

Research News Quarterly

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Letter from the Editor

The February ATS Research News Quarterly features an interview with the Director of the National Institute for General Medical Sciences (NIGMS), Jon Lorsch, Ph.D. Dr. Lorsch shares his vision for the institute over the next five years, how sequestration funding cuts affected NIGMS research and how NIGMS programs address sepsis and critical illness.

This month's Quarterly also features a article by Stephanie Davis, M.D. and ATS Vice President Tom Ferkol, M.D., on pediatric pulmonary research, including in rare lung diseases. The article also describes the growing shortage of pediatric pulmonary scientists and how this will impact the field. Next is an update on the Patient-Centered Outcomes Research Institute's (PCORI) new grant opportunities.

We then bring you the latest from NHLBI on the institute's priorities and budget outlook, followed by the NIH's announcement of the naming of the First Chief Officer for Scientific Workforce Diversity, Hannah Valantine, M.D. The February Research News Quarterly concludes with an update on 2014 health research and services funding. We hope you enjoy it!

Sincerely,

Linda Nici, MD
Editor

New ATS Awards for Innovations in Health Equality

The ATS has created the Awards for Innovations in Health Equality to highlight and support individuals and programs that aim to reduce the differences in the quality of health and health care across different populations. The ATS will present two \$1,750 awards: one will be granted to a clinically focused initiative, and the other will be granted to an initiative that focuses on health equality policy, training, or career development.

[Learn more and apply.](#)



AGENCY SPOTLIGHT – NIGMS

Interview with NIGMS Director Jon Lorsch, PhD

Q. What is your vision for the institute over the next five years?

A. We have just begun working on a new five-year strategic plan, so this is a timely question. Our focus will be on three key goals – efficacy, efficiency and adaptability. We want to ensure that we are investing the taxpayers' money in the most effective and efficient ways possible to promote our mission of enabling the fundamental biomedical research that is the foundation for most breakthroughs in medicine. We also want to make sure that our internal procedures and funding mechanisms allow both us and the biomedical research community to adapt rapidly to changes in science, medicine and society.

A specific goal of this strategic planning process is to bolster our historical commitment to investigator-initiated research. As part of this effort, we will be reducing our use of funding opportunity announcements that are targeted at specific research areas. We will also be exploring the development of funding mechanisms that allow more flexibility and stability for individual investigators and teams of researchers.

Q. What impact has the sequestration funding cut had on the NIGMS in FY2013?

A. Sequestration had a considerable impact on NIGMS' ability to fund the best fundamental biomedical research in FY 2013. The Institute's budget decreased by \$134 million, or 5.5%. Our success rate – the number of funded research project grants (RPGs) divided by the total number of RPG applications – decreased from 24.4% in FY 2012 to 19.9% in FY 2013. This translated into 142 fewer funded RPGs in FY 2013 than in FY 2012. Funding of new investigators fell by 8.4%. We also had to reduce our research training support by 206 positions, or 4.8%. Our FY 2014 budget restores about \$67 million of the funds sequestered in FY 2013.

Q. Sepsis is a significant cause of death in the U.S. How are NIGMS's programs addressing sepsis and critical illness?

A. We are one of a number of NIH institutes and centers that fund research in this area. NIGMS currently has 47 active grants related

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Agency Spotlight *(Continued from page 2)*

to sepsis, which run the gamut from career development awards to R01s to clinical trials. All of these grants are for investigator-initiated research. Looking forward, we will maintain our focus on funding the best investigator-initiated, fundamental research in this area. We encourage the sepsis research community to move from a focus on animal models to studies in human cells and tissues and to increase the use of computational modeling to better understand the complex events that take place during this pathological state. We would also like to see more applications in this area from the emergency medicine community. Finally, a major NIGMS-sponsored clinical trial of standardized care in early sepsis has just been completed, and we are awaiting the publication of its results.

Q. What are some of NIGMS's collaborative initiatives with other NIH institutes on critical illnesses, such as NHLBI?

A. We have a new joint initiative with NHLBI called "Blood and Vascular Systems Response to Sepsis" [RFA-HL-14-028](#) that will fund multidisciplinary teams of investigators to study the molecular and cellular events that take place in the vascular system during sepsis. These teams will come together once a year to share ideas and develop common strategies. The initiative was announced in November 2013, and the response from the community has been very good so far.

Q. The ATS welcomes partnerships between clinicians and scientists in the fields of basic biomedical research such as Bioinformatics and Computational Biology, Pharmacology, Genetics, and Cell Biology to address important clinical issues such as sepsis. What is your perspective on the role of NIGMS in steering basic discovery closer to the bedside of patients and how NIGMS could catalyze these partnerships?

A. NIGMS has worked to accomplish this goal for a number of years now. Connecting basic science to improvements in clinical outcomes has always been a focus of our trauma and burn injury program, for example. We also supported a 10-year project that followed transcription profiles over time in severely injured or burned patients. For the clinical trials we fund, we require a basic research component as well as a prospective therapeutic component. We also emphasize the importance of basic research in the training programs we support for clinician-scientists.

We see a lot of promise in the investigator-initiated, multi-principal investigator R01 award mechanism for catalyzing partnerships between basic laboratory scientists and clinical scientists. As part of our strategic planning process, we will be considering additional mechanisms to support team-based research as well.

Q. NIGMS is home to the new Office of Emergency Care Research (OECR). How do you envision the role of NIGMS in promoting clinical studies and trials relevant to sepsis and critical illness?

A. Although it is housed in NIGMS, OECR coordinates and fosters emergency care research across NIH. Within NIGMS, as mentioned earlier, we hope to see more applications from investigators in emergency medicine doing research in the clinical areas we support – sepsis, trauma, anesthesia, burn injury and wound healing. OECR will work closely with the community to stimulate research in emergency medicine departments. One way OECR will do this is to help secure funding from multiple NIH institutes or centers for outstanding applications in emergency medicine research that do not align with the mission of a single institute or center. The office is also working to enhance career development programs for emergency medicine researchers. ■

PEDIATRIC LUNG RESEARCH

The Disappearing Pediatric Pulmonary Scientist

by Stephanie Davis, M.D. & Tom Ferkol, M.D.

The opportunities for discovery research in pediatric lung diseases have never been greater. Newer technologies, such as genomics, proteomics, bioinformatics, computational modeling and systems biology, have the potential to unlock the complex interactions between genes and gene products in health and disease. During the past decade, we have witnessed scientific breakthroughs that have led to novel treatments that have the potential to profoundly change the course of lung diseases in children and adults. Indeed, we are poised to enter an era of miracles and wonder.

However, we are producing fewer pediatric pulmonary scientists who can translate discoveries at the bench to the patient, thereby hindering our ability to develop novel approaches to prevent and cure both childhood and adult lung disease. A shrinking pool of young pediatricians enters pulmonology each year, and only a handful choose to pursue a research career, which is leading to a serious shortage of well-trained clinician-scientists. We have reached a crisis point.^{1,2}

To train and mentor the young physician scientist, we first must increase the number of pediatric residents entering the field of respiratory medicine. The number of residents entering pediatric pulmonology has changed little over the past decade, and each year, many pediatric pulmonology fellowship positions go

unfilled. This apparent disinterest must be tackled. Residents may not enter the field for a number of different reasons, ranging from the perceived long hours, the poor reimbursement rates for chronic care, and increasing clinical demands. In addition, pediatric pulmonology is becoming more of a consultant service at many hospitals; thereby, leading to less contact with residents.

Attracting young scientists early may be possible through mechanisms such as the Ruth L. Kirschstein National Research Service Award Short-Term Institutional Research Training Grant (T35), a National Institutes of Health-supported program for medical students interested in academic careers, or Cystic Fibrosis Foundation-sponsored programs where pediatric residents have the opportunity to attend the North American Cystic Fibrosis Conference each year. The American Thoracic Society could potentially adopt similar approaches. Academic programs that allow pediatric residents interested in science greater time for research and opportunities to interact with successful pulmonary scientists is yet another mechanism to attract our younger colleagues. Such innovations are critical to attract the next generation of pediatric scientists.

The growing clinical demands placed on the pediatric pulmonologist have reduced opportunities for academic investigation. There are just short of 900 American Board of Pediatrics-certified pulmonologists currently practicing in the United States, and on average, one pediatric pulmonologist for over 83,000 children. In some regions of the country, the ratio exceeds one in 200,000.³ Ultimately, clinical demands often outweigh the research interests, and pediatric pulmonologists have often chosen clinical care and shied away from the increasingly less traveled road of the physician scientist. This phenomenon is not surprising given that

¹ Rubenstein RC, Kreindler JL. On preventing the extinction of the physician-scientist in pediatric pulmonology; *Frontiers in Pediatrics*; 2014; 2 (4): 1-6.

² Ferkol T, Zeitlin P, Abman S, Blaisdell CJ, O'Brodovich H. NHLBI Training Workshop Report: The Vanishing Pediatric Pulmonary Investigator and Recommendations for Recovery. *Pediatric Pulmonology*; 2010; 45: 25-33.

³ American Board of Pediatrics Workforce Data 2012-2013; <https://www.abp.org/abpwebsite/stats/wrkfr/workforcebook.pdf>

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Pediatric Lung Research *(Continued from page 4)*

many academic divisions need clinicians to care for the rising number of children with respiratory difficulties. It is a rare section that does not have this need.

Lack of resources and hospital support are additional barriers to training and recruiting the physician scientists. Effective research mentors are also in short supply. A 2009 survey of pediatric department chairs reported that few if any pediatric pulmonologists at their institutions had National Institutes of Health Research Project Grants (R01). This leads to a vicious spiral – the lack of well-established research mentors with a productive research career means that fewer young faculty at that institution will be competitive or even capable of submitting a Mentored Research Career Development Award (K01 and K23). The impact of restrictive pay lines at the National Institutes of Health and other funding agencies on the career choices of young pulmonologists is still unclear, though it likely has not had a positive effect.

Another obstacle occurs at the hospital or institutional level, where they choose top priorities to funnel resources. Pediatric research is often ignored. Too often the focus is on conditions that primarily affect adults, and the fact that the origins of these diseases occur early in childhood and even in utero is disregarded. We know that pulmonary function tracks over time, and for many respiratory diseases, including those that seem to begin in adulthood, the die was cast early in life. Better diagnostics, earlier recognition, primary prevention, and effective treatments of respiratory diseases beginning in childhood should improve outcomes during adulthood. As pediatric scientists, we have the ability to change the trajectory of lung diseases with earlier treatment targeted at the

primary defect. But a very different message can be taken from institutional funding decisions – research in child lung health is not a priority.

During the past decade, pediatric pulmonology has focused on childhood asthma, cystic fibrosis, and increasingly pediatric sleep disorders. More recently, selected pediatric centers have developed expertise in rarer lung diseases, where research can be challenging and performing single-center clinical trials near impossible. The Cystic Fibrosis Foundation has served as a model for how best to study and develop therapies for a rare lung disease, but it is the exception. Funding for clinical and research centers in orphan diseases is needed, but difficult to obtain. As pediatric pulmonologists and physician-scientists, it is our duty to not only care for the child with rare lung disease, but to advance our knowledge of the pathogenesis of these conditions and ultimately improve outcomes. Establishing centers of excellence and partnerships with the National Institutes of Health Research, private foundations, societies, hospitals, and parent-based organizations will ultimately lead to improvement in our understanding and treatment of these diseases.

There are tremendous advantages and benefits in a career as a pediatric pulmonary scientist. We need to increase exposure to the subspecialty, better promote our field, and attract young physicians to pediatric pulmonary medicine. We should be strong ambassadors, and highlight the excitement of our specialty and impact we can have on our patients and their families. We can make a difference at the bench and bedside. Hopefully, these efforts will convince our younger colleagues to choose the “road less traveled” of the pediatric physician-scientist. ■

PCORI

PCORI Announces New Clinical Trial Grants and Spring Funding Cycle

The Patient-Centered Outcomes Research Institute (PCORI) has opened a new grant program to fund pragmatic clinical trials and large simple trials that compare outcomes between two or more approaches to addressing clinical challenges. Because these types of studies often require larger and longer funding commitments than PCORI's standard three-year awards, available funding for these projects will range from \$5 million to \$15 million in total costs, with grant terms of up to five years. PCORI will support up to nine large, multi-year studies totaling \$90 million in funding through this new grant program.

Interventions to be studied can include drugs, devices, and procedures, as well as other alternatives, such as medical and assistive devices and technologies, behavioral modifications, complementary and alternative medicine, and delivery-system interventions. Proposed studies should focus on outcomes that are meaningful to patients, such as morbidity, mortality, symptoms, functional status, quality of life, and absenteeism from work or school. PCORI is especially interested in trials that cut across clinical conditions and focus on patient-reported outcomes not previously studied, including pain, depression, or functional status.

PCORI's Spring funding cycle, which offers a number of different grant opportunities, has also opened. The Spring cycle supports up to \$81 million in funding for research grants through PCORI's five National Priorities for Research, which are:

- Assessment of Prevention, Diagnosis, and Treatment Options
- Improving Healthcare Systems
- Addressing Disparities

- Improving Methods for Conducting Patient-Centered Outcomes Research
- Communication and Dissemination Research

Letters of intent for the Pragmatic Clinical Studies, Large Simple Trials to Evaluate Patient-Centered Outcomes and the Spring funding cycle grants are due to PCORI by March 7. The funding announcement for all of the Spring cycle grants is available at <http://www.pcori.org/funding-opportunities/funding-center/>.

The funding announcement for the Pragmatic clinical studies and Large Simple Trials is available at <http://www.pcori.org/funding-opportunities/funding-announcements/pre-announcement-pragmatic-studies-to-evaluate-comparative-clinical-effectiveness/>. ■

HEALTH RESEARCH FUNDING

2014 Health Spending Finalized

During the week of January 17, 2014, Congress passed the final spending measure to fund government programs for FY 2014, sending it to the President for signature into law. The bill includes some mixed news for health research and services and environmental protection programs. The following are the budget specifics:

- The bill provides \$29,934 billion for the NIH for the remainder of FY 2014. This is a \$783 million, or 3.2% funding increase over the FY 2013 budget after the sequestration funding cut, but is \$706 million below FY 2013 pre-sequestration funding of \$30,640 billion.
- The bill provides \$2,998 billion for the NHLBI, an increase of \$87,312 million and 3% over the FY 2013 after sequestration.
- The bill provides \$6,900 billion for CDC, which is a \$618 million increase over the FY 2013 budget following sequestration and \$369 million, or 2.3% above the pre-sequestration budget, including

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Health Research Funding *(Continued from page 6)*

the following for CDC programs that the ATS monitors:

- \$135 million for CDC’s domestic TB program, which is \$2 million over the final FY 2013 funding level, following the sequestration funding cut, but 3.5% below the FY 2012 funding level of \$140.2 million.
- \$24,700 million for CDC’s asthma program, a funding increase of \$1,451 million over the FY 2013 sequestration funding level.
- \$205 million for CDC’s Office on Smoking and Health, an increase of \$19,592 million over the FY 2013 sequestration level.
- \$292,300 million for the National Institute of Occupational Safety and Health (NIOSH), an increase of \$9,176 million over the FY 2013 sequestration level.
- The bill provides \$8,200 billion for the EPA, which is a cut of \$278 million, or 3.5% below the EPA budget before sequestration.
- The bill provides \$236 million for USAID’s global TB program, which is a funding freeze with the FY2013 budget before sequestration. This funding is also the higher level proposed between the House and Senate for the program, which is a victory. The Senate had proposed a lower funding level of \$224 million for the program.
- The bill provides \$585,600 million for the VA Research program, a \$2.9 million increase over FY2013.

One of the final sticking points to agreement on the omnibus was concerning funding for implementation of the Affordable Care Act (ACA), with Republicans pushing for ACA cuts. The final bill freezes ACA funding but cuts \$1 billion from the law’s Prevention and Public Health Fund, which funds public health initiatives such as state tobacco cessation programs. The bill also cuts \$10 million from the Independent Medicare Payment Advisory Board, which was set up under the ACA to find savings in the Medicare

program and which has become unpopular with a number of members of Congress in both parties. The omnibus also maintains the same restrictions on federal agency staff travel as in FY 2013, which is a concern for the ATS.

Also important to note is that the bill appears to be free of controversial policy riders that have troubled other funding bills. Previous House appropriations bills have used the appropriations bills to prevent regulatory agencies like EPA and OSHA from moving forward on important regulations.

The spending process for FY 2015 will kick off on March 4 with the release of the President’s proposed budget. The President’s budget serves as a guideline for the congressional appropriations committees. Following its release, the congressional appropriations committees hold hearings and begin drafting the spending bills. Under regular order, which has not been followed for the past few years, spending bills begin moving through the appropriations committees in late spring and summer. Appropriators have expressed a strong desire to return to regular order and at this point there is some optimism that the appropriations cycle for 2015 may at least begin this way. ■

NHLBI

NHLBI Director Meets with Stakeholders

In January, National Heart, Lung and Blood Institute (NHLBI) Director Gary Gibbons, M.D., met with members of the NHLBI Constituency Group, a coalition of heart, lung and blood professional society and patient groups in Washington, to discuss the institute’s achievements, priorities and budget

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NHLBI *(Continued from page 7)*

outlook. Dr. Gibbons reported that sequestration funding cuts in fiscal year (FY) 2013 had a significant impact on the institute's capacity to support research. He said that in order for the NHLBI to continue funding as many grants as possible while maintaining training and career development awards (the only area that was not cut), competing and non-competing grants were reduced by about 5 percent. FY 2013 was the first time in more than fifteen years that the institute had to cut competing awards. Dr. Gibbons went on to say that with sequestration now hopefully behind us, "the institute is two-thirds of the way back" in terms of its budget.

Concerning NHLBI's priorities, Dr. Gibbons reported that fundamental science remains at the top. Implementation science is a growing area of focus for the institute and Dr. Gibbons stressed the potential in this area for making the best use of established science to impact public health practice. The institute's investment in training remains a top priority and Dr. Gibbons emphasized the institute's efforts to build "a workforce that looks like America."

Jim Kiley, Ph.D., Director of the Lung Division, gave an update on some of the key programs in lung research, including progress to new therapeutics in cystic fibrosis, upcoming results in COPD clinical trials and discoveries through the Centers for Advanced Diagnostic and Experimental Therapeutics (CADET) that hold the potential to drive personalized medicine. Dr. Kiley also discussed the institute's collaborative work with PCORI on reducing disparities in asthma care and better management of the disease in special populations. ■

WORKFORCE DIVERSITY

NIH Names First Chief Officer for Scientific Workforce Diversity

National Institutes of Health (NIH) Director Francis S. Collins, M.D., Ph.D. announced in January that he has appointed Hannah Valantine, M.D., as Chief Officer for Scientific Workforce Diversity at NIH. Dr. Valantine will lead the institute's work to diversify the biomedical research workforce by developing a comprehensive strategy to expand recruitment and retention, and promote inclusiveness and equity throughout the biomedical research enterprise. Dr. Valantine's appointment is in response to a recommendation by the Biomedical Research Workforce Diversity Working Group of the Advisory Committee to the Director (ACD) that called for a new position entirely dedicated to diversity.

Dr. Valantine most recently served as Senior Associate Dean for Diversity and Leadership at Stanford School of Medicine and Professor of Cardiovascular Medicine at Stanford University Medical Center. She graduated from St. George's Hospital Medical School in London, U.K., in 1978. Dr. Valantine is a past recipient of the NIH Director's Pathfinder Award for Diversity in the Scientific Workforce.

Dr. Collins said, "Recruiting and retaining the brightest minds regardless of race, ethnicity, gender, disability, and socioeconomic status, is critically important not only to NIH, but to the entire U.S. scientific enterprise." He continued, "Hannah possesses the experience, dedication, and tenacity needed to move NIH forward on this critically important issue." ■