RAAP
RARE ACCESS ACTION PROJECT

The Honorable Diana DeGette
The Honorable Fred Upton
United States House of Representatives
Washington, DC 20515

December 11, 2019

Dear Congresswoman DeGette and Congressman Upton:

Thank you both for your leadership in providing a foundation for much needed therapeutic innovation in the 21st century. We applaud your efforts and reaching out to stakeholders to solicit comments and feedback on issues that can build on the work of the original CURES Act, a framework focused on enhancing the tools, data and pathways for approval of novel therapies. Your work continues to make possible exploration into very rare diseases, which have been in need of hope. We applaud both your personal dedication to this effort, and the bipartisan approach to now bringing areas of access into the 21st century.

Situation

According to the Food and Drug Administration, "an orphan disease is defined as a condition that affects fewer than 200,000 people nationwide. Some diseases have patient populations of fewer than a hundred. Collectively, however, they affect as many as 25 million Americans, according to the National Institutes of Health (NIH), and that makes the diseases—and finding treatments for them—a serious public health concern."

Many advances have been made in rare disease treatment, with cures now within reach through new technologies such as cell and gene therapies. These advances were spurred by bipartisan commitment to finding treatments for rare diseases, of which there are over 7,000 and over 50 percent of rare diseases affect children. During the first 25 years of the Orphan Drug Act (passed in 1983), 326 new drugs were approved by the FDA and brought to market for all rare disease patients combined.

However, with major innovations on the horizon, including the promise of cell and gene therapies, the health care system in the United States has been slow in preparing and even slower in making those advances available to patients once

approved by the US Food and Drug Administration (FDA). We must not only continue to advance therapies through discovery and development, but we must also make certain that patients ultimately have access to them post-approval. As you pointed out in your background document, structural barriers in both Medicare and Medicaid can cause a delay in not just a few days or months post the approval of a new rare disease medicine. Some therapies are seeing delays beyond two years. And yet there are recommendations that we believe will lead to improved access, affordability and outcomes for rare disease patients.

As you know, the rare disease landscape is very diverse with innovation being driven by family foundations, educational consortium, start up private equity labs and ventures, pre-market companies with innovation rich pipeline products with no products on the market, as well as emerging companies with one or two small population medicines. The research is being driven in organizations as diverse as the diseases they intend to treat. However, access challenges put those efforts at risk, for raising capital and resources can also be a challenge as pioneering innovators must convince investors, foundations and the market that there could actually be a business case for very small population medicines.

RAAP is a coalition that explores creative solutions to address structural issues and payment policy issues in access and coverage to help ensure rare disease patients have access to the care and treatments that they need.

1. Rare Patient and Specialist Voice in Program Decision Making

During the previous work on 21st Century Cures, you focused on bringing the patient voice earlier and more firmly into research, development and ultimately the approval process. We again applaud that bipartisan approach to decision making that brought patient and community understanding to the table, and has fostered innovations in exploration of rare diseases, under standing natural history deficiencies and offering solutions in understanding onset of disease, patient experience or journey, and calibrating outcomes to capture data meaningful to the payers, health care professionals, patients and families, etc.

At the Rare Access Action Project, we believe it is now time to bring post approval decision making into alignment with that patient centric approach. The CURES 2.0 effort is an opportunity to bring rare patients and specialists into Medicaid and Medicare decision-making processes both at the federal and state levels. RAAP supports inclusion of rare patient input into payment and access reviews, deliberations and decision-making. All too often, a new rare medication is reviewed, access criteria are developed, and final decision-making includes very little input from patients or rare disease specialists and centers of excellence. A higher level of expertise will needed and patients are actively honing their expertise and

understanding during research and development leaving them prepared to help programs understand therapies, coverage criteria, and the intersection with the patient journey.

Further, as you know, patients with rare disease may also be disabled, so their participation as members of state Medicaid decision making processes must be structured to allow their expertise, experience and understanding of newly approved therapies to be actively at the table throughout all program deliberations and decision making. Notifications to them must be timely (for example New York state provides a 30 day notice, which is not observed uniformly by other states) and transparent to allow for scheduling, arranging transportation, and ensuring participation. Further, meetings should be constructed to ensure communications and deliberations accommodate patient needs and even specialist participation, such as use of web and phone based technology. And patients should be more than window-dressing within the Medicaid deliberative process. They should have an expectation of participation, as a member of the committee, with a voice and a vote so that program final decisions reflects patient and specialist expertise with a rare therapy.

2. Imperative for Patient Access

The federal Medicaid rebate statute requires that, as a condition of Medicaid coverage, drug manufacturers pay rebates on Medicaid fee-for-service (FFS) and managed care utilization of their "covered outpatient drugs". In exchange for these rebates, Medicaid programs must cover the manufacturer's outpatient drugs according to the drug's "medically accepted indication," or in other words the FDA approved indication.[1] While many states contract with managed care organizations (MCOs) to provide coverage for all or part of their Medicaid populations, the Centers for Medicare and Medicaid Services (CMS) requires that these MCOs provide coverage according to the standards set in the Medicaid rebate statute.[2]

Over the years, CMS has reminded Medicaid programs of drug coverage obligations through technical guidance due to ongoing patient access challenges. For example, on June 27, 2018, CMS reminded programs that drugs approved under FDA's accelerated approval pathway, which expedite access to novel therapies that fill an unmet medical need[3], meet the definition of a covered outpatient drug and must be

[1] SSA § 1927(k)(6)
[2] 42 CFR .§ 438.3(s)(1)
[3] "[F]our FDA programs are intended to facilitate and expedite development and review of new drugs to address unmet medical need in the treatment of a serious or life-threatening condition: fast track designation, breakthrough therapy designation, accelerated approval, and priority review designation." FDA, Guidance for Industry,

covered.[4] Further, CMS currently has authority under the Social Security Act, §1904, to enforce compliance, however more enforcement authority may be necessary to ensure CMS feels compelled to engage states and ensure patient access. .

In addition, some Medicaid programs employ a review process that can take between 180 to 365 days (or longer) to conclude, before there is any real access to a rare disease medicine. This is unacceptable when many of those rare diseases are life threatening, and patients, many of whom are pediatric, do not have the luxury of time to wait for Medicaid to make a product available after a year or two of delay, while their disease advances. Rather, FDA-approved drugs with a rebate agreement must be covered from the outset; there is no proscribed legal period to delay coverage based on the theory that a state must review it or make a coverage determination. So, while a state may review a drug through its P&T process, that drug must be made available prior to and post review according to its medically accepted indication. CMS included guidance regarding these statutory obligations in the preamble to its 2016 Medicaid managed care organization (MCO) final rule, making clear the imperative for immediate coverage of new drugs of a manufacturer with a rebate agreement.[5]

Medicaid programs, through P&T processes, may impose prior authorization requirements on drugs, provided they respond to requests within 24 hours and dispense a 72-hour supply of the drug in an emergency.[6] However, prior authorization cannot be used to deny coverage for a drug's medically accepted indication, including its FDA-approved indication. Further, federal law does not

---

Expedited Programs for Serious Conditions -Drugs and Biologicals (May 2014) (Expedited Pathways Guidance), at 1.

[4] CMS Medicaid Drug Rebate Program Notice, Release No. 185. State Medicaid Coverage of Drugs Approved by the FDA under Accelerated Approval Pathway. June 27, 2018. Available at: https://www.medicaid.gov/medicaid-chip-program-information/by-topics/prescription-drugs/downloads/rx-releases/state-releases/state-rel-185.pdf

[5] 81 Fed. Reg. 27498, 27551 (May 6, 2016).

[6] SSA § 1927(t)(1)(A)(5). States may also create Preferred Drug Lists (PDLs), lists of drugs that are not subject to prior authorization, and may seek supplemental rebates for PDL inclusion. CMS recently explained, "In general, [Medicaid beneficiaries] may not be denied access to covered outpatient drugs of manufacturers participating in the drug rebate program when such drugs are prescribed for a medically accepted indication. However, to determine whether the drug is prescribed for a medically accepted indication for the individual, the state or managed care plan may subject any covered outpatient drug to prior authorization as long as the prior authorization program meets the minimum requirements at section 1927(4)(5) of the Act." 81 Fed. Reg. 27498, 27553 (May 6, 2016).

permit a program to deny access to medically accepted indications of covered drugs based on state medical necessity laws, regulations, coverage determinations or the use of other utilization tools.

Critical to this entire recommendation is ensuring the Medicaid fee-for-service program publishes a coverage policy (aligned to medically accepted indication) so that the Medicaid Managed Care Organizations follow accordingly as required. As you are likely aware, currently, not all state fee-for-service programs publish their coverage policies or don't even create those policies, which then leaves the managed care organizations with no guidance. With no state guidance, the, MCOs will create their own coverage policies that are not always in compliance with federal statute. If the FFS program published a policy then the managed care organizations would be required to follow, offering equity across the state. Because of this current environment, both coverage and access varies from MCO to MCO within a single state.

And finally, the quality-adjusted life year or quality-adjusted life-year (**QALY**) is a generic measure of disease burden, including both the quality and the quantity of life lived. QALY is used in economic evaluation to assess the value for money of medical interventions. However, in the report entitled, ***Quality Adjusted Life Years and the Devaluation of Life with Disability,*** the National Council on Disability points out that the use of a QALY in coverage decisions as it is inherently bias and discriminates against patients with disabilities, which violates the Americans with Disabilities Act (ADA). We agree that new tools are required to assess impact, particularly in rare disease populations where both disability and very small numbers of patients affected by the disease will continue to be challenges not adequately addressed through the use of QALY in coverage decision making.

3. Reinsurance

In addition, cutting edge innovation requires new ways of thinking about payment and access. Value based pricing and, in the cases of one-time gene and CAR-T therapies, extended contracting, will require innovation in State and Federal payment policies which bring performance into our 21st century health system. However, State Medicaid programs have shared that their concerns with new innovations requires solutions to allow both access and affordability. This is an area where CURES 2.0 can once again innovate by creating a pathway for state Medicaid to purchase and utilize commercial reinsurance that would allow for States to build more certainty into their planning, enhance their ability to negotiate with managed care, and spread the risk of cost overruns for innovative medicines.

4. 21st Century Payment and Management Tools

New and innovative therapies will require new tools for payment and access. For many rare disease therapies, not just cell and gene therapies, an important approach would be to enable to the use of value based pricing. As with reinsurance, it allows the risk and the costs to be spread among stakeholders, with the use of performance, endurance and other related data metrics to determine the value and cost. Oklahoma and other states are already taking steps to deploy value based thinking and negotiations with manufacturers. Such efforts should be encouraged, providing states with a pathway designed to reduce barriers, and encourages both better understanding of the therapy and it's measurable impact. When programs are then provided flexibility for extended cell and gene therapy payment, coupled with commercial rare disease reinsurance, the programs will have an innovative and modern toolkit from which to manage access and risk.

We believe that an additional area of reform would be to untie rare cell and gene therapies from the diagnostic related grouping (DRG) system that bundles together therapies with services provided by hospitals, physicians and tests. Navigating the pass through process often leaves patients waiting in uncertainty because these new technologies, which are potentially curative, that are thrust in DRG codes, which are not structured to allow for the additional costs. Preparing this system for innovations will require bold steps such as this.

5. Medicare Part D Reform

As you know, when Medicare Part D was designed, no one envisioned the revolution that would occur in the treatment of rare diseases over the subsequent fifteen years since its passage and implementation. You will recall that copayments were instituted to ensure that patients were invested in their care and drug selection, and a five percent copayment was not considered a barrier. Fast forward to 2019 and there are now medicines that rare disease patients utilize in the outpatient setting. And the reality is that often there is still no or only one treatment option thus making a copayment for rare patients an unnecessary burden. Many adult rare disease patients will endure years of misdiagnosis during their patient journey, only to find once they are properly diagnosed that the rare disease medication is out of reach because of the copayment in Medicare Part D has placed it out of reach.

As we have seen, addressing the catastrophic copayment in Medicare Part D is a critical priority not just of this Congress, but also for rare disease patients who by and large also have fixed incomes. We believe that there are some reforms that could be implemented that would strengthen the successful program.

By simply capping out-of-pocket expenses for rare disease patients, Congress could provide immediate benefits. RAAP has advocated that the Gap Coverage Discount Program be amended so that rare disease companies can cover the 5 percent of patient catastrophic out-of-pocket costs. However, more stakeholders can be enlisted to help, including those that manufacture generic medicines that are exempt in current proposals from contributing to this effort to help the elderly and disabled. Coupling such a policy with rebalancing the federal subsidy would encourage plans to continue negotiating lower costs thereby both access and quality.

Further, spreading the out-of-pocket cost over a year, rather than paying a lump sum at the beginning of the year, could achieve additional financial relief for rare disease patients, as well as non-rare patients who rely on specialty medicines.

Conclusion

On behalf of RAAP member patient and life sciences organizations, we appreciate the opportunity to provide these comments to you for consideration within a CURES 2.0 initiative. We believe that now is the time to address bringing our reimbursement and access programs into the 21st century through exploring methods needed to support not just current therapies, but also the next generations of therapies on the horizon. As you know, rare therapies and patient access are on the cutting edge of this revolution, and if we are not looking at challenges with a fresh, innovative perspective, we will continue to bring forward 20th century tools that are not appropriate, and inadequate to advance innovation, balance access with value and performance, and be ill prepared for the exciting therapies of the future.

Thank you again for your consideration. We look forward to working with you and your staff in this process and are committed to designing 21st century access that is responsive to patients and prepare for future innovations that we can only imagine at this time.

Sincerely,

Michael Eging
Executive Director
Rare Access Action Project