weldon of Bridge the Gap-SYNGAP ERF and Geraldine Bliss of the Phelan-McDermid Syndrome Foundation research support committee greet one another. Panelist Dr. Ray Dorsey (r) of the Center for Human Experimental Therapeutics at the University of Rochester Medical Center speaks on developing better clinical outcome measures.

PHOTOS: ANDREW PROPP

SO PATIENTS 'FEEL, FUNCTION & SURVIVE BETTER'

NINDS, Nonprofits Work Toward Shared Goal at Forum

BY SHANNON E. GARNETT

Representatives from more than 75 nonprofit organizations across the country recently attended NINDS's 10th nonprofit forum, "Progress through Partnership." The 2-day meeting, which focused on data and clinical trial readiness, gave patient advocacy groups an opportunity to learn more about NIH and NINDS and network with each other and staff.

"Everybody here is traveling the same path—to try and get better therapies for patients with neurological disorders," said NINDS director Dr. Walter Koroshetz. "The idea, from all points of view, is that working together is what is going to get us there...The name of the game is high-quality data. The goal...is to discuss how best to acquire the data needed to empower therapeutic trials. We want to empower the organizations to move therapy development forward to therapeutic trials, but also to power the trials so you get the answers you are looking for."

The meeting began with experts sharing lessons in developing natural history databases, challenges and opportunities for data integration and management and strategies for biomarker identification.

"The thing that is important is that partnerships can be developed with the NIH programs," said Steven Kaminsky of the International Rett Syndrome Foundation, addressing nonprofit colleagues. "Don't try to build something yourself that costs a lot of money for your foundation and is going to break your bank account...Your future is through your past and your past is a really good natural history study. NIH, through the National Center for Advancing Translational Science's Office of Rare Diseases Research and the Rare Disease Clinical Research Network, has essentially built a foundation for all of us to use. You just have to figure out how to use it."

Acting NINDS deputy director Dr. Alan Willard presented a primer on NIH and NINDS funding, priority-setting and decision-making.

"I'm going to take you through the life cycle of an idea," he said as he described how a new grant award is made—from the initial plan of the investigator/organization, through various scientific review processes and finally to the funding itself. Willard concluded with ways organizations can affect the process such as helping principal investigators recruit for clinical studies or recruiting young investigators to the field.

Ron Bartek of the Freidreich's Ataxia Research Alliance and NORD led a panel on developing better clinical outcome measures. The session's speakers talked about classic measures as well as innovative technologies and approaches for collecting and tracking data including telemetry using wearables and smartphone apps.

"If you understand your patient and your patient's natural history, you are far better able to select the clinical outcome measures and biomarkers that measure what's most important to that patient," he said. "The trilogy, the gospel, is what makes the patient feel, function and survive better."

At a breakout session on ultra rare diseases, Dr. Petra Kaufmann of NCATS spoke about GARD (Genetic and Rare Diseases Information Center)—a service designed to provide comprehensive information about rare and genetic diseases to patients, families, health care providers, researchers and the public. GARD was developed in collaboration with NCATS and NHGRI. According to Megan O'Boyle of the Phelan-McDermid Syndrome Data Network, "[GARD] is not reinventing the wheel. It's putting all the spokes in one place."

A new forum highlight featured posters representing nonprofit

organizations and NINDS programs. Attendees were able to explore the various programs in an easy-to-understand way. Also new this year was the inclusion of the industry perspective on therapy development represented by Dr. Sean Ekins of Phoenix Nest and Dr. Ronald Marcus of Cerecor.

Dr. Cynthia Rothblum-Oviatt of the A-T Children's Project moderated "Cultivating Collaboration on a Shoestring and Investing in the Intellectual Pipeline," which focused on building a multi-stakeholder network and best practices for finding, funding and retaining researchers.

Panelist Dr. Carsten Bönnemann of the NINDS Neurogenetics Branch presented models of two clinical trials of rare pediatric disorders that involved multiple stakeholders including government, academia, industry and advocacy groups. Another panelist, Tish Hevel of the Brain Donor Project, described her work on raising awareness of and simplifying post-mortem brain donation.

The meeting ended with success stories from the Charlotte and Gwenyth Gray Foundation, CurePSP and the Alzheimer's Association. Their topics ranged from leveraging resources, to network building, to strategic planning.



NINDS director Dr. Walter Koroshetz (l) gives opening remarks at the institute's nonprofit forum. Gordon Gray (c), founder of the Charlotte and Gwenyth Gray Foundation, shares lessons during a success stories session. Dr. Shashi Amur (r) of FDA's Office of Translational Sciences at the Center for Drug Evaluation and Research provides strategies for biomarker identification.