

BY ELECTRONIC DELIVERY

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RE: Medicare Program: CY 2018 Updates to the Quality Payment Program
CMS-5522-P

The undersigned organizations represent diverse stakeholders, including life sciences companies and patient advocacy organizations, with a shared commitment to developing and ensuring access to treatments for the subset of rare disorders that impact extremely small patient populations. We appreciate the opportunity to offer our comments to the Centers for Medicare & Medicaid Services' (CMS) Proposed Rule updating the Quality Payment Program (QPP) for the 2018 performance year (Proposed Rule).

We appreciate CMS' outreach efforts and stated commitment to devising creative ways of improving the Medicare program toward patient-centered care. Our comments provide a brief summary of the challenges patients with rare diseases face, as well as a discussion of the inherent difficulties in capturing quality care for individuals with ultra-rare diseases in the QPP. Collectively, we are committed to preserving, and building upon the innovation-driving environment enabled by the Orphan Drug Act of 1983 (ODA), and offer the following comments as the Agency continues to refine the Quality Payment Program:

- We support CMS' weighting of the cost performance category at zero percent for performance year 2018, and urge the Agency to devise a structural exception from MIPS scoring for costs associated with diagnosing and treating ultra-rare disorders in future years;
- We urge CMS to (i) develop measures and improvement activities that reflect quality care for rare diseases in the Merit-Based Incentive Payment System (MIPS), (ii) enable clinicians to earn MIPS bonus points for diagnosing and/or appropriately managing and treating patients with ultra-rare disorders, and (iii) develop an outlier-styled mechanism to account for ultra-rare disorder diagnosis and treatment costs under Advanced Alternative Payment Models;

- We urge CMS to reverse its proposed policy of applying payment adjustments to Part B drug costs, or, at a minimum, exempt orphan drugs for ultra-rare conditions from that policy;
- We appreciate that CMS recognizes the need to ensure that clinicians treating Medicare’s sickest patients are not penalized. We support the proposed complex patient bonus under the MIPS, and urge CMS to implement a presumption that patients with ultra-rare disorders are “complex;” and
- We support the Proposed Rule’s focus on flexibility and reduced clinician burden, and urge CMS to utilize this transition to ensure that the QPP incentivizes high-quality care for patients with ultra-rare disorders.

Background

Congress drafted the ODA’s incentive framework to counter the commercial realities associated with research and development toward treatments for serious medical conditions affecting small populations. During the ten years preceding the ODA, just 10 rare disease products had obtained FDA approval; since the ODA’s implementation, over 600 rare disease drugs and biologics have been developed. Countless lives have been improved, or saved by new therapies spurred by the ODA, however, millions of Americans affected by a rare disease are still waiting and hoping for treatment or a cure:

- Of the approximately 7,000 rare diseases identified to date, 95% have no FDA-approved treatment option;
- 80% of rare diseases are genetic in origin, and present throughout a person’s life, even if symptoms are not immediately apparent;
- Approximately 50% of the people affected by rare diseases are children; 30% will not live to see their 5th birthday; and
- Approximately half of identified rare diseases do not have a disease-specific advocacy network or organization supporting research and development.

While the ODA clearly boosted interest in pursuing rare disease treatments, its incentives are a fixed set of counterbalances to the economic calculation of research and development costs, projected risk, and population-based revenue estimates. Reimbursement mechanisms and hurdles can tip the scales for or against pursuing a specific drug candidate for an orphan indication. For patient populations approaching the 200,000 orphan disease limit, the ODA incentives may be sufficiently robust to mitigate clinical trial and reimbursement risks. As affected populations dwindle below 20,000 or even into and below the hundreds, however, the balance is far more fragile.

Despite dramatically increased availability of novel treatment options, many Medicare beneficiaries with rare diseases still face hurdles accessing lifesaving and life improving FDA-approved therapies. For example, patients treated in the hospital inpatient setting for extremely rare disorders requiring high-cost therapies may find that inadequate reimbursement creates an

impenetrable barrier to access. Although orphan drugs may be the only treatment available to reduce a patient's disease burden, institutional providers are increasingly aware of, and hesitant to absorb, financial losses associated with treating extremely rare conditions with orphan drugs.

As Medicare's Quality Payment Program becomes an established factor in provider decisions on which patients to treat and how to treat them, we fear that its zero-sum framework will create risks and uncertainties for clinicians encountering patients with extremely rare conditions in the near-term, and exact a chilling effect on innovation in the long-term.

CMS Must Account for the Unique Challenges Ultra-Rare Disorders Present to Medicare's Quality Payment Program

We support system-wide reforms designed to expand equitable access to quality health care, and believe that the Quality Payment Program's goal of emphasizing care quality over quantity is an appropriate step toward such reforms. The Medicare Access and CHIP Reauthorization Act of 2015 (MACRA) represents a substantial refinement to Medicare incentives under the Physician Fee Schedule, and we recognize the magnitude of effort required of CMS in implementing the transition toward the Quality Payment Program. While the Quality Payment Program Proposed Rule does not discuss rare diseases or orphan drugs, the underlying policies have a clear potential to shift incentives in a manner that can impact providers treating patients with rare disorders. Ideally, the Agency's efforts will result in a flexible framework of value-based incentives that can be adapted to evolving standards of care for all patients, including those with extremely rare disorders.

We support CMS' weighting of the cost performance category at zero percent for performance year 2018, and urge the Agency to devise a structural exception from MIPS scoring for costs associated with diagnosing and treating ultra-rare disorders in future years.

For the 2018 performance year, CMS proposes to retain the category weights applicable to the 2017 performance year. This means that the cost performance category will remain weighted at 0% until 2019, when MACRA mandates a weight of 30% for the cost component of the MIPS total score. The Agency has requested stakeholder comment on potential alternatives to the large shift in the 2019 performance period, including weighting the cost category at 10% in the 2018 performance year.

We have serious concerns about the potential impact that the cost component might have on clinicians treating patients with extremely rare diseases. As a threshold matter, it is virtually impossible to reliably benchmark costs associated with treating Medicare beneficiaries with ultra-rare disorders, and even more so if the patient suffers from an additional chronic condition. Difficulties associated with applying a cost component to ultra-rare disorders include:

- Diagnosing a patient with a rare disorder is usually a multi-year process involving a series of primary care clinicians, specialists, and diagnostic testing regimens – extreme rarity of a disorder compounds the resources required for diagnosis;

- Diagnostic coding systems do not have the granularity to capture and precisely describe each ultra-rare disorder, so that these conditions are often grouped with similar disorders within an ICD-10 code;
- The relatively small population size for ultra-rare disorders precludes availability of clearly articulated, scientifically-validated treatment standards that would form the basis of a reliable benchmark;
- Patients with ultra-rare disorders may not have access to a specialist with experience in treating their condition, leaving their care to a set of providers in various specialties that address specific disease symptoms. It is, therefore, difficult to assess which costs to assign to a specific clinician; and
- Highly-specialized clinicians with expertise sufficient to manage the whole patient would appear, for MIPS purposes, to perform poorly with respect to care cost rather than as a high-quality clinician providing efficient care.

We urge CMS to devise a structural exception that would permit MIPS-eligible clinicians to treat patients with ultra-rare disorders, including administration of Part B drugs and prescribing of Part D drugs, without risking lowering their total MIPS score. Any cost benchmark for treating ultra-rare conditions is more likely to be arbitrary than accurate, and it would be unfair to both providers and patients to inject unnecessary risks and uncertainties that might jeopardize treatment access.

We urge CMS to (i) develop measures and improvement activities that reflect quality care for rare diseases in the Merit-Based Incentive Payment System (MIPS), (ii) enable clinicians to earn MIPS bonus points for diagnosing and/or appropriately managing and treating patients with ultra-rare disorders, and (iii) develop an outlier-styled mechanism to account for ultra-rare disorder diagnosis and treatment costs under Advanced Alternative Payment Models.

The QPP was built around relatively common conditions, and is not well-suited to capture either quality or, as noted above, costs of care for patients with extremely rare disorders that may be difficult to diagnose and costly to treat. From an operational standpoint, the program's shortcomings in addressing ultra-rare disorders may not impact its overall functionality in addressing quality care for the most commonly-encountered conditions in the Medicare population. The structure and criteria for implementing quality measures make it difficult, if not infeasible, to create measures reflecting care for each, or even related subsets of ultra-rare conditions as the measures would fail to meet both the benchmark and case requirement thresholds. Clinicians would likely not elect to report on measures for which a maximum of 3 points could be awarded.

We suggest that CMS develop alternative means to reward clinicians treating patients with ultra-rare disorders, including practice improvement and advancing care information measures specific to ultra-rare disorders. This might include incentivizing use of disease-specific patient registries, inclusion of communications regarding clinical trial participation within care planning, and use of FDA-approved therapies for the ultra-rare indication. Ideally, CMS would develop a

set of quality measures reflecting the main components of quality care for people with ultra-rare disorders:

- Recognition of patients at risk for the disease;
- Start the appropriate evaluation;
- Make the appropriate diagnosis;
- Start the appropriate treatment;
- Schedule the appropriate follow-up;
- Stimulate the appropriate compliance/adherence to treatment.¹

Similarly, patients with ultra-rare disorders should have appropriate access to clinicians electing the APM path. CMS must develop a mechanism through which these clinicians and their APMs can confidently utilize the resources necessary and appropriate to treat these patients, without concern that they will personally or collectively bear the cost burden. A relatively simple approach would permit clinicians to treat patients with elusive diagnoses or ultra-rare disorders as Medicare fee-for-service patients outside the APM.

The QPP could play an important role in advancing the quality of care for ultra-rare disease patients for which FDA-approved treatments are currently available, as well as advance overall understanding of disease processes for those conditions with no available treatment options. As CMS continues to refine the program, we urge it to devise any necessary protections to ensure that efforts toward quality care for commonly-encountered conditions does not impede or complicate treatment access for individuals with ultra-rare conditions.

We urge CMS to reverse its proposed policy of applying payment adjustments to Part B drug costs, or, at a minimum, exempt orphan drugs for ultra-rare conditions from that policy.

In its Proposed Rule, CMS stated that Part B items and services (e.g., purchasing and administering Part B drugs) could be subject to MIPS adjustment or included for eligibility determinations. Payments related to the purchase or administration of Part B drugs that CMS can associate with a MIPS eligible clinician would, under the Proposed Rule, be increased or decreased in the same manner as the clinician's payments for professional services.

We have serious concerns about the potential impact this policy might have on providers and patient access to therapy, particularly orphan drugs for ultra-rare conditions. The uncertainty injected by CMS' caveat regarding instances in which attribution of Part B drug and administration costs to a specific clinician may not be feasible is likely to compel many clinicians to steer their service offerings away from the uncertainties and risks associated with in-office administration of more costly Part B drugs. We expect that this will, over the long term, disproportionately impact patients with ultra-rare diseases, given the paucity of counterbalancing quality measures that might increase a clinician's total MIPS score.

¹ Health Indicators for Rare Diseases, Conceptual framework for the use of health indicators for monitoring quality of care, EUCERD, http://www.eucerd.eu/?post_type=document&p=1353 (accessed July 23, 2017)

We are similarly concerned that clinicians facing negative adjustments will be keenly aware of the financial impact associated with in-office drug administration and could shift patients to other providers, including hospital outpatient centers, or select older, less costly treatment options. Conversely, providers earning the highest levels of payment adjustments would tend to decrease drug administration referrals to outside providers and would have a strong incentive toward increasing in-office drug administration. It is unlikely that the net impact of this policy on overall Medicare Part B costs would be neutral, and it has the potential to dramatically increase Part B drug costs while disrupting continuity of care for patients requiring higher-cost therapies. Patients with ultra-rare disorders have limited options with respect to both treating physicians and disease-specific treatments – any constriction of either can present an absolute access barrier.

CMS' hospital outpatient prospective payment system proposal to reduce reimbursement for Part B drugs administered in hospital outpatient departments from ASP+6% to the ASP-22.5% level reflecting the average 340B discount will compound the issues facing clinicians and patients. Any cost savings associated with shifting Medicare beneficiaries requiring Part B drugs to 340B covered entities will substantially grow an already-bloated 340B drug discount program while overburdening impacted outpatient departments and subjecting patients to increased travel burdens and waiting times to receive treatment. We believe that this type of shift would undermine CMS' goal of a patient-centered system focused on quality care, and that for patients with ultra-rare disorders, it could exact an insurmountable burden to obtaining necessary medical care.

We urge CMS to reconsider this proposal or, at a minimum, exempt orphan drugs for ultra-rare conditions from payment adjustment applications that could reduce provider reimbursement for treating these patients.

We appreciate that CMS recognizes the need to ensure that clinicians treating Medicare's sickest patients are not penalized. We support the proposed complex patient bonus under the MIPS, and urge CMS to implement a presumption that patients with ultra-rare disorders are "complex."

CMS has proposed to award 1 to 3 bonus points for clinicians who see a more complex patient population. The Agency proposes measuring complexity by using a Hierarchical Condition Category (HCC) risk score. The HCC score compares Medicare beneficiaries' FFS spending to the overall average for the entire Medicare population. The HCC score methodology has been used in other CMS programs to calculate risk adjustment.

We understand that CMS proposes this MIPS bonus opportunity to:

- Protect access to care for complex patients;
- Encourage high-quality care; and
- Mitigate any real or perceived disadvantage encountered by MIPS-eligible clinicians caring for complex patients.

We applaud CMS for its attention to the needs of Medicare's most vulnerable patients, and believe that patients with ultra-rare disorders should be presumptively complex for purposes of calculating a clinician's eligibility for the complex patient bonus. We ask that CMS monitor application and utility of the HCC in identifying patients with ultra-rare disorders as high complexity.

We support the Proposed Rule's focus on flexibility and reduced clinician burden, and urge CMS to utilize this transition to ensure that the QPP incentivizes high-quality care for patients with ultra-rare disorders.

CMS' measured approach toward implementing MACRA's payment system changes through the second performance year demonstrates the Agency's commitment to reducing clinician burden and uncertainties as Medicare transitions to a value-based reimbursement paradigm. We expect that this will enable providers and CMS to identify and mitigate many unforeseen or unintended impacts the new payment adjustment system might have on beneficiary access.

We applaud CMS' attention to the stated concerns of providers in small practices, and support the Agency's proposed set of additional flexibilities, including:

- implementing the virtual groups provision;
- increasing the low-volume threshold to less than or equal to \$90,000 in Medicare Part B allowable charges or less than or equal to 200 Medicare Part B patients (previously, set at \$30,000 in allowable charges and less than or equal to 100 Medicare Part B patients);
- adding a significant hardship exception for the advancing care information performance category; and
- providing for addition of bonus points to the final score of MIPS eligible clinicians in small practices.

As providers transition into the quality Payment Program, we urge CMS to maintain a level of transparency to providers and continue its small practice outreach efforts as it examines which policies to continue or expand in future years. We ask that CMS use this phase-in of the QPP to closely examine any adverse impact on patients with ultra-rare disorders, continue its stakeholder outreach efforts, with an expanded focus on rare and ultra-rare disorders, and develop the patient and provider protections outlined above.

Conclusion

Once again, we appreciate your outreach efforts and commitment to identifying ways to improve Medicare as a patient-centered care program. We look forward to working with you to ensure that our innovations toward treating and curing ultra-rare disorders reach the patients who need them. By aligning Medicare's approach to rare disease treatments with the public policy goals of the *Orphan Drug Act* and the *21st Century Cures Act*, our scientific advances can maximize their potential in improving the lives of rare disease patients.