HAYSTACK PROJECT
The Voices of Rare & Ultra Rare

VIA ELECTRONIC SUBMISSION
January 25, 2021

Seema Verma, Administrator
Centers for Medicare & Medicaid Services
Department of Health and Human Services
7500 Security Blvd
Attention: CMS-5528-IFC, Mail Stop C4-26-05
Baltimore, MD 21244

RE: CMS-5528-IFC; Most Favored Nation Model
Dear Administrator Verma:

Haystack Project appreciates the opportunity to comment on the Centers for Medicare & Medicaid Services' (CMS') Interim Final Rule with Comment Period (IFC) entitled "Most Favored Nations Model" (the MFN Model).

Haystack Project is a 501(c)(3) non-profit organization enabling rare and ultra-rare disease patient advocacy organizations to coordinate and focus efforts that highlight and address systemic reimbursement obstacles to patient access. Our core mission is to evolve health care payment and delivery systems with an eye toward spurring innovation and quality in care toward effective, accessible treatment options for Americans living with or caring for someone with a rare or ultra-rare condition.

Haystack is concerned that, if implemented, the MFN Model would represent an unprecedented expansion of CMS' use of the Center for Medicare and Medicaid Innovation (CMMI) to circumvent statutory provisions for Medicare Part B drug payment, without offering any real potential for maintaining, much less improving, patient access to care and health outcomes. Haystack has previously expressed its significant concerns that CMMI's effective implementation of innovation strategies to enhance care for individuals with rare diseases is substantially complicated by the risk of unintended consequences to these populations. Models like this one are designed as broad, one-size-fits-all initiatives to cut Medicare expenditures, and pose a heightened risk of unintended, disproportionate, and potentially profound impacts on patients with rare and ultra-rare disease and rare cancers.

Moreover, the MFN Model's adverse impact on Medicare patients generally cannot be characterized as potential or unintended. CMS clearly stated its projection that the MFN Model would broadly impact patient access to Part B drugs. This will leave beneficiaries scrambling to seek care from providers that somehow are able to obtain and administer treatments within the narrow financial margins the model presents. CMS acknowledges that some, and potentially many patients, may be unable to do this. Medicare's elderly and disabled population is disproportionately comprised of individuals with multiple chronic conditions, living with complex interactions between these conditions and their treatments. It would be difficult to imagine a population less suited to studying impacts of a drug pricing model that has never been implemented in the US and completely diverges from the nation's distribution and pricing realities. Without robust evidence clearly confirming that patient care, treatment options, and outcomes would not be adversely impacted for ANY patients, this model was not likely contemplated under the CMMI enabling statute. Instead, it looks much more like a federal Agency actively experimenting on a vulnerable patient population.

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Haystack urges CMS to abandon implementation of the MFN Model. Our comments:

- Provide a brief description of the unique challenges patients suffering from rare and ultra-rare conditions and rare cancers experience. We urge CMS to consider the impact that any CMMI model test would have on our patients.
- Urge CMS to limit use of the waiver authority granted to CMMI model tests to innovations in payment and care delivery that would achieve cost savings by enhancing patient care and improving outcomes through improved efficiency and care coordination.
- Identify patient protection guardrails that must be incorporated into CMMI model tests to ensure that CMS continues to fulfill its responsibilities as steward of the Medicare trust fund.

Medicare beneficiaries with rare and ultra-rare diseases and rare cancers are a unique subpopulation that disproportionately suffer from unintended consequences of health care policy changes and refinements.

Haystack patients and their clinicians continually struggle with payment systems and mechanisms designed to address more common conditions. Overcoming access obstacles requires focus, persistence, and a significant time commitment by our patients, their families and caregivers. Anything that reduces provider payments or otherwise erects new obstacles to care can represent the tipping point on access so critical to our patients.

As you know, Congress tackled the incentive framework for orphan drugs to counter the commercial realities associated with research and development toward treatments for serious medical conditions affecting small populations. Countless lives have been improved, or saved by new therapies since then. Although, millions of Americans affected by a rare disease are still waiting and hoping for treatment or a cure, there are many for whom treatments that are already here or in the pipeline cannot reach patients due to the realities of today's reimbursement structures.

The economic calculation of research and development costs, projected risk, and population-based revenue estimates must be accompanied by successfully clearing reimbursement mechanisms and hurdles that tip the scales for or against pursuing a specific drug candidate for an orphan indication. For patient populations approaching the 200,000-patient orphan disease limit, current incentives may be sufficiently robust to mitigate clinical trial and reimbursement risks. However, as affected populations dwindle below 20,000 or even into and below the hundreds, the balance can be far more fragile, and risks or uncertainties can discourage the investor interest required by smaller companies to take product candidates from bench to market.

Patients needing existing treatments, and those living with the hope that their currently-untreatable conditions will be addressed, should be able to rely on CMS and CMMI to ensure that they have the same level of access to treatment as patients with more common conditions have.

CMS should limit use of CMMI model test waiver authority to innovations in payment and care delivery that would achieve cost savings by enhancing patient care and improving outcomes through improved efficiency and care coordination.

Haystack understands the concerns driving CMS' interest in pursuing the MFN Model, i.e., that Medicare and US patients should have a level playing field with other nations with respect to drug pricing.

Unfortunately, CMS' MFN Model ignores the clear challenges inherent in simply identifying a "most favored nation" price and capping provider reimbursement based upon that price. The Medicare program is structured to reflect our nation's focus on patient health care needs. It is premised on the overriding goal of ensuring that America's most vulnerable patients retain access to treatment options, and on the autonomy in patient/physician decision making. America's elderly and disabled populations are, by statute, entitled to rely on Medicare to receive a level of access to promising treatment options that well exceeds their counterparts in many, if not most, parts of the world. International drug prices are, as CMS noted, variable. Access to higher-cost treatments is similarly variable - the negotiating power some nations achieve by simply refusing to pay for a promising new treatment due to its cost is repugnant to most Americans, regardless of whether or not it would eventually result in deep discounts. The MFN Model, reduced to its simplest form, uses Medicare beneficiaries and their health as a bartering tool to secure reduced drug prices for all US patients.

Haystack does not believe CMS is precluded from exploring ways to reduce expenditures or encourage cost-effective medical care. Section 1115A of the Social Security Act aligns with Medicare's overriding goals of ensuring treatment access and patient-focused care while providing the authority and framework for CMMI to incorporate "value" into care delivery and payment models. The authority to waive provisions of the Medicare law to test models uniquely positions CMMI to take the lead in moving Medicare toward value rather than volume. Two key components of CMMI models that justify waiving existing law are:

- identification of patient populations with care deficits and discrete interventions likely to improve outcomes at reduced costs; and
- the ability to evaluate the effectiveness of models through patient-centered outcomes.

Haystack strongly believes that CMMI models are intended to be well-designed research studies that foster program improvements without presenting a risk to patients.

Section 1115A's reliance on patient-centeredness in evaluating models similarly underscores the importance of the patient perspective in initial model selection and design. Where identified populations experience care deficits, CMMI models should focus primarily on addressing the care deficits rather than cost-containment; where avoidable costs are identified, models must be crafted to avoid compromising care. Put simply, CMMI's mandate implies that, at a minimum, model tests should do no harm.

This interpretation is supported by the exemption from budget neutrality Congress explicitly extended to CMMI's Section 1115A models during a Phase 1 model test. The statute clearly and intentionally, empowers CMMI to address care deficits in Phase I model tests without the condition-precedent of affirmatively demonstrating budget neutrality. Haystack believes this provision demonstrates recognition that when identified populations suffer care deficits, increased downstream care costs are virtually certain. These costs, however, may evade the precision required to establish budget neutrality, particularly given that costs are assessed from a short-term perspective.

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Haystack's patient and caregiver community urges CMS to:

- Focus on subpopulations with care deficits potentially leading to poor outcomes for which models could enhance care, and ensure that patients with rare disorders and their needs are addressed;
- Identify outcomes that patients care about to inform structure, design, and patient-centered outcomes for model evaluation, and include evaluation parameters within each proposed CMMI model;
- Encourage early and continuous engagement of patients with rare and ultra-rare conditions and rare cancers throughout the model selection and testing continuum;
- Further the development and use of patient-reported and patient-identified outcomes; and
- Ensure that economic inputs are considered in the context of the patient's experience and reflect long-term patient outcomes.

We strongly believe that a system-wide CMMI culture of patient engagement will illuminate the risk of unintended consequences that models may have on patient access to care, safety, or outcomes during the proposal phase when they can be avoided or mitigated. This is particularly important for Haystack's patient and caregiver community given that impacts on very rare disorders may not be readily apparent to CMS, or even to providers. When CMMI model tests cannot be implemented without an adverse impact on patients with rare and ultra-rare conditions and rare cancers, the model should be implemented to exclude these patients.

Patient protection guardrails must be incorporated into CMMI model tests to ensure that CMS continues to fulfill its responsibilities as steward of the Medicare trust fund.

Most CMMI models to date have been designed to improve patient outcomes while reducing costs, and have incorporated patient safeguards designed to mitigate any risks associated with the model tests. These mechanisms have included evaluation measures to assess service utilization, patient experience, and psychosocial factors and their impact on quality throughout the model test. Models have also included notification requirements and the opportunity for patients to opt out without changing providers.

Haystack also notes that CMS has previously expressed an interest in abandoning the concept of mandatory models in favor of testing innovations that foster a fully functioning, competitive market and enable win/win solutions across all stakeholders. We firmly believe that patients must be informed of the existence, contours, and potential impact any particular model may have on their healthcare access and treatment decisions, and have the opportunity to opt-out of participation without the fragmentation of care that accompanies switching providers.

Haystack strongly urges CMS to maintain a "patients first" focus by approaching each model proposal and test with the threshold questions of:

- what are the associated risks across the patient populations we would study,
- what additional or distinct risks are presented for patients with rare and ultra-rare disorders and rare cancers; and
- is the risk of harm for **all** patients sufficiently negligible to require patients to participate in the research.

These threshold questions and CMS' understanding of the relevant answers should be set forth in the notice and comment process associated with each model proposal. Any inability to achieve consensus on identifying and/or quantifying patient risk should give CMS pause and, at a minimum, justify a patient-centered opt-out consistent with CMS' long-standing standards of shared decision making and informed consent in connection with medical care. This is particularly important for individuals with rare and ultra-rare conditions and rare cancers for whom access hurdles and treatment delays can have enormous, life-altering, and potentially life-threatening, consequences.

Haystack has no doubt that the MFN Model presents the types of changes that patients would want to know about and fully understand; we also believe that, once informed, a substantial cohort of beneficiaries would, if possible, opt out of the MFN Model due to concerns about its impact on their access to care. We urge CMS to seriously consider the implications of nationwide, mandatory models that fail to fully inform Medicare beneficiaries that they are participating in a research study that could impact treatment options and access to care.

For individuals with rare disorders, model tests can inject a great deal of uncertainty, and to the extent that unintended consequences impact care access, the impact is likely greatest for extremely rare conditions. We urge CMS and CMMI to keep patients with rare conditions and their healthcare needs top-of-mind with a guiding principle reflecting a "first, do no harm" approach to considering and accounting for impact to all patients, including those with rare diseases. Similarly, where, as with serious, rare disorders, care deficits can lead to poor outcomes, we believe that CMMI activities must align with the primary goal of improving care. All models must be evaluated with a focus on patient-centered outcomes.

Similarly, a primary focus on cost reduction in selecting and evaluating models also creates an environment that inherently disfavors conditions and treatment options in direct proportionality to disease rarity and/or treatment costs. As a threshold matter, it is virtually impossible to reliably benchmark costs associated with treating patients with extremely rare disorders, and even more so if the patient suffers from one or more additional chronic conditions. Even when CMMI model tests are designed holistically and incorporate care coordination and management strategies, cost-reduction mechanisms or evaluation measures may disproportionately disadvantage rare disease patients and their providers:

- 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 extremely rare disorder, so that these conditions are often grouped with similar disorders within an ICD-10 code;
- The relatively small population size for extremely rare disorders precludes availability of clearly articulated, scientifically-validated treatment standards that would form the basis of a reliable benchmark;
- Patients with extremely 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 to perform poorly with respect to care cost rather than as a high-quality clinician providing efficient care.

We urge CMS to devise structural exceptions for rare disease patients when innovation models focus on cost-reduction or create provider incentives based on cost-containment or reduction. Clinicians treating Medicare patients must be empowered to use their clinical judgment to diagnose, refer, and treat patients, including administration of Part B drugs and prescribing of Part D drugs, without risk of financial consequences. Innovation in care delivery and payment should not disrupt the commitment the Medicare program has long maintained – that beneficiaries are covered for medically-necessary treatments whether their disease is common and its treatment cost low, or their disease is extremely rare with one, costly, available treatment.

Conclusion

Once again, Haystack appreciates the opportunity to offer our comments to the MFN Model. We look forward to working with you to ensure that innovations toward treating and curing rare and ultra-rare disorders and rare cancers reach the patients who need them. If you have any questions or need additional information, please contact Saira Sultan at 202-360- 9985.

Sincerely,

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mds foundation the myelodysplastic syndromes foundation, inc.

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ican international cancer advocacy network the virtual lifeline for proactive cancer patients

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