July 16, 2020

**VIA ELECTRONIC DELIVERY**

The Honorable Seema Verma  
Administrator  
Centers for Medicare & Medicaid Services  
Department of Health and Human Services  
Attention: CMS–2482–P  
P.O. Box 8016  
Baltimore, MD 21244–8010

**RE:** Establishing Minimum Standards in Medicaid State Drug Utilization Review and Supporting Value-Based Purchasing for Drugs Covered in Medicaid, Revising Medicaid Drug Rebate and Third Party Liability Requirements [CMS-2482-P]

Dear Administrator Verma,

On behalf of patients impacted by rare diseases, the EveryLife Foundation for Rare Diseases is pleased to offer the following comments regarding the Center for Medicare and Medicaid Services’ (CMS) proposal to advance value-based purchasing (VBP) programs for prescription drugs by addressing best price reporting and other issues under the Medicaid drug rebate program. The EveryLife Foundation for Rare Diseases is a 501(c)(3) nonprofit, nonpartisan organization dedicated to empowering the rare disease patient community to advocate for impactful, science-driven legislation and policy that advances the equitable development of and access to lifesaving diagnoses, treatments and cures.

The most recent estimates suggest there are about 7,000 rare diseases. While the majority of rare diseases (93%) lack any FDA-approved disease-modifying therapies, we are grateful for recent breakthroughs that have extended the life expectancies of once-fatal conditions and we are optimistic about the research that is ongoing to develop treatments for additional conditions. For more than a decade, the rare disease patient community has played a significant role in the paradigm shifts and statutory changes that have occurred with respect to the inclusion of patients and patient experience data within clinical trial design and regulatory review. Yet, only recently have patient communities begun to apply this similar level of engagement to the access environment; engagement that often begins when patients learn that a long-awaited FDA approved product has launched into an unfavorable access environment. For patients, these access barriers and delays translate into irreversible disease...
progression and – in some instances – pediatric patients aging beyond the labeling requirements before life-saving access is granted. Potentially transformative new therapies, such as gene and cell therapies, promise life-altering improvements in patient health. But these innovative therapies also present complex new challenges for payers, and for patients and their families related to coverage and costs of care.

We are pleased to see CMS recognize the need to advance development of VBP initiatives, including by addressing operational issues within the Medicaid program that impact and may be hindering entering into such arrangements. We support the overarching policy goals and objectives within the proposal, particularly given recent and anticipated Food and Drug Administration (FDA) approvals of therapies that address unmet medical needs of those impacted by rare diseases and disorders.

At the same time, we recognize the complexity of what is being proposed and would like to see additional details and clarity prior to finalization of any model. We are particularly interested in how what is being proposed may impact beneficiary access to novel therapies for rare diseases and disorders, particularly given situations in which only a single FDA-approved therapy exists for any given disease. We are particularly interested in seeing further refinement and definition of evidence-based and outcomes-based measures to inform VBP arrangements that recognize the unique nature of therapies to treat rare diseases. These issues include the paucity of natural history data for many rare diseases, signals of efficacy that may increase over time and instances in which an efficacious treatment may slow or stop disease progression - but the patient may still have significant medical needs and costs.

Ultimately, we need to ensure that the needs of rare disease patients are protected while allowing for the advancement of value-based purchasing arrangements in Medicaid and beyond.

Below, please find additional comments on the proposal.

Medicaid and Patients with Rare Diseases

As noted above, the Medicaid program is of high importance to the rare disease community and other populations with complex health conditions or disabilities. To put this in perspective, data from the Kaiser Family Foundation indicates that about half (50%) of children with special health care needs – a category that includes rare diseases and disorders – are covered by
Medicaid or the Children’s Health Insurance Program (CHIP). This population includes beneficiaries whose family income is above traditional Medicaid eligible thresholds but who qualify for the program because of their health status. In these cases, Medicaid supplements commercial health insurance, particularly by addressing needs that may not be covered or adequately covered by other payers.

The Rare Disease Population More Broadly

While the FDA definition of rare disease means a condition that affects fewer than 200,000 Americans, collectively the population of Americans with a rare disease is estimated to exceed 30 million people or about 1/10th of the population. Beyond Medicaid, patients with a rare disease are covered by an array of health insurance products. This includes other public programs such as Medicare and Tricare and commercial plans including products in the group and individual marketplaces. Given this heterogeneity of insurance products and the application of the proposal to people in both Medicaid and non-Medicaid plans, it is of high importance that we fully understand both the potential opportunities and the challenges associated with any VBP proposal.

Health Insurance Coverage of Novel Therapies to Treat Rare Diseases

Coverage of novel therapies to treat rare diseases and conditions by all payers, including Medicaid programs, has been a very important issue to the rare disease community in recent years. While most rare diseases lack an FDA-approved, disease-modifying therapy, scientific breakthroughs over the past decade have led to treatments for some forms or mutations of rare diseases. These include subsets of Duchenne muscular dystrophy (DMD), Spinal Muscular Atrophy (SMA), Amyotrophic Lateral Sclerosis (ALS) and Cystic Fibrosis (CF). Many of these treatments have been approved via the Accelerated Approval Pathway based on surrogate endpoints, forcing patients to expend significant energies and resources in working with state Medicaid programs and Medicaid Managed Care Organizations (MCOs) and commercial plans to inform the outcomes upon which access would be based.

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One such example includes Duchenne muscular dystrophy, a fatal genetic disorder characterized by the progressive loss of muscle, that has experienced three FDA approvals in the past four years, providing a great deal of hope for patients. Since the first FDA approval, the patient community has worked to convene key stakeholder groups along with public and private payers to work collaboratively toward building consensus on the data needed to ensure access to approved therapies. In 2017, key neuromuscular specialists in Duchenne in the state of California worked directly with California Medicaid to agree upon a rationale for treatment across the spectrum of the disease, regardless of ambulation status, proposing a reasonable set of outcomes\(^2\) to be collected in order to assess the impact of therapy over time.

The EveryLife Foundation recognizes the current and looming challenges the costs of novel therapies will pose to payers, particularly public payers like Medicaid. Because of this reality, we recognize and appreciate the need to drive innovation in access policies and believe VBP models, when properly crafted, offer superior benefits to patients compared to blunt instrument approaches that seek to limit costs by limiting access. It is our hope that VBP initiatives, including several state supplemental rebate models that allow for additional rebates if certain measures are not achieved, will ultimately benefit both the patient at the micro level and our healthcare system overall.

Developing a VBP Framework for Rare Disease Therapies – Metrics, Measures & Personalized Medicine

As noted previously, the EveryLife Foundation supports the overarching concept of developing a pathway within the Medicaid program to enable implementation of additional VBP models. We recognize the complexity of the Medicaid “best price” requirements and how the policy could hinder more aggressive development of VBP programs. We are intrigued by the ideas put forth by CMS to address these challenges, particularly allowing for the use of multiple price points, including those under VBP arrangements and those not under a VBP model, as well as a blended or bundled approach to establish a best price.

However, given the complexity of this issue particularly in the area of metrics, we encourage CMS to give further consideration and definition to how such a proposal would be operationalized. As we noted in our introductory comments, a paramount concern for rare disease stakeholders lies in how VBP arrangements will be defined, including the types of data

that will inform evidence-based and outcomes-based approaches. In further refining this definition, we urge CMS to consider the following:

- Recognize that for rare diseases in particularly, there are often limited evidence-based measures given limited natural history data, small patient populations and other challenges.

- Recognize the highly personalized nature of many therapies for rare diseases which are often indicated for a genetic subtype of an already small population.

- In establishing linkages between payments and evidence or outcomes-based measures, recognize that for many patients with a rare disease, even a successful therapy, such as one that slows or stops disease progression, may still leave the patient with significant medical needs and corresponding costs.

- Recognize and speak to the role that outcomes measures developed by – and in partnership with - patients (Patient-Reported Outcomes) should play in informing VBP arrangements. This should include a specific reference to PROs and other patient-developed metrics as part of any VBP models.

- Recognize that many innovative rare disease products are being developed via the Accelerated Approval Pathway, and thus limited clinical data will be available at the time of regulatory review and approval.

- Recognize that the lack of a VBP arrangement should not be a justification for a payer to deny coverage of an FDA-approved gene therapy. VBP arrangements should serve as additional tool to inform payer decision-making, and not an additional access hurdle for innovative therapies.

By recognizing and speaking to these points in the final rule, CMS can help provide the additional clarity the rare disease community needs to see to more fully understand the ramifications of what is being proposed. We are particularly interested in seeing a recognition of the growing role patient-developed and patient-informed measures should play in informing such arrangements. Over the past decade, Congress and the FDA have moved decisively forward with policies to allow for a strengthening of the patient voice in the development and review of medical products. We believe strongly that this commitment to patient engagement needs to extend into coverage policies and that the final VBP rule should recognize this.
Conclusion

The EveryLife Foundation applauds CMS for recognizing the need for further regulatory clarity within the Medicaid program to enable more widespread movement toward VBP models. We offer our comments with the hope of achieving greater clarity and definition that is of high importance to the rare disease patient community. We support the overarching policy goals and objectives within the proposal, particularly in light of the recent and anticipated Food and Drug Administration (FDA) approvals of therapies that address unmet medical needs of those impacted by rare diseases and disorders. Ultimately, we need to ensure that the needs of rare disease patients are protected while allowing for the advancement of value-based purchasing arrangements in Medicaid and beyond.

We thank you for this opportunity. The EveryLife Foundation would be happy to serve as a resource or to facilitate engagement with rare disease community partners; please contact Annie Kennedy, Chief of Policy & Advocacy at akennedy@everylifefoundation.org.

Sincerely,

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CC: Mark Dant, Board Chairman
     Frank J. Sasinowski, Board Vice-Chairman