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Seema Verma
Administrator
Centers for Medicare & Medicaid Services
7500 Security Boulevard
Baltimore, MD 21244-1850

BY ELECTRONIC DELIVERY

Re: Medicare Program: Proposed Changes to Hospital Outpatient Prospective Payment and Ambulatory Surgical Center Payment Systems and Quality Reporting Programs; Requests for Information on Promoting Interoperability and Electronic Health Care Information, Price Transparency, and Leveraging Authority for the Competitive Acquisition Program for Part B Drugs and Biologicals for a Potential CMS Innovation Center Model (CMS-1695-P)

Dear Administrator Verma,

Celgene Corporation (Celgene) appreciates the opportunity to respond to the Centers for Medicare & Medicaid Services' (CMS) Hospital Outpatient Prospective Payment System proposed rule ("Proposed Rule") – and, to CMS' Request for Information (RFI) on a potential Competitive Acquisition Program (CAP) for Part B drugs and biologicals.

Celgene is a global biopharmaceutical company specializing in the discovery, development, and delivery of therapies designed to treat cancer and inflammatory and immunological conditions. Celgene strongly believes that medical innovation can lead to better health, longer life, reduced disability, and greater prosperity for patients and our nation. To this end, we seek to deliver truly innovative and life-changing therapies for the patients we serve. We are currently engaged in 160 clinical trials with 42 novel medicines across 60 indications. In 2017, we reinvested 45.5%

of our revenue into research and development to discover and develop the therapies of tomorrow.[1]

Celgene strongly supports the Administration's efforts to ensure that all patients have affordable access to the care they need. As committed as Celgene is to discovering and developing new treatments, we are equally committed to patient support and access to those medical advances, which is a guiding principle for our company. We believe all who can benefit from our discoveries should have the opportunity to do so. Celgene focuses on putting patients first with programs that provide information, support, and access to our innovative therapies.

Request for Information on Leveraging the Authority of the Competitive Acquisition Program for Part B Drugs and Biologicals

Celgene supports the Administration's continued focus on value-based arrangements for prescription drugs. We strongly support the removal of regulatory barriers to the development and evolution of value-based arrangements, and we appreciate the Administration's ongoing dialogue with stakeholders on the most appropriate regulatory paradigm for value-based arrangements.

We believe that value-based arrangements can enhance the negotiations that take place across markets, including in Medicare Part B and Part D programs. We support a demonstration to test value-based arrangements for Part B drugs and biologics while encouraging HHS to take broader action that would enable the development of value-based arrangements outside of Part B.

The RFI highlights several types of value-based arrangements, including indication-based payments, outcomes-based payments, payment over time, and payments based on total cost of care. We strongly encourage CMS to focus on indication-based payment as a cornerstone of any Part B demonstration. We believe that, with certain updates to regulatory and coding rules, biopharmaceutical companies and payers are well positioned to negotiate indication-based payment models that tie both payment and beneficiary cost sharing to an indication-specific, value-based price.

Indication-based payment models can help ensure that a product's pricing reflects this dynamic by linking the price for each product indication to its value to the specific patient population it serves. Sophisticated payers, providers, and biopharmaceutical companies have the data and expertise necessary to negotiate indication-based payments.

We believe that indication-based payments could play a role in public and private insurance programs, including Medicare Parts B and D, and are ideally suited for the demonstration CMS describes. Many Part B products have multiple indications, making them potential candidates for an indication-based approach. Further, indication-based models offer a relatively more straightforward and less burdensome pathway to value-based payment. For example,

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[1] Celgene 2017 Annual Report. Available at: http://files.shareholder.com/downloads/AMDA-262QUJ/6204845187x0x978672/138C3639-1839-499D-8191-34F9E08A0CBD/Celgene_AR_complete_PDF_041718.pdf.

indication-based payment models require less intensive patient monitoring and retrospective claims analysis. However, current product coding rules make these contracts difficult to implement in an accurate and consistent manner. Therefore, we reiterate our recommendation that CMS permit biopharmaceutical companies participating in any Part B demonstration to apply for indication-specific J codes at the time of FDA approval.[2]

Issuing separate drug codes would address many of the challenges associated with indication-based payments today, including:

- Aligning beneficiary cost sharing with each indication's value (i.e., connecting cost sharing to the appropriate list price);
- Accurately recording indication or diagnosis at the patient level;
- Reducing provider and payer confusion; and
- Enabling appropriate oversight of the contract.

Our detailed comments, grouped by RFI section header, are below.

Biopharmaceutical Company Participation

We understand that CMS wishes to evaluate value-based arrangements in Medicare Part B through a CAP-like model. We fully support the goal of promoting value-based care, and we believe that value-based arrangements have the potential both to facilitate patient access to high-value care, and to ensure that cost sharing reflects a medicine's value.

However, our experience tells us that value-based arrangements are mostly likely to be successful when they reflect a medicine's unique clinical and other attributes. Well-designed value-based arrangements can also be very complex, often requiring biopharmaceutical companies and health plans to establish new data collection and sharing pathways, among many other supporting processes.

Therefore, we firmly believe that participation in a potential CAP-like model should be voluntary for biopharmaceutical companies. We also believe that biopharmaceutical companies will participate in the demonstration if CMS removes regulatory barriers to value-based contracting, offers flexibility in contract structures and specifics, and addresses potential government pricing concerns.

With respect to pricing we encourage CMS to ensure that any demonstration remains a true "test.". Coding, pricing, and price reporting within and outside of the demonstration should remain separate and distinct, with the current model continuing to apply outside of the testing environment. Given the complexities in the current prescription drug supply chain and in government price reporting rules, it will be critical to identify and adjust for any unintended consequences or lessons learned via the demonstration before contemplating broader change.

[2] Celgene comments on HHS Blueprint to Lower Drug Prices and Reduce Out-of-Pocket Costs, submitted June 27, 2018.

Included Drugs and Biologicals

The RFI suggests a phased approach to a value-based agreement demonstration, with an initial focus on a set of therapeutic classes. Celgene strongly supports such a phased approach, which will best position CMS, biopharmaceutical companies, and any program vendors for success in the short and long term. A phased approach will also allow CMS to apply learnings in real time as the demonstration progresses. As CMS evaluates potential therapeutic classes for inclusion in the initial test model, we recommend that CMS consider several factors:

- The availability of multiple therapeutic options in the class, including multiple options designated as preferred or recommended treatment options in clinical guidelines;
- Suitability of value-based arrangements to medicines in the therapeutic class;
- Whether program vendors and biopharmaceutical companies could achieve a "critical mass" of utilization to support the development of a viable innovative contract; and
- Whether biopharmaceutical companies, suppliers, and vendors can ensure a sufficient supply of medications in the drug class to enable patient access and prevent delays in care.

CMS should consider the importance of beneficiary access and provider medical judgment for all physician-administered medications and establish an exceptions process that preserves patient access and provider choice for included drugs and biologicals, as described elsewhere in this letter.

CMS also asks stakeholders to identify any drugs or drug classes that should be excluded from the demonstration. We believe that highly complex, personalized medications are not appropriate candidates for the demonstration, particularly in the early stages of testing.

The rapid pace at which cell and gene therapy science is evolving complicates the development of value-based arrangements for these medicines. For example, while indication-based payment models may be appropriate for some cell and gene therapies, it would be premature to design an arrangement based on total cost of care for these emerging therapies because sufficient data are not yet available.

Cell and gene therapies are also specialized and differentiated at the product level; even within the same disease area, not all patients will be candidates for all therapies based on FDA-approved labeling. As the evidence base supporting cell and gene therapies continues to grow and mature, we expect to see significant fluidity and change in target patient populations, indications, and other key clinical features of these medicines. It would be prohibitively complicated, particularly within a demonstration construct, for biopharmaceutical companies and vendors to synchronize value-based arrangements terms with frequent changes in the clinical profile of cell and gene therapies. Further, it may be difficult to achieve a critical mass of patients in specific sub-groups, limited vendors' and CMS' ability to conduct a rigorous evaluation of the contracts.

Finally, we would strongly urge CMS to consider the unique manufacturing processes for cell and gene therapies. For example, manufacturing processes for chimeric antigen receptor (CAR) T cell therapies involve numerous interdependent steps that must completed on a prescribed

schedule. Given the complexity and specificity of the CAR T cell manufacturing process and the critical health status of CAR T cell patients, it is particularly critical for beneficiaries and providers to have access to multiple treatment options.

Included Providers and Suppliers

Provider participation in any Part B demonstration should be voluntary at the provider or group level, both because a voluntary model will support the targeted, hands-on approach that we believe will be necessary to achieve success, and because providers are best suited to determine whether participation is appropriate based on their patient panels and ability to support value-based agreements.

We are pleased CMS recognizes that many providers prescribe and administer a significant number of Part B drugs and agree that seeking feedback on how to mitigate changes in provider revenue under the demonstration is both practical and appropriate. Providers will play a critical role in ensuring that value-based arrangements are implemented correctly and that all demonstration participants have the data necessary to support contract execution. For example, in an indication-based payment model, providers may need to undergo or provide additional training and oversight to their offices to ensure that beneficiary diagnoses are recorded accurately and consistently. In addition, providers participating in indication- and other types of value-based arrangements will need to adjust their billing practices to collect the appropriate amount of cost sharing for each included drug or biologic. We agree that addressing potential shifts in provider revenue is appropriate and critical to any demonstration's success, as evidenced by the high rates of provider attrition in the original CAP model test. Therefore, we recommend that HHS reimburse participating providers at fair market value for administrative costs associated with any demonstration through a new "payment innovation coordination fee," shared savings, or other mechanisms.

Beneficiary Access, Cost Sharing, and Financial Considerations

Beneficiary cost sharing should align with the value-based price for a product; demonstration vendors or contract administrators should be required to ensure that beneficiaries realize a portion of any savings associated with value-based arrangements in the form of lower cost sharing at the point of sale. For example, in an indication-based payment model, an individual beneficiary's cost sharing at the time of administration should reflect the price of the product for his or her specific diagnosis.

When properly designed, value-based arrangements should improve beneficiary access to medicines. Affordability is a key component of patient access; accordingly, cost sharing should be capped at 20 percent of a drug's value-based price, and any program vendors should be required to apply the full benefits of any supplemental coverage for beneficiaries included in the demonstration.

In addition to affordability, the demonstration should address key patient protections based on the Competitive Acquisition Program and the attributes of the Medicare population. At a

minimum, these guardrails should include rigorous exceptions processes that allow a provider to access the most appropriate drug for an individual beneficiary based on the patient's unique clinical history and presentation; contingencies for emergency supplies of medication and a process for providers to obtain different doses or treatment options in real time; appropriate notification to beneficiaries who are enrolled in the demonstration; and ongoing monitoring of patient access through proactive outreach to patients and providers. Should the arrangements negotiated via the demonstration include utilization management protocols, these patient protections will be especially critical.

All program vendors must have the ability to monitor and oversee complex contracts, and to consistently and fully enforce the term of any value-based arrangements. For example, any indication-based payment framework has the potential to encourage mis-coding. While we expect that the vast majority of providers who elect to participate in a demonstration would record utilization accurately and consistently, any vendor administering an indication-based contract should be responsible for auditing beneficiary-level data.

CMS will need to establish a strong program integrity and compliance framework, particularly because value-based arrangements are likely to require the exchange of sensitive information. CMS should consult with potential vendors and biopharmaceutical company participants to develop a data validation strategy, as companies will also need to audit vendor data to ensure that contracts are implemented according to their specific terms.

Finally, CMS should ensure that demonstration vendors collect the minimum set of data necessary to implement a value-based arrangement and establish specific standards and guidelines for the safe maintenance and use of beneficiary data. Vendors should be required to inform beneficiaries about what data will be collected to support the demonstration and how the vendor will protect beneficiaries' personal or health information.

Model Vendors

Vendors participating in a new Part B demonstration focused on value-based arrangements will need to have substantial expertise and technical capabilities to deliver CMS' desired outcomes. We believe that the following attributes are particularly critical for all vendors, for reasons elaborated below:

- Ability to intake, link, and analyze medical and pharmacy claims data on an ongoing basis;
- Insight into and experience analyzing beneficiaries' total cost of care;
- Ability to accurately connect claims through a unique patient identifier, consistent with privacy and security best practices; and
- Experience with and access to retrospective claims data for the Medicare population.

All vendors will need to demonstrate certain technical capabilities, such as the ability to link records through a unique patient identifier that can distinguish demonstration participants from other beneficiaries, securely manage and store beneficiary data, or identify patient sub-populations based on demographic or clinical attributes. Beyond these core competencies, we

believe that any demonstration vendor should also have experience with developing or analyzing total cost of care models and utilization patterns for Medicare beneficiaries.

Vendors must be able to monitor and analyze the impact of the demonstration on access to care, beneficiary outcomes, and provider administrative burden. As described in greater detail below, all vendors should be required to submit a rigorous quality evaluation plan and to provide progress reports to CMS.

Value-based arrangements are complex and both population- and patient-specific. The Medicare population is unique, and we believe that it would be very difficult for a demonstration vendor to negotiate a sophisticated contract – and a "good deal" – on behalf of beneficiaries without a firm understanding of the health needs they face. Therefore, we strongly recommend that CMS evaluate not just a vendor's expertise in negotiating value-based arrangements or in managing large volumes of clinical data, but also the vendor's knowledge of the specific therapeutic areas included in the demonstration, the Medicare population as a whole, and how beneficiaries use physician-administered prescription drugs.

CMS poses several questions about potential operational models for a Part B demonstration. As CMS continues to evaluate design options, we encourage the Agency to consider the additional complexity associated with introducing new entities, distribution models, or financial flows, and to focus on the models that achieve the greatest benefit for demonstration participants and the Medicare program. For example, based on our understanding of the "consignment" approach, a vendor could be required to execute and administer contracts with each entity in a prescription drug's supply chain to ensure accurate payment according to value-based contract terms. In addition to the administrative time needed to put multiple contracts per value-based agreement into place, vendors would also need to exchange and manage data across multiple entities in the supply chain – substantially increasing the risk of operational failure. We encourage CMS to leverage existing distribution models and financial flows to the extent possible to allow all stakeholders to focus on the primary purpose of the demonstration: testing value-based payment approaches in Part B.

Model Scope

In developing any new demonstration, we urge CMS to consider the landscape of existing demonstrations, and to avoid overlapping tests wherever possible. CMS should consider existing requirements for providers who participate in the Oncology Care Program and evaluate the potential unintended consequences of introducing a new payment model. We discourage CMS from creating overlapping models that could increase provider confusion, increase administrative burden, and create competing priorities for the subset of providers who are highly engaged and well positioned to succeed under these models.

We recommend that any Part B-focused demonstration emphasize the fee-for-service program – especially in the initial phases of testing. Allowing entities outside of the demonstration program, such as Medicare Advantage plans or Medicaid managed care organizations, to "buy in" to the demonstration could substantially increase operational and contractual complexity. More importantly, it may not be appropriate for organizations that operate in different markets and that enroll different patient populations to access a demonstration contract. For example, a

biopharmaceutical company and a demonstration vendor may agree on certain clinical outcomes as part of a value-based arrangement focused on the Medicare population; the same clinical outcomes may be significantly less relevant – or even inapplicable – to a pediatric or younger adult population.

Regulatory Barriers and Transparency Issues

We appreciate the Administration's continued focus on removing regulatory and legal barriers to the development of value-based arrangements. As we have stated in prior comments, we strongly encourage the Administration to update government pricing and anti-kickback rules to address and accommodate value-based arrangements. We recommend that CMS undertake rulemaking to update all government pricing calculations, and that the Department work with OIG to establish safe harbors for value-based arrangements under the anti-kickback statute. These regulatory actions will lay a critical foundation for further innovation and sophisticated negotiations between biopharmaceutical companies and payers.

Medicaid Best Price has been identified as a key barrier to value-based contracting; however, HHS should update all government price reporting (including average manufacturer price, average sales price, non-federal average manufacturer price) to ensure a consistent and comprehensive approach across public programs.

New rulemaking should establish when companies may exclude payments made pursuant to value-based arrangements from government price reporting, in recognition of the fact that value-based payments reflect complex and population-based metrics. Value-based arrangements, when they are designed correctly, consider patient populations; while outcomes may be assigned at the patient level, these contracts tend to reflect a holistic and population-focused assessment of value. By definition, a population-based approach will incorporate variation at the patient level. Therefore, the price paid for any unit of medicine in a value-based arrangement may or may not represent the overall structure of the value-based arrangement. In fact, prices that could implicate Medicaid Best Price are particularly likely to be outliers.

Key issues to address in new rulemaking include: value-based arrangements generally, and which types of value-based contracting structures would be excluded from government price reporting; the role of financial risk, and whether a minimum level of risk would be necessary for a contract to be excluded; and any additional contract parameters that would protect against potential gaming (e.g., bona fide contracts that require payment terms to be determined in advance and remain fixed throughout the contract term).

Regulation should also clarify the treatment of value-based arrangements under the anti-kickback statute. Because "remuneration" is broadly defined in the statute, companies would benefit from additional clarity on how the existing safe harbors apply to value-based arrangements. We recommend that CMS work with OIG to clarify that HHS would not view value-based payments as "kickbacks" by updating regulatory safe harbors and corresponding guidance.

We recognize that some of these questions and issues are included in the recently released Request for Information on safe harbors under the Antikickback Statute and look forward to offering our recommendations in response.

Finally, CMS seeks feedback on how to evaluate a potential Part B demonstration. We recommend that program vendors and CMS monitor the impact of the demonstration on patients, providers, and the Medicare program by assessing at least the following key metrics:

- Beneficiary access to care, including changes in utilization patterns and patient and provider perceptions of access to care;
- Beneficiary cost sharing;
- Provider participation, including provider retention;
- Provider experience, with a focus on administrative burden;
- Medicare program spending for beneficiaries enrolled in the demonstration, to include both Part B drug and total cost of care.

CMS should require vendors to conduct and report on CMS-approved evaluation plans. In addition, CMS should consider whether existing quality ratings – such as the Medicare Advantage Star Ratings – could be adapted for program vendors. For example, CMS could modify and apply measures related to member experience, complaints, and customer service to evaluate vendor performance.

Status Indicators for New Category III Current Procedural Terminology (CPT®)[3] Codes for Chimeric Antigen Receptor T-cell Therapy (CAR T) Services

Earlier this year, the American Medical Association (AMA) created four new Category III CPT codes for CAR T therapy that will become effective January 1, 2019. CMS included the four new codes in Addendum B of the Proposed Rule with status indicator "B," indicating "Codes that are not recognized by OPPS when submitted on an outpatient hospital Part B bill type (12x and 13x); Not paid under OPPS; May be paid by MACs when submitted on a different bill type, for example, 75x (CORF), but not paid under OPPS; An alternate code that is recognized by OPPS when submitted on an outpatient hospital Part B bill type (12x and 13x) may be available."[4]

HCPCS Code	Descriptor	Status Indicator
05X1T	Chimeric antigen receptor T-cell (CAR-T) therapy; harvesting of blood-derived T lymphocytes for development of genetically modified autologous CAR T-cells, per day	B
05X2T	preparation of blood-derived T lymphocytes for transportation (e.g., cryopreservation, storage)	B

[3] CPT is a registered trademark of the American Medical Association (AMA).

[4] Addenda B and D1 of the Proposed Rule is available at: https://www.cms.gov/Medicare/Medicare-Fee-for-Service-Payment/HospitalOutpatientPPS/Hospital-Outpatient-Regulations-and-Notices-Items/CMS-1695-P.html.

HCPCS Code	Descriptor	Status Indicator
05X3T	receipt and preparation of CAR T cells for administration	B
05X4T	CAR T cell administration, autologous	B

On August 20, 2018, the Advisory Panel on Hospital Outpatient Payment (HOP Panel) recommended that CMS reassign the status indicators for these CPT codes from "B" to "S" and offered proposed payment rates for each code.[5]

CMS Should Remove References to Leukapheresis from HCPCS Codes for CAR T

The Healthcare Common Procedure Coding System (HCPCS) codes for the two marketed CAR T therapies – Q2041, YESCARTA® (axicabtagene ciloleucel), and Q2040, KYMRIAH® (tisagenlecleucel) – currently include "leukapheresis and dose preparation procedures" in the descriptor. At the May 16, 2018 HCPCS Public Meeting, however, many organizations requested that this language be removed because it includes provider services that traditionally are billed separately from the drug product. In addition, speakers at the meeting asked that CMS adopt permanent J-codes for these drugs instead of temporary Q-codes.

Celgene urges CMS to remove "leukapheresis and dose preparation procedures" from all existing and future CAR T HCPCS code descriptors, consistent with CMS' approach to pharmacy service costs for other drugs and biologicals under the OPPS. We also urge the Agency to adopt J-codes instead of Q-codes for all existing and future CAR T cell therapies.

In addition, CMS should cover and pay separately for leukapheresis, which is resource-intensive and distinct from the administration of CAR T cell therapy; for example, leukapheresis may be performed at a different facility than where the CAR T drug ultimately is administered. CMS also should clarify that the National Coverage Determination (NCD) for Apheresis (Therapeutic Pheresis)[6] does not apply to harvesting of blood-derived T lymphocytes for development of genetically modified autologous CAR T cells.

CMS Should Refrain from Classifying CAR T Cell Therapy Services as "Emerging"

Celgene is concerned that the AMA created Category III codes, are described as codes "for emerging technology, services, procedures, and service paradigms. Category III codes allow data collection for these services/procedures."[7]

YESCARTA® and KYMRIAH® are not experimental, emerging technologies. While CAR T cell therapy is certainly innovative, these medicines are and will continue to be Food and Drug

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[5] The Panel recommended that CMS assign CPT codes 05X1T and 05X4T to Ambulatory Payment Classification (APC) 5242, Level 2 Blood Product Exchange and Related Services with a proposed payment rate of $1,222.97, and CPT codes 05X2T and 05X3T to APC 5241, Level 1 Blood Product Exchange and Related Services with a proposed payment rate of $383.39.[5]
[6] CMS, National Coverage Determination (NCD) for Apheresis (Therapeutic Pheresis), available at: https://www.cms.gov/medicare-coverage-database/details/ncd-details.aspx?ncdid=82&ver=1.
[7] American Medical Association, CPT 2018, Professional Edition, at 724.

Administration (FDA) approved biological products that have gone through clinical trials and the rigorous Biologics License Application (BLA) pathway under section 351 of the Public Health Service Act (PHSA). Likewise, the services performed to harvest cells, prepare them for transportation, receive the CAR T cells, and infuse them are not experimental, emerging services; rather, these services are performed today for CAR T patients, and have been performed for other drugs and biologicals (e.g., Provenge®).

Finally, we note the pending National Coverage Analysis (NCA) for CAR T-cell therapy for cancers, which CMS expects to complete May 17, 2019.[1] Not only can this NCA ensure national coverage for YESCARTA®, KYMRIAH®, and future CAR T-cell therapies for cancers, but it can clarify coding and coverage. In the interim, CMS should clarify in the final rule that providers have the option of billing a Category I CPT unlisted code or the codes for intravenous infusion chemotherapy and other highly complex drug or highly complex biologic agent administration for the administration of CAR T cell therapies rather than 05X4T, CAR T cell administration, autologous, until the NCA is complete and CMS issues additional coverage and coding guidance.

In summary, Celgene recommends (1) that CMS remove "leukapheresis and dose preparation procedures" from all existing and future CAR T HCPCS code descriptors and (2) that CMS clarify that providers should bill separately for leukapheresis and for CAR T cell therapy administration using existing category I CPT codes.

Conclusion

Celgene shares the Administration's goal of ensuring that all Americans, irrespective of their source of coverage, have affordable access to the medicines they need. We are proud of the innovation and value that prescription medicines bring to our healthcare system but recognize that scientific innovation can only realize its full potential if patients can access care. We hope to help advance the Administration's work in this important area, and we would welcome the opportunity to discuss our comments and any of these issues in further detail.

Thank you for your consideration of our comments.

Sincerely,

Richard H. Bagger

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