



Statement for the Record
by Dr. Grayson Norquist, M.D., M.S.P.H., Chairperson, PCORI Board of
Governors, on Behalf of the Patient-Centered Outcomes Research Institute
(PCORI)
Hearing Before the Energy and Commerce Health Subcommittee
On “Investing in America’s Health”
June 4, 2019

On behalf of the Patient-Centered Outcomes Institute (PCORI), we appreciate the opportunity to submit this statement for the record in support of PCORI reauthorization and commend Chairwoman Eshoo, Ranking Member Burgess, and the Members of the Energy and Commerce Health Subcommittee for focusing on this critically important issue, including legislation recently introduced by Rep. DeGette and Rep. Beyer, H.R. 3030, which would extend PCORI’s authorization. We look forward to continuing to work with the Committee in a bipartisan way to extend the work of the Institute beyond the current fiscal year.

I am Doctor Grayson Norquist, Chairperson of the Board of Governors for the Patient-Centered Outcomes Research Institute (PCORI). I am currently the Vice Chair of the Emory Department of Psychiatry and Behavioral Sciences and Chief of Psychiatry Service at Grady Health System in Atlanta. As a psychiatrist in Atlanta, I treat people every day who struggle with a range of serious mental illnesses. I have been caring for patients for almost forty years and I have practiced both in large urban areas like Atlanta and Los Angeles as well as in rural communities in the Mississippi Delta. I have treated patients from a wide range of social and economic backgrounds, including poor underserved areas as well as highly educated affluent communities. I have been a clinician, a researcher, division director at the National Institutes of Health (NIH), and held leadership positions at several major academic medical centers as well as the National Institute of Mental Health (NIMH). I have been a member of the PCORI Board of Governors for almost 9 years and served as the Chair of the Board for the past 6 years.

The patients I have cared for all have one thing in common – they are individuals in need of empathic, effective and individualized medical care. They are people with lives, families to care for, and jobs they depend on to support their families. To care for my patients and help them achieve the health outcomes that matter to them, the care I provide must begin and end with their priorities and values and incorporate their needs and perspectives. And that is PCORI’s sweet spot – transforming the clinical research enterprise by putting patients, and their real-world needs, at the center of everything we do and designing research to answer real world questions that matter to patients and the physicians who care for them.

I recently treated a woman suffering from severe depression, with episodes of uncontrolled crying, detachment from her world, and feelings that she no longer wanted to live. She had times when she heard voices telling her she was a terrible and unlivable person. I knew there were multiple medications that could help improve her mood and address the occasional hallucination. But each of these medications has side effects which could potentially worsen other medical problems she suffers from. It was unclear which of these medications might work for her – given that response to an antidepressant is very individual – and whether she would benefit from, or be harmed by, an additional medication to address her hallucinations.

My patient is a wife and mother of young children whom she needs to care for and has a job she needs to maintain to help support her family. I had multiple treatment options to choose from, with a wide range of effectiveness and associated side effects of varying severity. The research studies that evaluated these treatment options were largely designed to answer questions of interest to the investigators, or to the manufacturers sponsoring the trial. They often excluded patients with multiple co-morbid conditions, like my patient, and looked at a narrow set of outcomes for the “average patient” in the trial. To achieve the best clinical outcome for my patient I needed the results of a different kind of research, research that captured and assessed the outcomes that matter to patients and include in the research design patients with a variety of co-morbid conditions.

This is, in fact, PCORI’s mandate – to play this unique role the country’s research enterprise. Our work over the last nine years has focused on filling the evidence gap, and providing patients and clinicians with research results that enable a shared and informed decision about the right treatment option for an individual patient – research that begins with questions that matter to patients and clinicians, that

incorporates patient values and priorities in the research design, and delivers results that are useful and impactful at the point of care.

Today I want to share with you PCORI's accomplishments in providing meaningful information to empower patients and clinicians to make truly informed and individualized decisions. I will also speak to what PCORI will be able to achieve if reauthorized by Congress.

I. PCORI's Role in the Healthcare System – *Research Done Differently*

Just like the woman in my office, patients work with their doctors and other clinicians every day to make choices about care in medical offices, clinics, and hospitals across our nation. Other healthcare stakeholders do the same. All want to know which treatment or care options might be best for individual patients, so they can make the most informed decisions possible. Too often, though, those decisions are made in the absence of complete or relevant evidence for the person who presents for help.

That's why PCORI was authorized by Congress – to fund research that fills gaps in knowledge and enables patients, working with their doctors and other clinicians, to choose the care options best for them, based on their personal circumstances, values, and desired outcomes. PCORI is there to inform the decisions that patients and clinicians make together by funding research that is relevant and responsive to their needs. At the heart of PCORI's work is our commitment to involving patients and other healthcare stakeholders, including caregivers, researchers, clinicians, insurers, health systems, policy makers, and employers, every step of the way from topic selection and research question design to dissemination of results and support for implementation and uptake into practice. We engage patients and other stakeholders in all aspects of our work and require our funded researchers do the same.

We seek to ensure we ask the right research questions, study the outcomes that matter most to patients and produce useful and relevant results that will be implemented in practice. By engaging the end users of study results throughout the research process, we are funding research that will produce results that are more likely to be used in practice. This approach is called patient-centered outcomes research, or PCOR.

II. A Robust Portfolio Designed for a High Impact

PCORI funds a robust portfolio of patient-centered outcomes research that addresses a variety of high-priority conditions and topics. Focus areas include some of the most common medical problems such as cancer, cardiovascular disease, dementia, diabetes, kidney disease, mental illness and substance abuse, multiple sclerosis, obesity, pain care and opioid abuse. Other areas of focus include specific populations, such as veterans, older adults, children, patients with rare disorders, and patients with multiple chronic conditions and advanced illnesses. Our funded research also explores new and emerging approaches to care, such as telehealth, community health workers, transitional care and other system level interventions. **Exhibit A** provides an overview of our research portfolios in each of these areas.

We also have a broad geographic reach, funding research in a wide variety of communities. Overall, we have funded more than 600 research studies across 44 states. In Texas, for example, we support the BEST-MSU Study which is comparing two ways to provide rapid brain-saving emergency care for patients who appear to be having a stroke. This research team from the Memorial Hermann Health System and Medical Center is comparing mobile stroke treatment in an ambulance with care that starts in the emergency room. Another study in Texas looked at whether a patient decision aid could provide the evidence to help heavy smokers make better and more informed decisions about lung cancer screening.

In California, the BCSC-ADVANCE Study is looking at how women can use information about breast density to improve their decisions for breast cancer screening and diagnostic testing. And in the face of the crisis in maternal mortality, PCORI is partnering with the University of California, San Francisco to compare group-based and one-on-one approaches to enhanced prenatal care in an effort to identify the best way to improve maternal and child health outcomes.

III. Hitting Our Stride and Building Momentum

PCORI has spent nine years establishing a transparent and efficient infrastructure, building trust and buy-in with stakeholders across the health sector, and developing its portfolio. We have built momentum, and exciting and practical results are coming in from our 600-plus research projects funded to this point. PCORI-funded research results are publicly available on our website, written in clear language for both professional and lay audiences. These studies have also been featured in 1,947 peer-reviewed journal articles. In addition to the 278 fully completed studies, 354 studies are still underway

in our pipeline. PCORI also supports efforts to drive implementation of impactful findings into practice in real-world settings. Here are some highlights of our funded research studies that have delivered results of interest to stakeholders.

One PCORI-funded study found that programs implemented in community mental health centers can help people with serious mental illness improve the management of their health conditions and prevent later health problems. This study examined two models of behavioral health homes and found that both intervention models improved patients' quality of life, involvement in their healthcare and satisfaction with this care.

Another PCORI funded study resulted in findings that support options to reduce risky opioid prescribing. This study compared rates of opioid use in clinics in Washington State that implemented an initiative focused on encouraging and supporting more-cautious prescribing of opioid drugs with clinics that did not use such strategies. This health system-based initiative led to reductions in high-dose opioid prescribing, and importantly, patients did not report less ability to control their pain.

Yet another PCORI-funded study found that an intervention using a simple questionnaire enhanced decision making by emergency department physicians caring for patients experiencing chest pain. A questionnaire called the Chest Pain Choice was found to help people who go to the emergency department with chest pain, decide whether to immediately have follow-up tests or go home and have the tests later when they can be routinely scheduled. People who used the aid were much more likely to go home from the emergency department, with no increase in later heart-related problems and fewer out-of-pocket costs. Over five years, 9.4 million people could benefit from using this decision aid that decreases time spent in the hospital and reducing the need for unnecessary and potentially risky tests.

These are just a few examples of the real difference PCORI-funded research is making for patients, their doctors, other clinicians and the healthcare system. In a number of these studies, we found that providing care in a more patient-centered way not only improved their outcomes, but also cut down on the future use of medical resources like re-hospitalization and emergency department usage and increased time at home. In addition, other studies completed or underway will provide patients, their caregivers, and providers with evidence-based information to make decisions focused on what matters

most to them and incorporating their personal preferences, values, circumstances, and the outcomes they care most about.

That's PCORI's guiding principle: putting the patient first. It is the Country's first and only independent research organization that gives everyone in the healthcare community a seat at the table, so patients—alongside clinicians, insurers, and others—drive the research agenda, identifying the questions to be studied and getting the information they need to make care decisions that are right for them.

PCORI does more than just generate new scientific results, we are also deeply engaged in disseminating these results and getting them adopted into clinical practice. Our Patient-Centered Outcomes Research Translation Center works with researchers to produce easy-to-read summaries for patients and the public describing research results and separate summaries for clinicians and researchers with study technical details and findings. We prepare Evidence Updates in concise, accessible formats and deliver them in partnership with patient organizations and others. We also work with a variety of stakeholders to communicate findings and promote their use in practice through dissemination and implementation awards.

IV. Delivering Value to Patients, Clinicians, and the Health System

Thanks to the initial Congressional investment in PCORI in 2010, today we are delivering meaningful results for patients, clinicians, payers and the broader health system. PCORI funds research that is answering critical clinical questions – different questions than those typically studied by other research funding agencies. When patients have the information needed to make more informed choices about their care, they are more likely to have improved health outcomes and avoid unnecessary treatments that could be of low value or lead to undesirable outcomes such as needing to be hospitalized or having adverse side effects.

Of course, PCORI's value should not be measured by the number of studies we fund or the reports we have published. PCORI is best measured in the ways in which we have impacted the health of patients and improved the healthcare system. We expect the results of the research we support will be used by patients, clinicians and the health system to improve decisions about health care. We believe research done differently will empower patients to obtain the health care outcomes they most desire and provide

more meaningful information to clinicians as they struggle to decide what treatments are best for the patients with whom they work every day.

PCORI's work is also strengthening the wider health system in the United States, particularly as the US health system explores innovative delivery and payment methods. Our work to put the patient at the center of everything we do affects the dialogue in healthcare – shifting the focus towards patient-centered outcomes.

We are improving the efficiency and speed of conducting health research through PCORnet, a PCORI-funded initiative to enable patient-centered clinical research to be conducted on a larger scale in a more efficient manner. For example, PCORnet has just completed recruitment of 15,000 persons who are known to have heart disease for a clinical trial of the very important question of “what is the right dose of aspirin to prevent another heart attack or stroke without increasing risks for bleeding”. PCORnet currently includes more than 45 large health care delivery systems and draws on clinical and electronic health record data of more than 30 million persons in the U.S. It can reach out directly to these patients and to their clinician. It is well suited to conduct both clinical trials and “big data” studies that observe how clinical outcomes occur in everyday practice across large numbers of diverse people. These are “real-world” data and come from routine care through electronic health records (EHRs), patient-reported outcomes, health claims, and other sources. By leveraging health information collected in everyday healthcare situations and settings, the evidence that PCORnet produces reflects realistic information about the comparative clinical effectiveness of therapies, diagnostics, and prevention strategies. The large size of PCORnet's database is critically important for understanding differences among patient populations, and also for identifying enough patients to allow studies of those with rare diseases, for measuring healthcare use and outcomes, and for conducting ongoing evaluations of the safety of drugs after they're approved. PCORnet unites patients, clinicians, researchers, health systems, and health plans to expedite research that can improve healthcare and patient outcomes.

V. **Going Forward**

The past two years have brought significant achievements to PCORI in the form of 278 completed studies that promise to change clinical practice. Today, though, I would like to speak not only to what PCORI has done since it was created in 2010, but what we are poised to do with further funding following Congressional reauthorization. During 2019, PCORI plans to invest an additional \$400 million in

research and other initiatives on high priority areas, including opioids and cancer, identified by patients and other stakeholders. We continue to work closely with our stakeholders to study to identify the important research questions that need to be answered by decision-makers and patients. With re-authorization of funding for PCORI, we will be able to fund larger landmark studies of innovative treatments and their health impact on patients that will provide answers to the most pressing questions facing patients and clinicians on what treatment interventions are best and inform health systems and payers on what interventions are of greatest value. This is especially true for the many new—and typically high-burden—therapies and technologies that may have the potential to revolutionize treatments for certain conditions. PCORI’s style of research asks, specifically, “who” will benefit from these new treatments. PCORI will continue to take advantage of the infrastructure made available through infrastructure initiatives like PCORnet. For example, PCORI will work with the rare disease community to use the large populations available through PCORnet to advance research that can help answer questions on what works best for patients with rare diseases.

As results from many more of our funded studies become available, we will continue to focus on making those findings available to the public as widely and quickly as possible and promote the use of this information in practice. To leverage the important potential of PCORI funded research findings, we will continue to work with patients and clinicians, health plans, integrated delivery systems, employers and others to implement these research findings at the point of care and decision making. Enhancing and supporting the crucial bond between a patient and their clinician has always and will continue to be a major focus of PCORI’s efforts. This is why payers and employers; physicians and patient groups support reauthorization.

PCORI was created to fund research that fills gaps in evidence on different health treatments and enables patients, working with their doctors, to choose the care options best for them, based on their personal circumstances, values, and desired health outcomes.

PCORI has spent nine years establishing a transparent and efficient infrastructure for funding patient-centered outcomes research, building trust with stakeholders across the health sector, and developing its research portfolio based on their information needs. And PCORI’s research has made a real impact—demonstrating how to improve patient outcomes and increase value for patients and society—including many of our country’s most pressing health care challenges, such as addressing the opioid epidemic,

heart disease, mental and behavioral health, cancer, obesity, and diabetes. Importantly, PCORI funded research is all publicly available in understandable language.

Going forward, PCORI will build on that foundation to continue funding user driven research in high priority areas and supporting the decisional needs identified by patients and other stakeholders. This includes:

- **Supporting Landmark Research on Innovative Therapeutics**– As new and innovative treatments and therapies continue to be developed, PCORI is best situated to assess the health impact of those options in real-world settings to help patients make informed decisions about their choices. This is especially true when we consider the many new—and typically high-burden—therapies and technologies that potentially have great promise to revolutionize treatments for certain conditions that were previously untreatable.
- **Addressing Issues of Importance to Americans:** PCORI will continue to make significant research contributions to help answer pressing questions about best ways to treat and ameliorate the opioid epidemic, manage multiple chronic conditions so patients can have the best quality of life and independence, and addressing emerging public health needs.
- **Leveraging Real-World Data using Efficient and Re-usable Research Networks** – PCORI’s investment in the Patient-Centered Outcomes Research Network (PCORnet) is leveraging real-world data to conduct research more efficiently and at much lower cost while ensuring the quality of that data is reliable. Additionally, PCORnet makes doing research on rare diseases more efficient because PCORnet represents over 30 million patients. PCORI will continue to expand our partnerships with existing databases, both public and private, such as NIH’s *All of Us*, FDA’s Sentinel Initiative and patient registries, to harness the power of real-world data to support research that leads to knowledge about new innovations and advances personalized and patient-centered care.
- **Advancing Patient-Centered and Value-Based Care while Improving Health Outcomes** – Unique among research funders, PCORI not only invests in patient-centered research that is user driven so that PCORI-funded research is uniquely responsive to everyday health care questions. PCORI funded research is already improving the health outcomes of patients, including those with serious mental illness and diabetes. Earlier, I described a simple decision aid can help people who go to the ER with chest pain better understand their risk of having a heart attack and decrease unnecessary hospitalizations for testing. Over five years, this could benefit 9.4

million Americans and save \$4.8 billion nationwide. As more research findings become available, PCORI will continue to be a leader in making research findings available to the public and the health care system. Our investment in inventive dissemination and implementation of results and partnering with patients, clinicians, and health care systems to get the research to the right patient and the right time. PCORI will work with patients, stakeholders from across the health sector, and policymakers to advance patient-centered and value-based care through the practical implementation and use of Shared Decision Making. The time that it takes for research to make its way into practice takes a very long time and PCORI will be dedicated to shortening that period, so the research investments can have the biggest positive impact on individuals and the health care system.

Thank you for this opportunity to submit written testimony about PCORI's important work and future potential. I look forward to the day when I will have enough information to help patients like the woman I saw with depression, so they can obtain quickly the outcomes they desire from the treatments we have to offer them. I urge Congress to renew its investment in patient-centered research, so we can attain that opportunity.

EXHIBIT A

Highlights of PCORI-Funded Research Results

A growing number of PCORI-funded studies have produced important results that are being reported in leading medical journals and have the potential to improve patient care and outcomes. Here are some examples:

For Many with Type 2 Diabetes, Daily Finger Sticks Offer Little Health Benefit

People with type 2 diabetes who are not using insulin are often advised to check their blood sugar levels using daily finger sticks, which can be painful and inconvenient, as well as run up out-of-pocket costs for test strips. This study suggests that for these patients, daily self-monitoring does not help control diabetes or delay the need to start insulin compared with not doing so. Young L et al. *JAMA Intern Med.* 2017 Jul 1; 177(7).

Initiative to Reduce Risky Opioid Prescribing Works

This study compared rates of opioid use in clinics in Washington State that implemented an initiative focused on more-cautious prescribing of opioid drugs with clinics that did not use such strategies. This health system-based initiative led to reductions in high-dose opioid prescribing, and patients did not report worse pain control. Von Korff M et al. *J Pain.* 2016 Jan; 17(1).

Engaging Parents in Hospital Rounds to Ensure Patient Safety

Improving communication among patients and staff in the hospital can help reduce harmful medical errors, a leading cause of death. A PCORI-funded research team found that using a program called I-PASS, which includes parents as active participants in clinicians' rounds of pediatric units, reduced preventable adverse events by 38 percent. Landrigan C et al. *BMJ.* 2018 Dec 5; 363:k4764.

Bypass Shown to Be Most Effective Weight-Loss Surgery Procedure

This study, the largest to date to compare weight-loss surgeries, analyzed 46,000 patients' outcomes using PCORnet. Adults who had Roux-en-Y gastric bypass, a long-used approach, lost more weight and kept it off better than those who had the newest procedure, sleeve gastrectomy. Both bypass and sleeve were more effective than adjustable gastric banding. Risks of major adverse events shortly after surgery were small for all three surgeries but were highest for bypass. Arterburn D et al. *Ann Intern Med.* 2018 Oct 30;169(9).

Simple Questionnaire Enhances Shared Decision Making about Chest Pain

A questionnaire called Chest Pain Choice can help people who go to the emergency department with chest pain, but who are found to not be having a heart attack, decide whether to be admitted to the hospital for follow-up tests or go home and have the tests later. People who used the aid were much more likely to go home from the emergency department, with no increase in later heart-related problems. Hess E et al. *BMJ.* 2016 Dec 5; 355.

For Earaches and Strep Throat in Children, Narrow-Spectrum Antibiotics Are Better

Narrow-spectrum antibiotics did just as well for clearing up ear infections and sore throats caused by bacteria as more-expensive broad-spectrum antibiotics did. Broad-spectrum drugs caused more side effects, such as vomiting. Unnecessary use of broad-spectrum antibiotics is associated with increasing bacterial drug resistance. Gerber JS et al. *JAMA*. 2017 Dec 19;318(23)

Oral Antibiotics Work as Well as IV, with Fewer Costly Complications

In preventing a recurrence of infection, children discharged from the hospital after a serious bacterial infection did just as well on oral antibiotics as those sent home with an IV line to deliver antibiotics intravenously. They also had none of the frequent complications that IV lines can cause. Keren R et al. *JAMA Pediatr*. 2015 Feb; 169(2) Shah S et al. *Pediatrics*. 2016 Dec; 138(6) Rangel S et al. *Ann Surg*. 2017 Aug; 266(2).

Disadvantaged Patients with Chronic Pain Benefit from Tailored Nondrug Therapies

Chronic pain occurs more frequently in people with lower education and income levels. In this study, both cognitive behavioral therapy and pain educational material, each tailored to match patients' education levels, significantly lessened pain and improved physical function compared with usual care. These nondrug approaches can help manage chronic pain and reduce the need for opioids. These findings show such approaches can be effective in patients with low incomes and limited reading skills. Thorn B et al. *Ann Intern Med*. 2018 Apr 3;168(7).

Findings Help Men Choose among Prostate Cancer Treatment Options

Two PCORI-funded studies provided men with up-to-date information about the rates of several major side effects associated with current treatments for prostate cancer. The information applies to robot-assisted surgery and newer forms of radiation therapy, as well as active monitoring instead of immediate treatment. These results will help men with prostate cancer and their families better weigh the benefits and risks of each treatment in consultation with their clinicians. Barocas D et al. *JAMA*. 2017 Mar 21; 317(11) Chen R et al. *JAMA*. 2017 Mar 21; 317(11).

Blood Thinner Keeps Stroke Survivors in Their Homes

Using the blood thinner warfarin helped stroke survivors reduce future hospitalizations and stay in their homes—on average 46 more days at home over two years — compared with those who didn't take the drug after being discharged from the hospital. The drug also lowered the rates of stroke recurrence and heart attack, but staying at home rather than having to go to a nursing home or hospital was the outcome that mattered most to patients. Xian Y et al. *BMJ*. 2015; 351 O'Brien EC et al. *Circulation*. 2015 Oct 13; 132 (15).

Shared Decision Making Helps Decisions Related to Advanced Heart Failure

Surgically implanting a left ventricular assist device (LVAD) can prolong the lives of people with end-stage heart failure. But the surgery and device carry significant risk for harms, such as infections and stroke. Using a shared decision-making tool improved patients' knowledge about the potential benefits and risks of an LVAD compared with typical educational pamphlets. The

aid also helped them make initial decisions better aligned with their values. Allen LA et al. *JAMA Intern Med.* 2018 Apr 1; 178(4).

Behavioral Health Homes Improve Outcomes for People with Mental Illness

Behavioral health homes, a patient-centered way of coordinating care for patients with both mental and physical health needs, can help people with serious mental illnesses manage their conditions and possibly live longer, healthier lives. Two ways of providing a behavioral health home—a patient self-directed approach and a healthcare provider-supported approach—both significantly increased patients’ knowledge and confidence to manage their own care and increased their engagement in care, but the provider-supported approach did so faster. Schuster J et al. *Health Aff.* 2018 Feb; 37(2).