August 2, 2024

The Honorable Diana DeGette
U.S. House of Representatives
2111 Rayburn House Office Building
Washington, DC 20515

The Honorable Larry Bucshon
U.S. House of Representatives
2313 Rayburn House Office Building
Washington, DC 20515

Dear Representatives DeGette and Bucshon,

On behalf of the Partnership to Improve Patient Care (PIPC), we appreciate the opportunity to comment on the recent RFI on the 21st Century Cures initiative — an important next step in efforts to advance medical research and foster a new era of medical innovation. Both 21st Century Cures and the policies included in Cures 2.0 have been positive steps forward to accelerate innovation and bring treatments to patients who desperately need them. As your offices work to shape the broader 21st Century Cures initiative, we stress that patients must be kept at the forefront of our health care system.

We are at a seminal time in medical innovation, and patient communities are filled with hope as disease modifying treatments and gene therapies come to market for diseases that have long had no treatment options. We appreciate the 21st Century Cures initiative's efforts to expedite patient access to treatments, particularly through the FDA's guidance on cell and gene therapy, expedited drug approval processes, and the integration of real-world evidence in regulatory decision-making. We urge you to build on that work and acknowledge that once a treatment is approved, there are often additional barriers that must be addressed around coverage and reimbursement. To promote affordability and access to novel treatments, we ask you to consider the following policy recommendations:

Congress should provide increased oversight of coverage and reimbursement decisions to ensure they are using transparent, nondiscriminatory methods and meaningfully engaging patients.

Medicare Negotiation

Patients are concerned that some proposals to promote value in our health care system — including reliance on third-party cost-effectiveness analyses or an International Pricing Index to determine coverage of treatments — would restrict patient access to needed therapies. In particular, the patient community is anxiously awaiting public disclosure of CMS's methodology used in negotiating the first round of drugs under the Inflation Reduction Act's (IRA) Medicare Drug Price Negotiation Program to ensure it does not rely on discriminatory methods that may unintentionally harm patients and people with disabilities. We have also strongly urged CMS to consistently engage with patients to quickly identify and address any impacts that negotiations may have on their access to care.

Medicaid and State Plans

We are increasingly concerned about state activities related to coverage and reimbursement, particularly in the context of Pharmacy and Therapeutics (P&T) Committees, Drug Utilization Review Boards (DURBs) and Prescription Drug Affordability Boards (PDABs). Feedback indicates that these boards and commissions do not consistently value feedback and input from patients, people with disabilities, caregivers, and experienced clinicians, nor do they offer robust and predictable opportunities for meaningful engagement on decisions affecting access to care. It is imperative for

patients to know when meetings will happen in advance, the questions that they should be prepared to answer, how their answers will be used to make decisions and be given both verbal and written opportunities to comment.

For example, in response to ineffective engagement strategies, patients have developed a survey for use by PDABs that authentically allows a policymaker to understand the source of affordability challenges, whether it be due to utilization management, copays, transportation, caregiving etc. Patients and people with disabilities applauded the decision by the State of Oregon's PDAB to pause its affordability reviews until 2025 in response to local advocacy. The Board explicitly recognized that it does not have an accurate definition of "affordability," and therefore is taking time to review the data collected, as well as data from the new survey developed by patients, and better use the data in its work. We hope other state PDABs will follow Oregon's lead in pausing their work to better understand its implications for patient access to care and clinicians' ability to prescribe care that will be covered by insurers. In the meantime, we urge Congress to oversee PDAB activities to ensure they do not hinder patients' access to necessary and prescribed FDA-approved drugs.

While we were very pleased to see the latest Notice of Benefits and Policy Parameters for Exchange Plans call on P&T Committees to include a patient on the committee, we also recognize that Medicaid and other plans should also be doing more to assure feedback patients and people with disabilities plays a meaningful role in their decisions. Today, P&T committees rely heavily on third party contractors to develop their preferred drug lists, and the evidentiary basis for those decisions is not publicly disclosed and is considered proprietary. Therefore, patients and people with disabilities have no means to identify the use of discriminatory value assessments that may be barred by current law — whether it be under the Affordable Care Act or the new rules governing Section 504 of the Rehabilitation Act — to file a complaint with the HHS Office for Civil Rights or otherwise. We encourage Congress to conduct rigorous oversight of state Medicaid plans, as well as P&T Committees and DURBs whose decisions may delay or deny care to patients seeking access to novel treatments approved by the FDA.

Congress should pass H.R. 485, the Protecting Health Care for All Patients Act.

We appreciate that your RFI posed the question of what additional elements are missing that are essential for further progress in advancing the goals of the 21st Century Cures Act. It is essential to extend to all federal programs the Affordable Care Act's statutory ban on Medicare's use of Quality-Adjusted Life Years (QALYs) and similar measures in coverage and reimbursement. If Congress is to advance health equity and protect patient access to the most advanced treatments, it must be a priority to protect against the use of discriminatory value assessments in deciding who gets what treatment. H.R. 485, the Protecting Health Care for All Patients Act, would create consistency in the law across federal programs. Therefore, we encourage you to support the bill as a component of your Cures initiative.

The patient and disability communities have long had concerns about the use of the discriminatory Quality-Adjusted Life Year (QALY) to determine cost effectiveness or "value" of treatments. QALYs and similar measures are referenced in other countries and in studies by third party entities, such as ICER. The National Council on Disability (NCD), an independent federal agency, concluded that QALYs place a lower value on treatments which extend the lives of people with chronic illnesses and disabilities. NCD recommended that policymakers and insurers reject QALYs as a method of measuring cost-effectiveness

for medical care and avoid referencing international pricing due to its reliance on QALYs.[1] Patients and people with disabilities also have concern about other similar metrics to the QALY, such as the equal value of life year gained (evLYG). The evLYG is a simplistic fix attempting to address criticism that the QALY devalues life years lived with a disability, yet it fails to account for oversimplified measures of quality-of-life gains in expected life years (not extended life years) and it does not account for health improvements in extended life years. Similar to the QALY, the evLYG relies on average estimates based on generic survey data and obscures important differences in patients' clinical needs and preferences, particularly those with complex diseases and from underrepresented communities.[2] It assumes people value life year gains more than quality of life improvements, thus devaluing health interventions for patient populations with lower life expectancy or fewer life years gained from treatment. This may affect people with disabilities, chronic conditions, older adults, and certain communities of color.[3]

Acting on the long-standing advocacy of patients and people with disabilities, the final updated regulations governing Section 504 of the Rehabilitation Act were published on May 9, 2024. The rule sends a strong message that we need better solutions for U.S. decision-making that do not rely on the biased, outdated standards historically used by payers. The new regulations bar health care decisions relying on measures that discount gains in life expectancy — which would include the quality-adjusted life year (QALYs) and the combined use of QALYs — and equal value of life years gained (evLYG). The agency broadly interpreted what constitutes the discriminatory use of value assessment in its description of the rule. The agency also stated that methods used in value assessment, such as their utility weights, are subject to section 504 when they are used in a way that discriminates. The 504 rule is an incredibly positive step, but we encourage the passage of H.R. 485 to ensure these protections are appropriately codified in statute and enforced consistent with current Medicare law.

Congress should protect shared decision-making between patients and providers.

With the shift towards personalized medicine, it is imperative for individualized treatment plans to be affordable to patients. As policymakers advance laws addressing affordability, we emphasize that access to care for patients and people with disabilities be at the forefront of the policy discussion. It should be a clear priority not to entrench discrimination in our health care system or to promote increased use of utilization management strategies by payers that disregard the recommended treatment of clinicians. We suggest a broad investment in more patient-centered approaches to value health care that are consistent with the goals of shared decision-making and personalized medicine. Vital decisions on health care coverage and reimbursement must involve patients and providers, who are the true subject matter experts on treatment impacts and how different subpopulations respond.

Conclusion

PIPC was established to advance principles of patient-centeredness in an evolving health system. Since its founding, PIPC advocates for the voices of patients and people with disabilities and works to ensure patients are at the table with policymakers in the development, implementation, and evaluation of alternative payment models that seek to advance value-based payment and delivery models in public

[1] https://www.ncd.gov/sites/default/files/NCD_Quality_Adjusted_Life_Report_508.pdf

[2] DiStefano MJ, Zemplenyi A, Anderson KE, Mendola ND, Nair KV, McQueen RB. Alternative approaches to measuring value: an update on innovative methods in the context of the United States Medicare drug price negotiation program. Expert Rev Pharmacoecon Outcomes Res. 2024 Feb;24(2):171-180. doi: 10.1080/14737167.2023.2283584. Epub 2024 Jan 25. PMID: 37961908.

[3] Mike Paulden, Chris Sampson, James F. O'Mahony, Eldon Spackman, Christopher McCabe, Jeff Round, Tristan Snowsill, Logical Inconsistencies in the Health Years in Total and Equal Value of Life-Years Gained,
Value in Health, Volume 27, Issue 3, 2024, Pages 356-366.

programs such as Medicare. Ultimately, our goal is to ensure that value in health care is defined as achieving outcomes that matter to patients. We look forward to collaborating with you to ensure that the next iteration of the Cures initiative is patient-centric and supports the goal of innovative treatments reaching those who need them most.

Sincerely,

The Honorable Tony Coelho
Chairman
Partnership to Improve Patient Care