

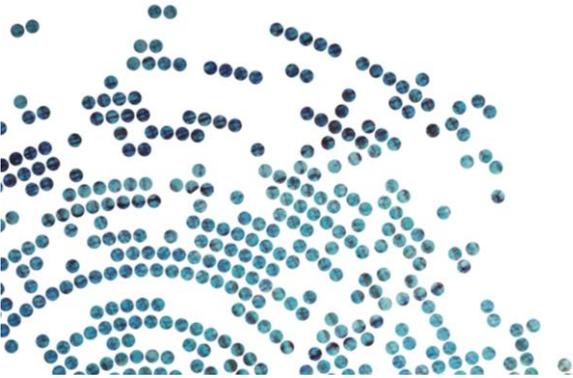


PMC Policy Committee Meeting
Tuesday, June 19, 2018, 12:00 p.m. ET

Business Meeting Materials Packet

Contents:

1. RFP: Diagnostic Odyssey Clinical and Economic Value of WES in Rare and Undiagnosed Diseases
2. PhRMA Foundation Value Assessment Initiative Request for Proposals Challenge Awards 2018
3. RFP: Landscape Analysis of the Integration of Personalized Medicine into U.S. Health Systems
4. PMC Comment Letter to CMS on Medicare National Coverage Analysis for CAR T-cell Therapy for Cancer (submitted June 15, 2018)



Request for Proposal: Clinical and Economic Value of WES in Rare and Undiagnosed Diseases

BACKGROUND

For patients with rare and undiagnosed diseases, whole-exome sequencing (WES) — determining a patient's entire unique protein-coding DNA makeup — may be an extremely valuable tool for discovering the genetic alterations that contribute to disease development, as well as for influencing treatment decision-making. These patients are most often children who may have already undergone standard genetic testing such as chromosomal microarray or single-gene testing, but still have not received a definitive diagnosis. They are sometimes referred to as "diagnostic odyssey" cases, as a child and family can bounce around various medical centers for years, undergoing numerous diagnostic procedures and generating increasingly high health care costs while their disease goes undiagnosed.

Going through their diagnostic odyssey, these patients' diseases progress and they lose time during which they could potentially receive effective therapies or be enrolled in a clinical trial, had they been accurately diagnosed.

OBJECTIVE

The adoption of genomic sequencing diagnostic technologies into clinical care depends on their ability to deliver clear value and on the recognition of that value by various stakeholders. This project is intended to generate evidence, that demonstrates improved clinical outcomes and an economic benefit of WES in clinical care for specific patient subgroups with rare or undiagnosed diseases. The project will include a comparison of patients with diseases of unknown etiology who have undergone WES within their course of management with patients who have not, and will examine each cohort's cost of care, disease progression, clinical outcomes, and lifetime benefits as well as health economic benefits to the patient and healthcare system.

RESEARCH OUTLINE

The project will be conducted in two phases for which separate research proposals will describe each phase.

The first phase, to be completed in— 6 - 9 months, will involve the development of a clinical and economic value model validated with retrospective data from the literature and existing real-world data (with the potential for some prospective data where possible) from Geisinger and other health care delivery institutions that are routinely sequencing patients with diseases of unknown etiology (and in some cases, the child and both parents). The project may also involve a value of information analysis and the identification of factors that significantly impact cost-effectiveness of WES-based diagnostic testing. The analysis will also explore cost- and clinical-effectiveness in various patient clinical subgroupings to better understand which patients gain most value from the approach.

Research will utilize existing data sets and/or ongoing studies to the extent possible — supplemented by the collection of data from health system, medical records and external databases as needed.

A project steering committee consisting of clinical experts, diagnostic developers, rare and undiagnosed disease health care provider organizations, and patient groups will work with the health economics research

team to help ensure the use of appropriate data elements and to identify and provide appropriate datasources, as well as to guide the project and review progress at various milestones..

A payer and employer advisory committee to include health insurers, large employers, and employer benefits management groups will also be formed to provide guidance, especially related to cost/benefit assumptions utilized in the value model, so the project results will incorporate the perspectives of these key stakeholders increasing the likelihood that the results will be useful in informing the coverage and reimbursement process..

The second phase, to be completed in 12 -18 months, will involve capturing a more robust set of data describing both direct and indirect costs for patients who have not been sequenced. These data could then be used to refine the assumptions used for the model developed in phase one, increasing the validity of the model.

The second phase will involve a retrospective database study utilizing electronic health records, enriched with primary data examining direct and indirect costs to patients and their caregivers.

As the convener of the study, PMC will coordinate a meeting with the research team, advisory committee members, and other partners to determine what data sets should be explored for use in the study and how we will gain access to these data sets.

The project's aim is to assess the value of sequencing rare and undiagnosed disease patients in general. The study will not compare technology platforms but will focus on utility for this approach in general.

Results of the studies will be published in a peer-reviewed journal and disseminated through robust communications plans, including presentations at appropriate scientific and policy meetings, to increase awareness and provide evidence for platform developers, payers, and providers whether WES diagnostic testing is both clinically useful and economically efficient when applied to the patient population under study.

FUNDING

Each phase of the study will be funded separately. Total study budget and funding for each phase will be determined based on the funding needs for the successful proposals. Proposals should include estimated budget requirements and timing of study and publication milestones. Proposals will be rated based on study merit and design regardless of cost estimate.

SUBMISSION

Separate proposals should be submitted for each phase of the study. Proposed research concepts should provide an explanation of all project objectives, an overview of the analytical approach to the project, and a description of the project's overall value. Submissions will be evaluated based upon a combination of factors, including the potential impact of the research; and duration; certainty of outcome; how the research can be presented; and to what extent the research is important to key audiences. Submissions should generally be between 3-5 pages, with the possibility to expand and revise after initial review.

Interested institutions should contact Daryl Pritchard at dpritchard@personalizedmedicinecoalition.org.

About the Personalized Medicine Coalition

The Personalized Medicine Coalition (PMC), headquartered in Washington, DC, representing innovators, scientists, patients, providers and payers, promotes the understanding and adoption of personalized medicine concepts, services and products to benefit patients and the health system.

Key Dates

Opening Date	April 2, 2018
Letter of Intent Due	May 24, 2018
Notification to Proceed with Full Paper	June 15, 2018
Full Paper Submission Deadline	August 15, 2018
Anticipated Award Notification	September 15, 2018
Event to Showcase Award Winners	November/December 2018 (Location TBD)

Challenge Award

Personalized medicine – in which prevention and treatment strategies are guided by genetic tests, other biomarkers, and patient preference – is taking hold as a significant element of clinical care, particularly in the field of oncology. In 2017, FDA approved a record number of targeted medicines. The field of precision medicine has given rise to, and been enabled by, increasingly sophisticated electronic health data systems capable of capturing and analyzing large volumes of genetic, clinical, and patient-generated data. Thought leaders are seeking to harness this capacity to rethink health care and drive unprecedented transparency around the value of health care. Yet, conventional methods for value assessment remain rooted in conventional, population-level methods and evidence hierarchies.

The Foundation and Personalized Medicine Coalition are seeking papers that describe solutions to the following question: What are potentially transformative strategies and methods to define and measure value at all levels of decision making that are aligned with personalized/precision medicine?

Examples may include, but are not limited to:

- Develop tools to facilitate value-based personalized decision-making based on an individual patient’s specific genetic characteristics and preferences
- Propose a mechanism to incorporate individual patient characteristics into value assessment so as to appropriately value treatments at the patient level.
- Define a set of measures to capture health care value that can be integrated into a framework to support decision-making from different perspectives
- Generate instruments capable of capturing relevant and standardized measures of value
- Design value-based or outcomes-based contracts that incentivize improved quality, sustainability and continued innovation in medical devices, diagnostics and medicines
- Build decision-making tools that adapt behavioral economic principles to promote value

Award Funding Details

Recipients of Challenge Awards will be honored and asked to present their winning papers at a public forum in 2018. Awards will be given in the following amounts:

- The winner will receive \$50,000
- The runner up will receive \$25,000
- Third and fourth place will receive \$5,000

Evaluations of specific healthcare interventions will not be supported.

Application Process

The Challenge Award application process has two stages.

- 1 Candidates should submit letters of intent (LOIs) to Eileen Cannon, President, PhRMA Foundation, at Foundation@PhRMA.org no later than May 24, 2018, to initiate the application process. LOIs will be reviewed for potential program fit, novelty, importance, rigor and clarity.
- 2 Qualified applicants will be contacted no later than June 15, 2018, with a request to submit the full paper describing their response to the challenge question. Papers are due August 15, 2018, and will be evaluated by a panel of qualified reviewers.

Award Expectations

The PhRMA Foundation is committed to driving real change in health care delivery and recognizes the benefit of shared knowledge. Therefore, the Foundation will establish a Value Assessment Research Network to encourage collaboration and dissemination of findings borne out of the program.

Recipients of all PhRMA Foundation awards under the Value Assessment Initiative will become members of the Network and be asked to participate in periodic calls or in-person meetings to discuss and drive advancement in the field.

The PhRMA Foundation will host a public forum in 2018 to highlight activities funded by this program. Awardees must be willing and available to present their winning papers at this forum.

Eligibility

Award opportunities are open to all individuals and organizations with training in health economics, outcomes research, clinical sciences or health care evaluation. Eligible applicants should hold an advanced degree in a field of study logically or functionally related to the proposed activities. Collaboration across stakeholder groups and fields of discipline is encouraged.

Letter of Intent and Application Considerations

Evaluating the value of health care interventions is challenging. But, when designed well and used appropriately, tools that quantify the value of a health care treatment can inform decision-making for patients, providers and payers. There are several criteria to consider in developing solutions to drive high-quality value assessment.

Stakeholder Engagement A vital step to a successful shift toward a value-driven health care system is ongoing engagement with stakeholders. It is particularly important to incorporate patient perspectives and acknowledging that all individuals are future recipients of health care and are driving factors of high-quality value assessment. Recommendations for patient engagement processes are made available by the National Health Council.ⁱ

Real-World Applicability All funded activities should generate resources, evidence or ideas that can be applied feasibly in the U.S. health care system. Variations in practice patterns or disparities in care (e.g., demographics, socioeconomic status and type of insurance) should also be acknowledged.

Adherence to Best Practices Proposed methodology should follow relevant, well-accepted recommendations, such as those published by the Agency for Health Care Research and Quality, International Society for Pharmacoeconomics and Outcomes Research, National Pharmaceutical Council Guiding Practices for Patient-Centered Value Assessment or Second Panel on Cost-Effectiveness in Health and Medicine.ⁱⁱ

Review and Validation Research activities should be subject to systematic ongoing validation to ensure that accurate, truthful and non-misleading and reproducible findings are generated. Results should not be disseminated until validated through expert review, with input provided by all relevant and qualified stakeholders. The process of review should be well-documented and accompany the dissemination of the results.

Patient-Centered Decision-Making Value assessment tools create opportunities to support patient-centered decision-making, if patients and other stakeholders are able to review and customize value information based on their own preferences. For example, the second panel on cost effectiveness in health and medicine recommends that all potential consequences of care should be presented in a transparent and disaggregated form, such as in an “impact inventory table”.ⁱⁱⁱ Additionally, all criteria should be quantified and included in assessments, if possible.

Addressing Uncertainty Tools or frameworks that assess care value should adequately explain and address all sources of uncertainty (e.g., in parameter selection, decision process, measurement) and conduct and present relevant sensitivity and scenario analyses.

Submission Components

This program is requesting that prospective candidates submit a letter of intent prior to the submission of a paper. The letter of intent should include the following:

Letter of Intent Components:

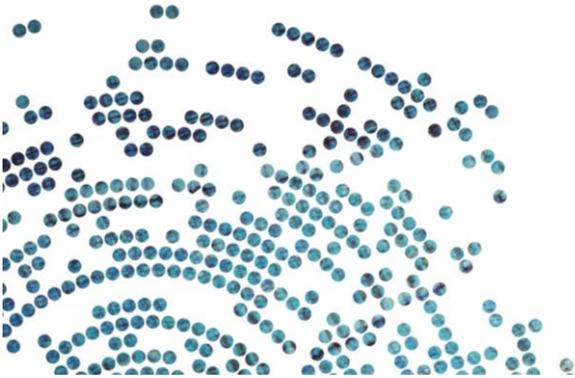
- 1 Descriptive title of proposed paper
- 2 Name, address and telephone number of the applicant(s)
- 3 Names of other key personnel (if applicable)
- 4 Applicant(s) CV or biosketch
- 5 Affiliated or participating institutions (if applicable)
- 6 Proposed response to challenge question, not to exceed 600 words

Paper Submission Components::

- 1 Descriptive title of proposed paper
- 2 Name, address and telephone number of the applicant(s)
- 3 Names of other key personnel (if applicable)
- 4 Applicant(s) CV or biosketch
- 5 Affiliated or participating institutions (if applicable)
- 6 Response to challenge question in a paper suitable for publication, not to exceed 3,000 words

References

- i National Health Council, Patient Engagement. Available at: <http://www.nationalhealthcouncil.org/public-policy/patient-engagement>
- ii Agency for Health care Research and Quality (AHRQ) Guidance for the Conduct and Reporting of Modeling and Simulation Studies in the Context of Health Technology Assessment. Available at: <http://effectivehealthcare.ahrq.gov/index.cfm/search-for-guides-reviews-and-reports/?productid=2315&pageaction=displayproduct>;
International Society for Pharmacoeconomics and Outcomes Research Good Practices for Outcomes Research Practices. Available at: https://www.ispor.org/workpaper/practices_index.asp;
National Pharmaceutical Council Guiding Practices for Patient-Centered Value Assessment. Available at: <http://www.npcnow.org/guidingpractices>;
Sanders GD, Neumann PJ, Basu A, et al. Recommendations for Conduct, Methodological Practices, and Reporting of Cost-effectiveness Analyses: Second Panel on Cost-Effectiveness in Health and Medicine. *JAMA*. 2016;316(10):1093-1103. doi:10.1001/jama.2016.12195.
- iii Sanders GD, Neumann PJ, Basu A, et al. Recommendations for Conduct, Methodological Practices, and Reporting of Cost-effectiveness Analyses: Second Panel on Cost-Effectiveness in Health and Medicine. *JAMA*. 2016;316(10):1093-1103. doi:10.1001/jama.2016.12195.



Request for Proposal

Landscape Analysis of the Integration of Personalized Medicine into U.S. Health Systems

BACKGROUND

To integrate personalized medicine into clinical practice, provider institutions must overcome novel challenges related to adapting to new requirements, processes, standards, and care models associated with the field. The magnitude of these challenges and the capacity to overcome them, however, varies widely among health care delivery institutions with different community demographics, institutional missions, resources, and technological readiness.

Perspectives about personalized medicine vary, and different types of provider institutions have taken different approaches to its implementation in clinical practice. Several pioneering institutions, academic health centers and community hospital systems across the U.S. have implemented coordinated personalized medicine programs. Others have implemented personalized medicine practices for various health conditions in specific instances, without developing a formal personalized medicine program. Still other systems adhere to standards of care without consideration of whether they are practicing personalized medicine. A better understanding of the current landscape for implementation within the U.S. health care delivery system will clarify the extent to which personalized medicine has penetrated health care and help identify areas where integration efforts are most needed.

OBJECTIVE

This project will examine varying perspectives and practices in order to capture a holistic picture of the clinical adoption of personalized medicine strategies and technologies within the U.S health care system by querying provider institutions about baseline community, institutional, and service delivery details as well as practice patterns and viewpoints related to personalized medicine and its utilization. This, in turn, can help inform efforts to address the most critical outstanding integration challenges. The landscape analysis will include a representative sample of U.S. health care delivery institutions and could include both quantitative and qualitative results to ensure that a U.S. health system-wide picture of the integration of personalized medicine is captured.

RESEARCH OUTLINE

The project, to be completed in 4 - 6 months, will involve a short survey and/or a series of interviews of U.S. health care delivery systems and subsequent analysis that will help demonstrate the current landscape of personalized medicine integration.

The survey/interview strategy can include different levels of questions based on responses.

The first level of questions could involve a simple survey designed to show basic perspectives of personalized medicine, and to what extent personalized medicine is being integrated into practice (i.e. the health system has a personalized medicine program, the system is applying a personalized medicine approach for some health conditions or circumstances, the system follows standards of care without consideration of personalized medicine approaches, etc.) at organizations in different demographic, institutional, and community settings.

Subsequent levels of survey/interview questions could be designed to show what implementation approaches and what technologies are routinely being utilized at institutions that are more advanced in personalized medicine.

The project proposal will outline a clear strategy to ensure a representative sample of respondents from urban, suburban, rural and otherwise diverse community settings.

Where possible, analysis should break down survey results by institutional setting and/or community demographics.

The survey should examine personalized medicine practices across service lines and should not be restricted to any particular disease area.

Survey/interview questions related to perspectives and practices should refer to an established definition of personalized medicine (for example, the PMC's definition of personalized medicine published in "the Case for personalized Medicine" 2014) but allow for and perhaps capture different perspectives as to what constitutes personalized medicine at different health care institutions.

A project steering committee will work with the research team. The committee will consist of health care delivery experts with experience in personalized medicine implementation and, where possible, in developing surveys and analyzing community data. The committee will contribute to strategic planning to ensure a representative sample of survey respondents, help define the survey instrument, guide the project, and review progress at various milestones.

The results of the survey will be submitted for publication in a peer-reviewed journal and published in a PMC project summary document that describes the current landscape of perspectives, practices, and institutional program implementation regarding personalized medicine. The materials may also be presented at relevant scientific, clinical practice, and policy meetings. Target audiences include providers, health care delivery support organizations, pharmaceutical and diagnostic manufacturers, researchers, and policymakers.

FUNDING

The total project budget will be determined based on estimates from submitted proposals and should not exceed \$50-\$75K. Proposals should include estimated budget requirements and timing of study and publication milestones. A separate budget line should be included for estimated dissemination and presentation costs that go beyond submission of a manuscript. Proposals will be rated based on study merit and design.

SUBMISSION

Proposed research concepts should provide an explanation of all project objectives, a plan to ensure a representative survey sample, an overview of the analytical approach, and a description of the project's overall

value. Submissions will be evaluated based upon a combination of factors, including the potential impact of the research; duration of the research; certainty of outcome; how the research can be presented; and to what extent the research is important to key audiences. Submissions should generally be between 3 - 5 pages, with the possibility to expand and revise after initial review.

Interested institutions should contact Daryl Pritchard at dpritchard@personalizedmedicinecoalition.org.

About the Personalized Medicine Coalition

The Personalized Medicine Coalition (PMC), headquartered in Washington, DC, representing innovators, scientists, patients, providers and payers, promotes the understanding and adoption of personalized medicine concepts, services and products to benefit patients and the health system.



June 15, 2018

Tamara Syrek Jensen, J.D.
Director, Coverage & Analysis Group
Centers for Medicare & Medicaid Services
Mailstop S3-02-01
7500 Security Blvd.
Baltimore, MD 21244

Sent electronically

RE: Medicare National Coverage Analysis for Chimeric Antigen Receptor (CAR) T-cell Therapy for Cancer (CAG-00451N)

Dear Ms. Syrek Jensen:

The Personalized Medicine Coalition (PMC), a multi-stakeholder group comprising more than 230 institutions across the health care spectrum, appreciates the opportunity to submit comments regarding the Centers for Medicare & Medicaid Services (CMS)' National Coverage Analysis (NCA) for Chimeric Antigen Receptor (CAR) T-cell Therapy for Cancer.¹ While we recognize that a national coverage policy could provide some uniformity in the path to coverage for FDA-approved CAR T-cell therapies, the timing of this NCA may be premature and could complicate beneficiary access.

Personalized medicine is an evolving field that uses diagnostic tools to identify specific biological markers, often genetic, to help determine which medical treatments and procedures will be best for each patient. By combining this information with an individual's medical history, circumstances, and values, personalized medicine allows doctors and patients to develop targeted prevention and treatment plans.

Personalized medicine is helping to shift the patient and provider experience away from trial-and-error and toward a more streamlined process for making clinical decisions, which will lead to improved patient outcomes, a reduction in unnecessary treatment costs, and better patient and provider satisfaction. As noted above, PMC's members are leading the way in personalized medicine and recommend that patients who may benefit from this approach undergo appropriate testing and tailored treatment as soon as possible during their clinical experiences.

PMC recently released "*Personalized Medicine at FDA: 2017 Progress Report.*" The report documents a record number of new personalized medicines approvals by the

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U.S. Food and Drug Administration (FDA). This marks the fourth consecutive year that personalized medicines accounted for more than 20 percent of all new drug approvals.ⁱⁱ Two of the 19 personalized medicines approved in 2017 were CAR T-cell therapies, an adoptive cell transfer (ACT) class of immunotherapies where a patients' immune cells are collected and genetically changed to treat his/her cancer. Because of its potential to further transform cancer care for patients with incurable blood cancers, the American Society of Clinical Oncology named ACT using CAR T-cells their 2018 "Advance of the Year."ⁱⁱⁱ

CAR T-cell therapy is the result of decades of research in biology, genetics and immunology supported by the National Institutes of Health and the biopharmaceutical industry. A study conducted by the Massachusetts Institute of Technology NEWDIGS FoCUS Project predicts that by the end of 2022 there will be about 40 gene therapy products approved by the FDA, with 45 percent of these for products targeting cancer.^{iv} In light of this entirely new approach to treatment the FDA has developed new structures for evaluating cell and gene therapies and has demonstrated remarkable speed in bringing CAR T-cell therapies to market. Like FDA, CMS has signaled a belief that CAR T-cell therapies represent a significant advancement in cancer care and we appreciate the opportunity to provide feedback on how CMS can ensure continued beneficiary access to CAR T-cell therapies.

Statement of Neutrality

Many of PMC's members will present their own responses to CMS and will actively advocate for those positions. PMC's comments are designed to provide feedback so that the general concept of personalized medicine can advance, and are not intended to impact adversely the ability of individual PMC members, alone or in combination, to pursue separate comments with respect to the NCA on CAR T-cell therapies for cancer.

Burden of Disease and Treatment with CAR T-cell Therapies

The two CAR T-cell therapies approved by the FDA in 2017 are intended to treat children and young adults with acute lymphoblastic leukemia (ALL) and adults with diffuse large B-cell lymphoma (DLBCL). ALL and DLBCL are cancers with a very poor prognosis.

The expected survival for adults with relapsed ALL is less than six months. Among children with cancer, relapsed ALL is a leading cause of cancer-related death.^v Treatment with CAR T-cell therapy for ALL resulted in complete remissions in up to 90 percent of adults and children with this disease. In contrast, other available treatments for relapsed ALL have a response rate of 30 percent to 43 percent, depending on the type of treatment. In some patients, remissions have lasted up to two years. Such durable remissions offered many patients the chance to undergo bone marrow transplants, a procedure that can be curative.^{vi}

DLBCL is the most common type of non-Hodgkin lymphoma. In a multicenter clinical trial of patients with DLBCL that worsened after at least two prior therapies, the cancer responded to a CAR T-cell therapy called tisagenlecleucel in 59 percent of 51 patients and the cancer went into remission in 43 percent of patients. At 6 months, 79 percent of patients had not had a recurrence of lymphoma. In a different clinical trial, patients with relapsed or refractory DLBCL, refractory primary mediastinal B-cell lymphoma, or transformed follicular lymphoma, received another CAR T-cell therapy, axicabtagene

ciloleucel. Among the first 92 patients who were treated, the response rate was 82 percent, with complete remissions occurring in 54 percent of patients.^{vii}

CAR T-cell therapies have made a profound impact in extending the lives of some people with ALL and DLBCL, however this course of treatment can have serious side effects that are still under investigation. Side effects include high fever, chills, flu-like symptoms, neurological changes, infection, low blood cell counts, and a weakened immune system. Oncologists who administer CAR T-cell treatments often use the immune-suppressing drug tocilizumab, which was approved by the FDA to manage adverse reactions like these, but it does not always reverse complications. Researchers are actively working to understand the causes of adverse reactions like these and exploring methods to further mitigate them. One study is examining the use of anakinra, prior to administration of CAR T-cells to target neurotoxicity.^{viii}

Researchers are also pursuing CAR T-cell therapies in other types of cancer. There have been promising results, especially in multiple myeloma. An early clinical trial of patients with relapsed, treatment resistant multiple myeloma reported that 33 out of 35 (94 percent) patients demonstrated clinical remission upon receiving a CAR T-cell therapy targeting B-cell maturation protein. In this study most patients had only mild side effects.^{ix}

Considerations for CMS in Exploring National Coverage

Tremendous advancement in CAR T-cell therapy should be supported by a thoughtful reimbursement structure. Unfortunately, the complexity of CAR T-cell treatment has created a daunting reimbursement landscape for patients and providers to navigate.

Medicare currently limits settings where beneficiaries can receive CAR T-cell treatment due to the acute nature of treatment side effects, Medicare coding and claims procedures do not sufficiently cover the upfront cost to hospitals that provide care for patients with these novel therapies, and some providers are excluded from administering CAR T-cell therapy altogether. We recognize that CMS recently proposed improvements that would address some of these access concerns in its Medicare Hospital Prospective Payment System Proposed Rule for 2019. PMC will be submitting comments separately on the proposed changes.

Manufacturers of the FDA-approved CAR-T therapies have responded to the current patchwork of coverage by developing programs that provide financial assistance to patients and their caregivers. These programs help cover co-pays and transportation costs so that patients can receive care in facilities that administer CAR T-cell therapy. Solutions like these are making CAR T-cell therapies available to some patients, as all stakeholders work to ensure that these therapies are accessible to all patients who may benefit from them.

Despite its complexity we believe this reimbursement landscape can be best shaped through ongoing, robust stakeholder dialogue that is already occurring outside of the NCD process. CMS should continue efforts to educate providers and others in the healthcare community on these innovative products and instruct its Medicare Administrative Contractors and providers on how to cover and process claims. If CMS advances a proposed national coverage policy for CAR T-cell therapies we respectfully ask you to consider the following:

- FDA Commissioner Scott Gottlieb has stated that the field is at a turning point and the FDA may have to accept more uncertainty for pre-market approval of cell-based therapies like CAR T.^x In instances like these, CMS should work with biopharmaceutical companies to begin conversations about data collection as early as possible in product development process to establish what evidence will be necessary for coverage upon FDA approval. Data collection and reporting requirements should not create unnecessary burdens for patients and providers.
- Coverage policies are often based on treatment criteria and clinical guidelines. This approach may not be feasible for CAR T-cell therapies given their personalized nature. The lack of treatment criteria and clinical guidelines has been problematic for some commercial payers in determining coverage for CAR T-cell therapies. CAR T-cell therapies are transformative and address the individual needs of patients who are often without other options. A Medicare national coverage policy should not create the same barrier for patients and providers in the commercial market when the biological rationale for treatment exists but treatment criteria are still evolving.
- CAR T-cell therapies may have certain attributes at the time of FDA approval but there are specialized considerations for delivery of each therapy. A national coverage policy would need to be flexible enough to keep pace with new research that produces results to reduce treatment complications or yield other improvements in patient response to CAR T-cell treatment.

Conclusion

Thank you for considering our comments. If CMS moves forward with developing a proposed decision memo on CAR T-cell therapy for cancer, PMC welcomes the opportunity to serve as a resource for you. We hope any additional activity by the CMS on this issue can achieve the goal PMC shares with the agency of delivering appropriate, efficient, and accessible health care to every patients. If you have any questions about the content of this letter, please contact me at 202-589-1769 or cbens@personalizedmedicinecoalition.org.

Sincerely,



Cynthia A. Bens
Senior Vice President, Public Policy

CC: Katherine B. Szarama, Ph.D.
Lead Analyst
Centers for Medicare & Medicaid Services

Lori A. Paserchia, MD
Lead Medical Officer
Centers for Medicare & Medicaid Services

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- ⁱ Centers for Medicare & Medicaid Services. *National Coverage Analysis for Chimeric Antigen Receptor (CAR) T cell Therapy for Cancer*. May 16, 2018 <https://www.cms.gov/medicare-coverage-database/details/nca-tracking-sheet.aspx?NCAId=291>
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