August 02, 2024

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

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

Re: 21st Century Cures Act and Cures 2.0 Initiative RFI

Dear Representatives DeGette and Bucshon:

Thank you for the opportunity to provide feedback on your initiative to build on the successes of the 21st Century Cures Act and Cures 2.0 initiatives. We are deeply appreciative of the work you have done to secure wide-ranging reforms that have immeasurably benefited patients and our health care system as a whole.

California Life Sciences (CLS) represents more than 1,200 members from across the entire life sciences ecosystem in California, including early-stage innovators and startups as well as established industry leaders in the fields of biotechnology, pharmaceuticals, and medical technology. As integral components of a healthy and collaborative ecosystem, CLS works closely with universities, academic and research institutions, the investment community, and other critical partners that promote a vibrant sector.

On behalf of CLS's member companies and the patients they serve, we offer the following comments to your request for information.

Medicare Coverage for Breakthrough Devices

CLS has long supported efforts to accelerate coverage of, and seniors' access to, the latest innovative medical technologies, which have the potential to improve diagnosis and treatment, address previously untreatable conditions, and improve quality of life. For many novel technologies, a significant amount of time elapses between approval by the U.S. Food and Drug Administration (FDA) and Medicare coverage for beneficiaries. Research by the Stanford Byers Center for Biodesign found that only 44% of novel technologies authorized by the FDA between 2016 and 2019 achieved nominal Medicare coverage by the end of 2022, and the median time to achieve this nominal coverage was actually 5.7 years.[1] This not only prevents life-saving and cost efficient medical devices, technology, new therapies and diagnostic technologies from getting to beneficiaries and their physicians, even though the FDA has already determined meet the standards for safety and effectiveness required for marketing in the U.S. The uncertainty and

[1] Sexton ZA, Perl JR, Saul HR, et al. Time From Authorization by the U.S. Food and Drug Administration to Medicare Coverage for Novel Technologies. JAMA Health Forum. 2023;4(8):e232260. doi:10.1001/jamahealthforum.2023.2260.

unpredictability also acts as a disincentive to investment in medical technology development, as evidenced by findings from a previous peer-reviewed Stanford Biodesign study.[2]

We were pleased to see that, on June 22, 2023, the Centers for Medicare and Medicaid Services (CMS) publicly released its Notice on the Transitional Coverage for Emerging Technologies (TCET) pathway to establish a clear and expeditious coverage process, based on scientifically sound clinical evidence with appropriate safeguards, for emerging technologies that will benefit Medicare-eligible patients. However, the guidance does not create a new dedicated pathway for these technologies, and it excludes diagnostic tests and devices without benefit categories. The TCET notice is a positive incremental step forward and represents CMS' continuing commitment to ensuring Medicare beneficiaries have access to new and innovative technologies that improve health and outcomes. Unfortunately, the open comment period for the rule ended on August 18 of last year and the rule has still not been finalized. Because it is unclear when, or if, CMS will finalize this rule, it is critical that Congress take action to ensure that Medicare patients are not left without access to these critical breakthrough medical products.

CLS supports the work of those in Congress who understand the value of this policy by encouraging passage of the *Ensuring Access to Critical Breakthrough Products Act* (H.R. 1691), which is a legislative solution to provide automatic coverage for FDA-approved breakthrough-designated products. The bill would ensure FDA-designated breakthrough technologies are covered for four years by Medicare and creates a roadmap for additional evidence collection for CMS to make a permanent coverage decision after that period. It would also provide a pathway for breakthrough digital technologies that are otherwise not covered by Medicare.

Important Fixes to the Inflation Reduction Act

CLS is concerned that the seismic shift caused by the CMS price setting authority in the *Inflation Reduction Act* (IRA) will drive the U.S. away from the current market-based systems that underpin both Medicare Part D and Medicare Part B and will erode patient access, as well as undermine continued biopharmaceutical innovation. Many medicines today are developed because of continued research and development on an approved medicine. Researchers follow science to develop medicines for new diseases, new patient populations, and in new forms of administration. Much of this post-approval research and development happens in the later stages of a medicine's lifecycle, the very part of the lifecycle that is cut off by the timeline set forth in the IRA price controls. While we appreciate the steps that CMS has taken to establish a dialogue with key stakeholders about the Medicare Drug Price Negotiation Program (MDPNP) and other drug pricing elements of the IRA, CLS has significant concerns about the effects the implementation of this law will have on the life sciences ecosystem and companies' abilities to bring new, lifesaving medicines to patients.

Congress Should Pass the *ORPHAN Cures Act*

[2] Ruggles SW, Perl JR, Sexton ZA, Schulman K, Makower J. The Need for Accelerated Medicare Coverage of Innovative Technologies: Impact on Patient Access and the Innovation Ecosystem. Health Management, Policy and Innovation (www.HMPI.org). 2022; 7(1).

First, the structure of the Orphan Drug Exclusion in the MDPNP undermines the incentives of the Orphan Drug Act by exposing orphan drugs to price setting eligibility prematurely. The exclusion is only available when an orphan drug is designated for a singular rare disease or condition, and when FDA approval for the drug is solely within that designation. This narrow exclusion ignores the value of research often undertaken today that results in multiple designations and approvals for a product to treat multiple rare diseases.

CLS urges Congress to evaluate the impact of the Orphan Drug Exclusion and the protections needed to ensure that rare disease innovation continues to be incentivized, such as those found in the *Optimizing Research Progress Hope And New* (ORPHAN) *Cures Act* (H.R. 5539 /S. 3131). The *ORPHAN Cures Act* is designed to ensure that the Orphan Drug Exclusion from negotiation eligibility allows products to remain excluded so long as their FDA approved uses are exclusively for rare diseases as well as clarifies that the clock for negotiation eligibility starts at the date of a product's first non-rare approval.

Congress Should Pass the *EPIC Act*

Second, the IRA created a "pill penalty" that discourages the development of small molecule medicines, or drugs that typically come in simple to use forms such as a pill or capsule. The IRA does this by subjecting small molecules to government pricing nine years after FDA approval, rather than the 13 years afforded to biologics. Congress must remedy this discrepancy by considering the *Ensuring Pathways to Innovative Cures (EPIC) Act* (H.R. 7174), which would equalize the timeframe for government pricing at 13 years post-approval for both biologics and small-molecule drugs.

Additionally, the IRA exempts drugs produced by small biotech companies from price setting until 2029. In order to ensure that the exemption is workable for the small companies it was created to support, Congress should extend the exemption beyond its current end date, allow additional companies to qualify in the future, and establish a clear dispute resolution process for manufacturers.

Rather than placing additional barriers in the way of scientific progress and the development of new treatments, Congress must instead advance policies that provide incentives to future research and development.

Pharmacy Benefit Manager Reforms

Pharmacy Benefit Managers (PBMs) play a large role in determining prices, formulary placement, and medication access for patients. Unfortunately, misaligned incentives and a lack of transparency by PBMs have led to increased costs and limited patient choice. If policymakers are serious about increasing access to life-saving treatments, Congress should consider meaningful legislative PBM reforms. These reforms must go beyond simple transparency measures to address financial practices that result in higher out-of-pocket costs, that are critical to foster innovation in our health care system and ensure affordable access to vital medications.

For example, PBM compensation should not be based on the list price of medicines. Currently rebates and administrative fees that PBMs receive are calculated as a percentage of a drug's list price, leading PBMs to favor drugs with higher list prices and larger rebates so that PBMs can collect higher revenues. Congress should consider delinking PBM revenues from the cost of the medicine as proposed in the *Delinking Revenue from Unfair Gouging (DRUG) Act* (H.R. 6283/S. 1542).

Additionally, PBMs often block third-party payments provided to patients by manufacturers that would help decrease the burden of patients' copays. Some PBMs and insurers have erected barriers to patients trying to utilize manufacturer copay assistance by exploiting this assistance, preventing the copay assistance from counting towards a patient's out of pocket costs. Congress can protect patients from higher cost-sharing through solutions put forward by the *Help Ensure Lower Patient (HELP) Copays Act* (H.R. 830/S. 1375).

By addressing the misaligned incentives of PBMs and enacting meaningful reform, your constituents will have greater choice in the medicines they receive at a lower cost.

Cell & Gene Therapies

CLS applauds the attention Congress has given to better understand cell and gene therapies (CGTs) and the various steps we have seen to provide regulatory guidance to industry. CGTs have the potential to revolutionize the treatment of various diseases by targeting specific disease-causing genes or cells, allowing for personalized treatment approaches. A clear and accurate diagnosis is the first prerequisite to accessing CGTs. However, the journey to diagnosis is often long and arduous for many people living with a rare disease. On average, these patients wait approximately six years after symptoms present before receiving a confirmed diagnosis—often having received several incorrect diagnoses during that time.[3] Expanding the use of powerful diagnostic tools, such as whole genome sequencing, can help patients with rare diseases get diagnosed more quickly and effectively. Shortening the time to diagnosis can help lead to more informed decision making and speed up access to treatments like CGTs.

One improvement to patient access to transformative therapies requires examining insurance coverage policies that impose excessive cost-sharing burdens and other access barriers on patients. Too often, the most vulnerable patients with chronic or complex illness face discriminatory insurance policies that demand higher out-of-pocket costs or deny/limit coverage, damaging the patient's ability to access the most promising treatment plan. For example, some insurance plans utilize copay accumulator programs to prevent co-pay assistance from counting towards patients' deductible or out-of-pocket maximums, resulting in higher costs for patients—especially patients with chronic conditions who rely on expensive medications. Additionally, plans may employ "fail first" or "step therapy" practices that could disrupt a patient's treatment. Creating barriers to access to necessary treatment indicates a failure in current insurance paradigms and suggests a need for the development of new risk pooling models that are responsive to the rapidly evolving treatment landscape for rare disease.

[3] About Us. EveryLife Foundation for Rare Diseases. Accessed August 1, 2023, https://everylifefoundation.org/about-us/

Granting patients access to CGTs is dependent on payment systems that reflect the true value of the therapy and related ancillary services. Policymakers need to ensure that CMS provides appropriate reimbursement of innovative therapies in the context of Medicare and Medicaid. It is also important to remove barriers to federal and state adoption of value-based arrangements for CGTs. Accordingly, by their very nature, CGTs present unique reimbursement challenges, particularly for payers like Medicare. New payment innovations play a significant role in adapting to the evolving landscape of CGTs. Private sector solutions such as value-based purchasing, outcome-based arrangements, and product warranties, should be encouraged and built upon. The commercial insurance market often takes coverage and reimbursement signals from Medicare and Medicaid; therefore, policy solutions must work across all payer types.

Ensuring CMS has Adequate Expertise

CLS believes that CMS should have the ability to hire additional clinical and research experts from outside the agency as full-time CMS employees. The FDA, National Institutes of Health (NIH), Centers for Disease Control and Prevention (CDC), and Health Resources and Services Administration (HRSA) have been afforded the authority to hire candidates with doctorates or clinical and research expertise under Title 42 of the Public Health Service Act. However, CMS is governed by the Social Security Act and is therefore not subject to these expanded authorities. As a result, CMS has had difficulty competing with other federal agencies for talent. Bipartisan members of Congress supported expanding FDA's hiring authorities in the *21st Century Cures Act*, to help the agency with its responsibility to review and regulate medical products. As medical technology continues to evolve, CMS should have the same authority to hire clinical experts uniquely qualified to understand the technologies its programs cover.

Extending Certain Public Health Emergency Flexibilities

Innovative technologies can be used to protect patients, improve outcomes, relieve already stressed and burdened health care workers, and reduce health care costs. During the COVID-19 public health emergency, health care providers experienced significant disruptions to their processes for delivering care. The use of telemedicine, including remote patient monitoring, streamlined and enhanced provider workflows while also limiting emergency department visits, improving care coordination and management, and enhancing research in patient populations and disease states. These virtual technologies proved to be extremely effective tools for providing access to health care services for Medicare beneficiaries—in large part because CMS has used broad waiver authority to expand the availability of these services during the crisis.

Congress has acknowledged that certain services were needed following the end of the public health emergency as the health care system developed new processes for delivering care to patients. CLS supports making these telehealth and remote monitoring flexibilities created during the COVID-19 pandemic permanent, while simultaneously preserving the important patient-provider relationship and protecting against potential negative impacts of the "digital divide." Doing so will improve patients' ability to get care outside of doctors' offices and other traditional health care settings and save and improve countless lives.

Developing Antimicrobial Innovations

CLS appreciates the continued Congressional attention in addressing the growing public health crisis of drug-resistant bacterial and fungal infections, a phenomenon known as antimicrobial resistance (AMR). Overuse and misuse of current antimicrobials accelerates AMR, and a lack of new antimicrobials jeopardizes medical advances that depend on antibiotics to help fight infections, including joint replacements, organ transplants, and cancer therapies. We need a consistent stream of new antimicrobial treatments to catch and keep up with resistance. But the pipeline for antimicrobial medicines is lacking due to misaligned incentives.

To preserve these drugs' effectiveness, antibiotics are, inherently, used sparingly. Clinicians only prescribe them when appropriate and for short periods so as not exacerbate AMR. That means the market for antimicrobials differs from other types of medications and do not have the same market incentives in place to justify the financial risks companies take to invent new medicines. The bipartisan *Pioneering Antimicrobial Subscriptions to End Upsurging Resistance (PASTEUR) Act* (H.R. 2940/S. 1355) would revive the American antimicrobial industry by implementing subscription-style contracts for new treatments that address the most threatening infections.

Instead of paying companies per sales volume, the government would contract with firms for a reliable supply of a new drug, regardless of how much of the medicine was required—incentivizing the development of novel antimicrobials based upon the value they provide for public health, rather than the volume used.[4] Payments that are decoupled from sales volume provide a predictable return on investment for antimicrobial developers. This model would revitalize antimicrobial research and development and ensure patients' access to novel treatments while supporting their appropriate use. Importantly, the federal government only pays once, since the subscription payment is all-inclusive, and only supports antimicrobials that meet unmet AMR needs. CLS believes that the *PASTEUR Act* will help ensure a robust and diverse pipeline of treatments to tackle the growing threat of AMR.

Reauthorizing the Rare Pediatric Disease Priority Review Voucher Program

CLS appreciates Congressional attention towards supporting regulatory incentives for underserved patient populations and believes that it's imperative for the Rare Pediatric Disease Priority Review Voucher (RPD PRV) program to be reauthorized. Since its creation by Congress in 2012, the RPD PRV program has helped spur rare disease drug development in pediatric populations and brought therapies to market for children affected by almost 40 rare diseases.[5] Many of these diseases led to death or debilitating illness before the children reach adulthood, and almost none had any safe and effective FDA-approved therapies on the market before the program began. With more than 95% of rare diseases still lacking an FDA approved therapy, the RPD PRV program is important to patients and a source of hope for the future development of

[4] https://www.pharmaceutical-technology.com/features/fighting-amr-with-a-value-and-subscription-model/#catfish
[5] https://rarediseases.org/wp-content/uploads/2024/05/NORD_PRV-white-paper_FINAL.pdf

safe and effective treatments. Without timely reauthorization, FDA will no longer be allowed to initiate the process necessary to issue new RPD PRVs.

HHS Public-Private Partnerships to Accelerate Diagnostic Solutions for Public Health

According to the CDC, 70% of today's medical decisions depend on diagnostic test results.[6] While diagnostic tests and services are essential, patient access barriers still exist, leading to missed or incorrect diagnosis and treatment. As outlined in the World Health Organization 2023 Essential Diagnostics List, access to diagnostics within health care facilities with clinical laboratories as well as community settings and health facilities without laboratories is critical for the timely and accurate identification of diseases or health conditions.[7]

To overcome access barriers and expedite critical diagnostic tests and solutions for patients, HHS should utilize effective public-private partnership models developed during the COVID-19 response. On April 29, 2020, the NIH launched the Rapid Acceleration of Diagnostics (RADx) Initiative to speed innovation in the development, commercialization, and implementation of technologies for COVID-19 testing. RADx then established the Independent Test Assessment Program (ITAP) to accelerate regulatory review and availability of high-quality, accurate, and reliable diagnostic tests. RADx works with diagnostic testing manufactures and multiple federal agencies to streamline and accelerate the development and approval processes. The RADx and ITAP initiatives proved to be highly successful public-private partnerships that significantly reduced the time required to get high quality COVID-19 tests to health care facilities, laboratories, and patients.

Since the pandemic, the RADx/ITAP programs have successfully worked with private sector partners to facilitate the availability of diagnostics tests for other infectious diseases, including an Mpox test and a Flu A/B and Covid Multiplex test. RADx and ITAP should be leveraged further to promote the development and approval of novel diagnostic tests needed within other areas of public health, such as antibiotic resistant infections, respiratory infections, and sexually transmitted infections. Therefore, Congress should authorize further funding for the NIH RADx and ITAP programs to accelerate critical testing for public health and reduce patient access barriers.

Enacting SALSA to Stop Further Cuts to Clinical Lab Services

A robust laboratory infrastructure across the country is essential and clinical testing plays a critical role in healthcare. Congress passed the Protecting Access to Medicare Act (PAMA) in 2014. The legislation was designed to align Medicare payment for clinical labs with prevailing market rates across the country. Unfortunately, the first round of market data was collected from less than one percent of the nation's laboratories – far from representative of market rates. Flawed implementation has led to projected reductions in Medicare reimbursement for laboratory

[6] https://www.cdc.gov/csels/dls/strengthening-clinical-labs.html
[7] https://www.who.int/news/item/19-10-2023-who-releases-new-list-of-essential-diagnostics--new-recommendations-for-hepatitis-e-virus-tests--personal-use-glucose-meters

services by $10 billion, far eclipsing the $2.5 billion in cuts originally presumed by the Congressional Budget Office.

The current policy implementing PAMA has led to rounds of dramatic Medicare reductions for most diagnostic tests, and the next reductions are set to begin January 1, 2025. Without congressional intervention this year, laboratories across the country will face tough decisions potentially reducing services offered to patients and curbing investment in the next generation of diagnostic tests. Therefore, CLS supports the *Saving Access to Laboratory Services Act (SALSA)* (H.R. 2377/S. 100), and recommends legislative action be taken now to mitigate further Medicare cuts to laboratories through improved PAMA implementation and modernization of the Clinical Laboratory Fee Schedule to safeguard a robust national testing infrastructure.

Medical Device Electronic Labeling

CLS supports the broader application of electronic labeling for all medical devices and drug device combination products. Current law recognizes that medical device labeling, including instructions for use, may be provided electronically for a wide range of devices—including all prescription devices for use in health care facilities or by health care professionals, as well as other drug device combination products and in vitro diagnostic devices for use by health care professionals or in blood establishments. Other technologies, such as those used primarily in the home setting, require hard-copy, paper labeling and instructions for use for the device and associated supplies.

Congress should consider modernizing the *Food, Drug, and Cosmetic Act* to provide for electronic labeling for all medical devices, regardless of setting of use or the intended user, such as what is considered in the *Medical Device Electronic Labeling Act* (H.R. 3723). This bill would maintain requirements for device manufacturers to provide paper labeling and instructions for use when requested and at no cost, such as in cases where a user has limited or no internet access, or simply prefers hard copy versions.

Medical Device Support for Opioid Safety and Recovery

Medical devices can close safety gaps in opioid management and reduce harm by enabling physicians to prescribe the medications that they deem appropriate to manage pain while also keeping their patients safe. CLS recommends expanding access to FDA-cleared medical devices to help prevent overdose and significantly reduce opioid withdrawal symptoms, through incentives, coverage and reimbursement, pilot programs, and education. To achieve this goal, Congress should require the Substance Abuse and Mental Health Services Administration (SAMHSA) to publicly provide clarification, through letters and informational guidance, regarding the use of grant funding to assure grant recipients that funds can be used to purchase FDA-cleared medical devices. Further, Congress could consider providing a dedicated coverage and reimbursement pathway for innovative medical devices that increase opioid safety and assist in opioid withdrawal.

Providing New Process Flexibilities for the U.S. Preventive Services Task Force

The U.S. Preventive Services Task Force (USPSTF) is an independent group of national experts in prevention and evidence-based medicine that works to improve the health of all Americans by making evidence-based recommendations about clinical preventive services such as cancer screenings. “A” or “B” recommendations for a preventive service from the USPSTF ensures that all individual and group health plans provide the service at no cost to the patient. As a result, USPSTF recommendations are critical drivers of patient access and adoption of preventive services. Under its current structure, the USPSTF aims to review and update existing recommendations every five years. However, delays are common, resulting in significant lag time between the availability of new screening technologies and review by the USPSTF due to their complexity and cost. Delays in recommendations disproportionately impact low-income and historically underserved populations that may be unable to pay out-of-pocket for crucial preventive services. While the USPSTF has mechanisms that allow for early review, this rarely occurs, due to resource constraints.

In the 117th Congress, the *Preventive Services Early Action Act* (H.R. 9310) was introduced, which would enhance the ongoing scientific review process to broaden the sources considered, empower the USPSTF to modify a portion of a recommendation, improve the early action process, increase transparency, and create a process to quickly consider newly FDA approved screening modalities. CLS believes that this legislation is an important first step in elevating the conversation around USPSTF modernization, and we encourage more work be done to support and accelerate the work of the USPSTF.

The recent *Braidwood Management Inc. v. Becerra* decision struck down the *Affordable Care Act’s* requirement that preventive services receiving an A or B rating from the USPSTF be covered with no cost sharing for patients. Regardless of the ultimate outcome of this case, the USPSTF will still review and make recommendations on the preventive services that Americans should receive, a role it has filled going back to its creation in the 1980s. Physicians rely heavily on the recommendations published by the USPSTF, meaning that patient access to new screening tools is heavily reliant on the speed and outcomes of USPSTF recommendations. Making legislative changes to the USPSTF review process will not only help patients but also will not impact the outcome of the Braidwood Case, because the legislation does not change the coverage mandate nor the independence portions of the USPSTF statute.

Forums for Expert Driven Drug Development

Many meaningful initiatives have been established by FDA to accelerate rare disease drug development. However, there remains a lack of structured forums to systematically obtain scientific alignment from experts on drug development for specific diseases. It is critical that the nuances of rare diseases are understood to inform regulatory decisions, particularly for smaller rare diseases without precedent and a development path. A new forum is needed to facilitate earlier discussion and education about specific rare diseases or groupings of rare diseases between FDA leaders, reviewers, scientific & medical experts, industry, and patient representatives.

Congress could consider directing the FDA to conduct a new, informal meeting type to allow developers, regulators, scientific and academic experts, and patient advocates to proactively and systematically discuss drug development considerations in rare diseases. The objectives of the forum would be to share disease expertise and preclinical and clinical perspectives, identify approaches to study design including duration and intended population across modalities, address regulatory acceptance of biomarkers and novel surrogate endpoints, and understand clinically meaningful outcomes for patients to support drug development. Such meetings would be funded by external stakeholders, with no cost to the FDA beyond participation and close collaboration, and develop a structured format for scientific alignment and education on rare diseases.

Thank you again for the opportunity to respond to your request for information. CLS is a willing participant in advancing more effective policy solutions to address patient access and affordability challenges. We welcome any questions and further discussion on the topics above, and you can contact me at bfisk@califesciences.org.

Sincerely,

Brent Fisk

Brent Fisk
Senior Vice President, Government Relations & External Affairs
California Life Sciences