



MESSAGE FROM THE EXECUTIVE DIRECTOR

We are only a little more than a month into the new year, but 2018 is already shaping up to be fast-paced and exciting for the DCRI. In this issue of the DCRI Newsletter, we'll share some of this excitement!

We have lots of new projects and studies to tell you about: A new tool could help doctors predict the progression of Alzheimer's disease in certain patients. A faculty member helped write new guidelines for treating patients with ventricular arrhythmias. Financial incentives could help young diabetics adhere to their medication regimens. The Pediatric Trials Network is going global. And financial barriers may be preventing some patients from getting better cholesterol-lowering drugs.

These stories only represent a fraction of the work that goes on here. I hope you'll keep checking in with us as we continue on our mission to share knowledge that improves the care of patients around the world.



Eric D. Peterson, MD, MPH, FAHA, FACC
Executive Director,
Duke Clinical Research Institute
Professor of Medicine, Cardiology
Fred Cobb Distinguished Professor of Medicine

THE DCRI AT ACC 2018



The DCRI is gearing up for this year's Scientific Sessions of the American College of Cardiology in Orlando, Florida. Check out <https://dcri.org/dcri-acc> for the latest on the research that our faculty, fellows, and operational teams will be presenting.

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Feature Stories

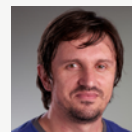
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NEW DCRI FACULTY HIRES:



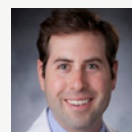
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Biostatistics and Bioinformatics



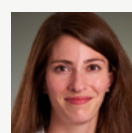
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Obstetrics and Gynecology



David Leiman, MD, MSHP
Gastroenterology



Sheng Luo, PhD
Biostatistics and Bioinformatics



Rachel Torok, MD
Pediatric Cardiology



Memory Models



Sheng Luo, PhD

In a recent study, researchers describe how novel statistical models could improve the prediction of progression-free survival in mild cognitive impairment (MCI) patients using multiple markers, all of which can be easily collected in a clinical setting.

“We have a burgeoning prevalence of Alzheimer’s because of the increasing number of people age 65 and older in the United States,” said Sheng Luo, PhD, senior author of the report and corresponding author of the study.

AN ESTIMATED 5.5 MILLION AMERICANS LIVING WITH ALZHEIMER’S DISEASE AND DEMENTIA IN 2017.

Alzheimer’s—a general term for memory loss and other cognitive abilities severe enough to restrict daily life—is the most common form of dementia, accounting for 60 to 80 per cent of dementia cases. The number of Americans living with Alzheimer’s disease is growing with an estimated 5.5 million Americans living with Alzheimer’s disease and dementia in 2017. Many patients with MCI will go on to develop AD.

“Given that the number of new cases of Alzheimer’s and other dementias is projected to soar and the lack of modifying treatments for the disease, the question that we wanted to ask was, how do we characterize the progression of Alzheimer’s for early detection and intervention in patients with MCI?”

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In Rhythm



Sana M. Al-Khatib, MD, MHS

The American College of Cardiology, along with the American Heart Association and the Heart Rhythm Society, has published new guidelines for the treatment of patients with ventricular arrhythmias and the prevention of sudden cardiac death.

SUDDEN CARDIAC ARREST AND SUDDEN CARDIAC DEATH ARE MAJOR PUBLIC HEALTH PROBLEMS, ACCOUNTING FOR APPROXIMATELY HALF OF ALL CARDIOVASCULAR DEATHS, WITH AT LEAST 25 PERCENT BEING FIRST MANIFESTATION OF CARDIAC DISEASE.

Ventricular arrhythmias are an abnormal heartbeat arising from the heart’s lower chambers, or ventricles. This condition can lead to cardiac arrest, which, in turn, results in sudden cardiac death if the abnormal rhythm is not quickly stopped to restore a normal rhythm.

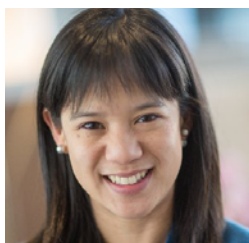
“The risks of ventricular arrhythmias and sudden cardiac death vary in specific populations with different underlying cardiac conditions, and with specific family history and genetic variants, and this variation has important implications for studying and applying therapies.”

“Sudden cardiac arrest and sudden cardiac death are major public health problems, accounting for approximately half of all cardiovascular deaths, with at least 25 percent being first manifestation of cardiac disease,” said the DCRI’s Sana M. Al-Khatib, MD, MHS, chair of the writing committee.

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Money Talks



Charlene Wong, MD

For children with type 1 diabetes, daily monitoring of blood glucose levels is vital for since glucose level awareness dictates the rest of their diabetes care, like insulin dose adjustments, eating behaviors, and physical activity. However, studies have shown overall diabetic glycemic control often deteriorates during adolescence, leading to increased risks of costly and potentially life-threatening complications.

A new study shows that using small financial incentives and accessible monitoring tools such as wireless glucometers and apps may motivate young people to engage in playing a more active role in the management of their condition.

The results of a randomized control trial, led by researchers at the DCRI and the Perelman School of Medicine at the University of Pennsylvania, show that participants in the intervention group, where a \$60 monthly credit was on the line, were nearly three times more likely to achieve daily glucose monitoring goals.

“Young people are often financially dependent on others, such as their parents, making financial incentives an attractive option for encouraging them to become more engaged in their own health as they move into adulthood,” said lead author Charlene Wong, MD, an assistant professor of Pediatrics at the DCRI.

PARTICIPANTS IN THE INTERVENTION GROUP, WHERE A \$60 MONTHLY CREDIT WAS ON THE LINE, WERE NEARLY THREE TIMES MORE LIKELY TO ACHIEVE DAILY GLUCOSE MONITORING GOALS.

“Our results showed that offering a small monthly financial incentive significantly improved daily glucose monitoring, and suggests similar financial incentives could also be an effective way to improve management of other chronic health conditions in youth, such as medication adherence in those who have received transplants or have asthma.”

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Around the World

The DCRI and its strategic partners have been awarded a grant from the U.S. Food and Drug Administration (FDA) to establish a coordinating center for a Global Pediatric Clinical Trials Network (G-PCTN). The G-PCTN will support efficient pediatric clinical trials worldwide by developing scientific and operational infrastructure, fostering collaborative networks, sharing knowledge, and engaging stakeholders. The principal investigators for this program will be Daniel Benjamin, Jr., MD, MPH, PhD, and Michael (Micky) Cohen-Wolkowicz, MD, PhD. The grant will be used to create a coordinating center comprising three cores for network operations, patient engagement, and scientific oversight and five clinical study groups devoted to study design, dosing, regulatory/pharmacy, network partnerships, and rare diseases.

THE G-PCTN WILL SUPPORT EFFICIENT PEDIATRIC CLINICAL TRIALS WORLDWIDE.



Daniel Benjamin, Jr., MD, MPH, PhD, and Michael (Micky) Cohen-Wolkowicz, MD, PhD

“Although we’ve made a lot of progress in recent years, pediatric trials are still hard to complete successfully,” said Benjamin. “There are perennial challenges – enrolling patients, finding access to supporting infrastructure, navigating a complex regulatory environment – that affect everyone working to advance pediatric research, but they can be especially challenging for research sites that don’t have access to resources or experience.”

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Sticker Shock



Ann Marie Navar, MD, PhD

Most prescriptions for a class of drugs heralded as game-changers for people with stubbornly high cholesterol are going unfilled because of high out-of-pocket costs and challenges by pharmacy benefit managers, according to a study from the DCRI.

The researchers found that fewer than one-third of patients prescribed a PCSK9 inhibitor — injectable drugs designed to lower cholesterol levels — actually got the drug.

Two factors limited access: lack of insurance approval for the prescription and high copays. Fewer than half of patients prescribed a PCSK9 inhibitor ever received approval for the drug by their insurer. Even after approval, one in three patients failed to fill their prescription.

ACCESS COMES DOWN TO PATIENTS WHOSE DOCTORS ARE PERSISTENT ENOUGH TO WIN PAYER APPROVAL THROUGH MULTIPLE APPEALS, AND PATIENTS WHO CAN AFFORD THE OUT-OF-POCKET COSTS.

The net result was that just 31 percent of patients who were initially prescribed a PCSK9 inhibitor ever actually received the therapy, the researchers found.

“This study basically reveals a system of rationing by roadblocks,” said lead author Ann Marie Navar, MD, PhD, assistant professor of medicine at the DCRI. “Access comes down to patients whose doctors are persistent enough to win payer approval through multiple appeals, and patients who can afford the out-of-pocket costs.”

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SELECTED PUBLICATIONS

Rethinking the current American Joint Committee on Cancer TNM staging system for medullary thyroid cancer

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