www.HaystackProject.org

November 3, 2022

Re: Centers for Medicare and Medicaid Services' Request for Information: Make Your Voice Heard: Promoting Efficiency and Equity Within CMS Programs

In response to CMS' request for information titled Make Your Voice Heard: Promoting Efficiency and Equity Within CMS Programs, the Haystack Project has submitted the following responses. CMS's RFI sought to gather public input on accessing healthcare and related challenges, understanding provider experiences, advancing health equity, and assessing the impact of waivers and flexibilities provided in response to the COVID-19 Public Health Emergency (PHE). CMS will use the comments received in response to this RFI to identify potential opportunities for improvement and increased efficiencies across CMS policies, programs, and practices. In addition, CMS hopes to learn how specific policies have benefited providers, practices, and the people we serve as we work to continually improve our programs.

1. Accessing Healthcare and Related Challenges

CMS wants to empower all individuals to efficiently navigate the healthcare system and access comprehensive healthcare. We are interested in receiving public comment on personal perspectives and experiences, including narrative anecdotes, describing challenges individuals currently face in understanding, choosing, accessing, paying for, or utilizing healthcare services (including medication) across CMS programs.

Examples may include, but are not limited to:

- Identifying CMS policies that can be used to advance health equity:
  - Challenges accessing comprehensive and timely healthcare services and medication, including primary care, long-term care, home and community-based services, mental health and substance use disorder services;
  - Challenges in accessing care in underserved areas, including rural areas;
  - Receiving culturally and linguistically appropriate care (e.g., tailoring services to an individual's culture and language preferences);
  - Challenges with health plan enrollment;
  - Challenges of accessing reproductive health services;
  - Challenges of accessing maternal health services;
  - Challenges of accessing oral health services and the impact on overall health;

- Understanding coverage options, and/or technology to support access to coverage; and,
- Perspectives on how CMS can better communicate quality standards and accessibility information to individuals, particularly those with social risk factors.

Haystack Project Response:

Haystack urges CMS to consider the unique challenges faced by patients with rare diseases. Approximately 7K rare diseases have been identified to date, 90-95% have no FDA-approved treatment.

- 80% of rare diseases are genetic and present throughout a person's life
- Approximately 50% of people affected by rare diseases are children
- 30% of children with a rare disease will not live to see their 5th birthday

While each rare disease, by definition, impacts a patient population of under 200,000, rare diseases cumulatively affect approximately 30,000,000 or 1 in 10 individuals in the U.S. A 2021 Report to Congress GAO entitled "RARE DISEASES: Although Limited, Available Evidence Suggests Medical and Other Costs Can Be Substantial" was compiled to assess the challenges and costs rare disease patients face accessing diagnostic and treatment services. The Report found that diagnostic delays place patients at high risk for compromised health outcomes, including disease progression, exposure to inappropriate interventions, emergence of comorbidities, and even death. They are also costly. The diagnostic journey is particularly long and complex. According to an economic study which included a survey of 1360 patients with 379 rare diseases cited to in the GAO Report, patients:

- Saw an average of 4.2 primary care physicians and 4.8 specialists for their diagnosis
- Made an average of 2.4 out-of-state trips related to their diagnosis
- Visited an emergency room an average of 3.7 times and
- Were hospitalized an average of 1.7 times for reasons related to their rare disease and prior to diagnosis.

The challenges common to rare disease patients can be an overwhelming burden for people of color and other underserved populations, including rural communities. Communities of color face significant disparities in symptom severity, disease progression and mortality for rare diseases such as systemic lupus erythematosus and myasthenia gravis even though these conditions tend to occur across populations. Rare diseases such as sickle cell anemia, thalassemia, sarcoidosis disproportionately impact people of color. The growing number of beneficiaries with sickle cell disease (SCD) are primarily young, medically complex, and likely impacted by social determinants of health.

- 75.5% utilized the emergency department, and 59.3% had an inpatient stay
- Hospital utilization was higher for individuals aged 18-45
- Common comorbidities for SCD include:
  - hypertension (65.8%)
  - fibromyalgia (64.9%)

    - depression (51.3%)
    - chronic kidney disease (47.0%).
- over 70% of Medicare FFS beneficiaries with SCD were dual-eligible
- over 80% are under 65 years of age.

Medicare's SCD patients' experience within the opioid epidemic illustrates policy inequities as well as the high potential for unintended harms when new population-level initiatives are implemented. CMS' policy to curb the opioid abuse crisis was firmly grounded in public policy imperatives and proactively excluded cancer patients. SCD patients experienced access hurdles and denials of adequate pain management treatment until CMS recognized that "[t]he complex nature of SCD pain management may be exacerbated by ongoing efforts to address the opioid epidemic" and determined to exclude SCD patients from opioid restrictions.

Accordingly, even the most well-meaning policies can have unintended consequences for rare disease patients. Patients with other rare diseases, especially those impacting very small numbers of patients like porphyria also manifest with acute episodes of pain requiring opioids and yet remain without reliable access to the pain management care they need. The rarer the condition, the more likely policymakers will not see the unintended consequences of otherwise well-reasoned policies.

In addition, most rare disease patients have no FDA-approved treatment for their condition. These patients rely on off-label treatments within evolving standards of care. Although these treatments are needed to reduce the burden and/or progression of disease symptoms, they are unlikely to be listed within CMS-accepted compendia and even less likely to be added to a treatment's label. Coverage restrictions, step therapy protocols, and prior authorization requirements can be prohibitively burdensome for providers and too confusing for patients and their advocates to navigate. Moreover, patients changing plans or payers often have to re-navigate these processes to remain on their prescribed treatment regimen. Given the challenges associated with developing treatments for small populations, FDA-approved treatments for very rare diseases are often costly and rely on accelerated approval based on small study populations and surrogate endpoints.

Recommendations for how CMS can address these challenges through our policies and programs.

Response:

The preference for focusing on common conditions permeates our health system from provider education through the population-level priorities that drive health policy. While this approach may appear pragmatic it drives unduly-long diagnostic journeys for rare disease patients. In addition, reimbursement policies frequently exact unintended burdens on the health and lives of our patient communities.

Haystack project has serious concerns that CMS' renewed interest in opening the National Coverage process to implement coverage with evidence development (CED) for newly-approved treatments could have dire consequences for rare and ultra-rare disease patients. Patients now relying on off-label therapies would likely have no covered treatment options if their prescribed

drugs had been subject to CED. Patients fortunate enough to have an available FDA-approved option in development would have their hope tempered by concerns that their only access path could be through clinical trials at distant provider sites. Haystack also has the more general concern that when CED is directed at FDA-approved drugs, it becomes an inflexible utilization management tool, beneficiaries become research subjects, and treatment "decisions" are subjected to randomization and even "blinding" on the precise intervention. CED does not simply enable access to promising treatments. Used in the context of FDA-approved drugs, it conditions access to safe and effective treatments on factors beyond the patients' control (clinical trial availability, eligibility, and randomization) and their willingness to place their care into the hands of researchers rather than the clinicians managing their condition(s).

We have serious concerns that any CED NCD for an approved therapy will place CMS' assessment of benefits versus risks above the very personal decisions on use of FDA-approved treatments that should be inherently within the practice of medicine and the patient/physician relationship. We continue to urge CMS to prioritize beneficiary protections and access over its interest in research as a condition of coverage and believe that the Agency should comply with all requirements ordinarily imposed on entities conducting research in human subjects, including submission of any CED design that would deny coverage to patients based on study enrollment (either through randomization or nonparticipation) to an Investigational Review Board (IRB). Haystack similarly urges CMS to create an alternative coverage pathway for Medicare beneficiaries who are unable to participate in a CMS-approved clinical trial (or are unwilling to provide informed consent) but seek coverage for use within the FDA-approved labeled indication or a medically accepted off-label use. Failing to do so expands the "control" population and injects a clear and impactful coercive element to informed consent. We similarly urge CMS implementation of a monitoring function over all studies to ensure that randomization of research subjects ceases when likely clinical benefit is shown in a manner generally sufficient for claim-specific payment by a Medicare Administrative Contractor (MAC).

Haystack also strongly urges CMS to establish a rare and ultra-rare disease Ombudsman to ensure that rare and ultra-rare disease patients are not subject to barriers in accessing meaningful, quality coverage for their unique healthcare needs. This would include identifying patient concerns regarding transition across plans/payers and from childhood to adulthood, access to specialists with disease-specific expertise as well as on- and off-label treatments identified by those experts as within the standard of care. We continue to believe that this is necessary to address patient-specific access impediments, identify systemic access hurdles and inequities, and ensure that newly-advanced initiatives are informed by the experience and concerns of the rare disease community.

2. Understanding Provider Experiences

CMS wants to better understand the factors impacting provider well-being and learn more about the distribution of the healthcare workforce. We are particularly interested in understanding the greatest challenges for healthcare workers in meeting the needs of their patients, and the impact of CMS policies, documentation and reporting requirements, operations, or communications on provider well-being and retention.

Examples may include, but are not limited to:

- Key factors that impact provider well-being and experiences of strained healthcare workers (e.g., compassion fatigue, retention, maldistribution);
- The increasing use of digital health technology on provider well-being and attrition;
- Feedback regarding compliance with payment policies and quality programs, such as provider enrollment requirements on healthcare worker participation in underserved populations, and what improvements can be made;
- Impact of CMS policies on patient panel selection, and on providers' ability to serve various populations; and
- Factors that influence providers' willingness or ability to serve certain populations, particularly those that are underserved and individuals dually eligible for Medicare and Medicaid.

Response:

While Haystack represents patients with rare diseases, we are uniquely positioned to also advocate for the providers who specialize in the conditions of our members. Of particular concern is equitable payment of providers who undertake the challenge of treating these conditions. Many CMS and private payer policies are not designed to consider the unique challenges involved in addressing rare diseases. This is especially true in the Medicare Shared Savings Program and the Quality Payment Program. Mechanisms that incentivize high-quality, cost-effective care in the general population can present strong disincentives to providing the testing, treatments, and provider oversight required to adequately manage rare and ultra-rare diseases.

While Haystack generally supports initiatives that incentivize clinicians to provide efficient, cost-effective, high-quality care. Individuals with rare and ultra-rare diseases are particularly vulnerable to changes in how care is received as well as provider reimbursement; We are concerned that reimbursement mechanisms that, like the Shared Savings Program, shift risk to clinicians, could have unintended consequences to individuals with rare conditions for which benchmark costs are unavailable. Although we recognize that Medicare program savings can result from aligning incentives toward reducing costs associated with common health conditions, we have learned that these frameworks tend to discourage use of the resources required to diagnose and treat individuals with rare and extremely rare diseases.

Haystack Project continues to advocate for specific carve-outs applicable to rare disease patients as well as incentives to reward timely diagnosis, treatment planning, and care coordination. We believe that a carve-out is a pragmatic mechanism given that it is virtually impossible to reliably benchmark costs associated with treating Medicare beneficiaries with rare disorders, and even more so if the patient suffers from one or more additional chronic conditions. In addition:

- 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.
- The relatively small population size for many rare disorders precludes availability of clearly articulated, scientifically-validated treatment standards that would form the basis of a reliable benchmark.

- Patients with 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.
- Highly-specialized clinicians with expertise sufficient to manage the whole patient would appear to perform poorly when compared to clinicians managing more common conditions within the same specialty.
- Last year's GAO report cited a number of relevant findings that complicate rare disease care within a value-based payment model such as the the Shared Savings Program:
- **Overlap with other diseases.** Rare disease symptoms are often non-specific and overlap with more common diseases. Patients not only face long diagnostic journeys, but often receive costly and potentially toxic treatments due to misdiagnoses.
- **Lack of clinician knowledge**. Because signs and symptoms of many rare diseases are not fully described or understood, patients and clinicians may fail to note significance of initial symptoms or discount patient/caregiver reports.
- **Multiple disease presentations**. Many rare diseases are without a single set of symptoms and are associated with symptom variability on an individual level as well as over time. Other rare conditions can impact multiple organ systems leading to care from multiple specialists before a correct diagnosis is made.
- **Comorbid conditions**. Comorbid conditions inject an additional layer of diagnostic complexity, particularly if the patient has two or more rare diseases. The GAO Report cited the example of acromegaly, a hormonal disease commonly accompanied by diabetes or cardiovascular disease. Acromegaly has a slow progression so that This rare disease follows a slow progressive course, so that individuals are generally not correctly diagnosed until they present with advanced disease and multiple comorbidities.

Recommendations for CMS policy and program initiatives

Response:

We urge CMS to devise an exception that would permit clinicians participating in two-sided risk arrangements, including the Shared Savings Program, to treat patients with rare disorders without absorbing the incremental cost of this care that would otherwise apply under a shared-risk model. This carve-out/exception would be triggered when either:

- A patient presents with a diagnosis for a rare disease that is not associated with a disease-specific cost benchmark for shared-risk purposes that is based on an accepted standard of care for that disease; or
- The clinician identifies a patient with a set of symptoms requiring further follow-up through specialist referral and diagnostic testing and facilitates appointment(s) for those services.

Additionally, the complex patient bonus should account for complexity associated with rare diseases and additional complexity associated with social determinants of health. Haystack

appreciates that CMS recognizes the need to ensure clinicians treating Medicare's sickest and most vulnerable patients are not penalized. We continue to support the complex patient bonus under the MIPS and urge CMS to implement a presumption that patients with diagnosed rare disorders as well as those with significant symptoms requiring a definitive diagnosis are "complex."

The GAO report mentioned above also found implicit biases in rare disease patient care, where pre-existing judgments related to race, socioeconomic, or gender beliefs can interfere with a clinician's ability to accurately diagnose a disease or refer a patient for specialist follow-up.

With regard to the QPP, we remain concerned that the focus on established indicia of "quality" for relatively common conditions represents a lost opportunity for meaningful improvement in rare disease care. Haystack recognizes that the QPP was designed to maximize its impact on the value of care for beneficiaries and, therefore, was built around common conditions. The structure and criteria for implementing quality measures make it difficult, if not infeasible, to create measures reflecting care for each rare disease, or even for related subsets of rare and extremely rare conditions as the measures would fail to meet both the benchmark and case requirement thresholds. Moreover, clinicians would likely not elect to report on measures that do not apply to the majority of their patients unless the potential "point" value is comparatively high. We believe, however, that the QPP could increase attention on rare diseases and reduce the disparities in care quality and access experienced by individuals of color and other under-served patients.

We urge CMS to

- develop measures and improvement activities that reflect rare diseases in MIPS
- enable clinicians to earn MIPS "bonus points" for diagnosing and/or appropriately managing and treating patients with ultra-rare disorders
- include measures applicable to rare diseases in its Chronic Care Management MVP
- increase clinician awareness of potential for a rare disease diagnosis in primary care by developing one or more measures incentivizing efficient rare disease diagnoses within the Promoting Wellness MVP and
- develop an outlier-styled mechanism to account for rare disorder diagnosis and treatment costs under Advanced Alternative Payment Models

We also suggest that CMS develop alternative means to reward clinicians treating patients with rare disorders, including practice improvement and advancing care information measures specific to rare disorders. Haystack is eager to work with CMS on measures reflecting the main components of quality care for people with rare and extremely rare disorders, including clinician activities that promote:

- Recognition of patients at risk for the disease
- Starting the appropriate evaluation
- Making the appropriate diagnosis and/or referring the patient to a specialist making the appropriate diagnosis

- Starting the appropriate treatment
- Appropriate follow-up to ascertain treatment adherence/compliance and response.

3. Advancing Health Equity

CMS wants to further advance health equity across our programs by identifying and promoting policies, programs, and practices that may help eliminate health disparities. We want to better understand individual and community-level burdens, health-related social needs (such as food insecurity and inadequate or unstable housing), and recommended strategies to address health inequities, including opportunities to address social determinants of health and burdens impairing access to comprehensive quality care.

Examples may include, but are not limited to:

- Identifying CMS policies that can be used to advance health equity:
    - Recommendations for CMS focus areas to address health disparities and advance health equity, particularly policy and program requirements that may impose challenges to the individuals CMS serves and those who assist with delivering healthcare services;
    - Recommendations on how CMS can better promote and support accommodations, including those from providers and health plans, for people with disabilities and/or language needs or preferences;
    - Input on how CMS might encourage mitigating potential bias in technologies or clinical tools that rely on algorithms, and how to determine that the necessary steps have been taken to mitigate bias. For example, input on how we might mitigate potential bias with clinical tools that have included race and ethnicity, sex/gender, or other relevant factors. Further, input on potential policies to prevent and/or mitigate potential bias in technology, treatments or clinical tools that rely on clinical algorithms.
    - Input on how CMS coverage and payment policies impact providers, suppliers, and patients, especially in the treatment of chronic conditions and the delivery of substance use disorder and mental healthcare, including individuals who are dually eligible for Medicare and Medicaid; and
    - Feedback on enrollment and eligibility processes, including experiences with enrollment and opportunities to communicate with eligible but unenrolled populations.

Response

Individuals with rare and ultra-rare diseases are particularly vulnerable to changes in how care is received as well as provider reimbursement; SDOH present an additional layer of vulnerability. Haystack Project remains concerned that changes to incentive frameworks, particularly drug pricing initiatives, will have a disproportionate impact on individuals with very rare diseases due to high cost of on-label treatments, and that this impact will be felt first and hardest on individuals without the financial and community resources to navigate challenges. In addition, our member organizations have significant concerns that any government action that serves to limit prices for new drugs will substantially curtail interest in developing

therapies for extremely rare conditions.

In addition, the increasing prevalence of "value-based" care models presents significant challenges for individuals with rare diseases. Incentives align toward reducing costs associated with common health conditions, and tend to discourage use of the resources required to diagnose and treat individuals with extremely rare diseases. Haystack Project has advocated for specific carve-outs applicable to rare disease patients as well as incentives to reward timely diagnosis, treatment planning, and care coordination. Moreover, while we suspect that the unduly lengthy journey from emergence of symptoms to diagnosis is even longer for patients in communities of color and other underserved populations, our member organizations do not have the resources to quantify those inequities or identify clear causative factors.

One of the significant gaps that our member organizations have identified is uncertainty in accessing treatments.

- Individuals relying on Medicare Part D often find that the off-label treatments used within the standard-of-care are not included in the set of compendia that define what is and is not a "Part D covered drug." The rarer the disease, the less likely it is that medically accepted treatments will be published in compendia. Patient access programs are not generally available since a manufacturer offering free or discounted drug in this patient population would face off-label promotion scrutiny and potential liability.
- Patients face this problem within the Medicaid program as well. To the extent that an individual has access to a provider willing to invest the time and resources in appealing denials, their treatment may be delayed but not completely denied. The hurdles to receiving treatment, however, are significant. Navigating these barriers requires a well-informed patient/caregiver and a tenacious clinician.
- Receiving care in the home is often the best option for low-income and rural patients and their families. SDOH can, however impede availability of this option due to lack of broadband internet capabilities and financial impediments to maintaining reliable housing and utilities.
- Although CMS enabled expanded access to in-home administration of Part B drugs through its COVID-19 telemedicine flexibilities, physician practices have not made use of this pathway for ensuring patients continue to receive their treatments. Patients have faced a great deal of uncertainty, including use of home infusion suppliers for administration of treatments. These entities have been hesitant to work with physician practices to enable coverage of treatments under Part B, leaving patients with financial uncertainties associated with Part D coverage.

Understanding the effects on underserved and underrepresented populations when community providers leave the community or are removed from participation with CMS programs.

Response:

Patients with rare diseases face elevated challenges in finding a provider capable of treating their condition. This is already very difficult due to the small number of experts in the field who are located at major academic medical centers across the country which tend to be located in large, metropolitan cities. Even when patients live near by, challenges still exist in accessing experts including issues with local travel and reliance on public transportation, inability to take time off

work, and competing priorities like caregiving for other family members. Travel to these centers and insurance coverage varies, leaving many patients without adequate or proper diagnosis or treatment plans. And this is certainly exacerbated during a PHE with social distancing concerns and overwhelmed facilities. Even if a specialist is available, it is likely that they will be outside the patient's network. Receiving inadequate treatment and/or improper diagnosis can have a long-term impact on an individuals' disease management and progression.

Last year, the Government Accountability Office (GAO) complied a report to Congress entitled "RARE DISEASES: Although Limited, Available Evidence Suggests Medical and Other Costs Can Be Substantial" in collaboration with EveryLife Foundation for Rare Diseases and the National Organization for Rare Disorders. The report assessed the challenges rare disease patients face accessing diagnostic and treatment services as well as the personal and economic costs associated with treatment delays. Among its many findings, the GAO identified that rare disease patients are unable to access specialists due to geography or failure to receive a referral for follow-up care at initial symptoms, and often progress to more severe disease states by the time they receive an accurate diagnosis. Forty-one percent of rare disease patients also receive at least one misdiagnosis, and many are treated for a condition they did not have, and approximately 7 percent of rare disease patients reported that they were given a false psychological/psychiatric diagnosis that further impeded and delayed their treatment. Making access to appropriate experts through telemedicine could make a meaningful difference in addressing some of these findings.

- Recommendations for how CMS can promote efficiency and advance health equity through our policies and programs.

Response:

Haystack Project believes that technology can be leveraged to reduce the diagnostic journey for rare and ultra-rare disease patients as well as to ensure that all patients have access to the expertise needed to effectively treat or manage their condition. This includes:

- Increased access to and use of telehealth within the patient's home. For rare disease patients subtle changes in disease symptoms and/or progression could have profound impacts on longer-term outcomes. Use of wearables, monitors, and access to layperson friendly medical equipment would enhance remote monitoring capabilities and provide key patient information that may not be ascertained from periodic in-person visits;

- Providing patients with mobile devices and bandwidth (5G or 4G access) that are capable of delivering high-quality video resolution so that remote visits are as helpful to both clinician and patients as they can be.

- Ensuring that many patients and their families have sufficient education, training, and support to identify and utilize technologies that could improve day-to-day care burden and health outcomes.

Haystack Project expects that the value to patients in terms of improved outcomes and fewer acute events would outweigh the costs of wearable devices, improved video within remote visits, and other resources that can improve information available to patients and clinicians. However, CMS should take care to ensure these technologies are only available for patients with the

resources to afford them. Unless there is a level playing field on access to emerging technologies, a family's financial resources will enable or limit the impact that technology can have on improving patient outcomes.

Haystack has long called for a uniform mechanism for providers to qualify for accepting Medicaid payments from other state programs. Patients with conditions best addressed by sub-specialists should be able to obtain treatment without incurring additional costs. If providers deem appropriate, Medicaid programs would have to allow telemedicine options for specialist care management, reducing patient disruption and likely reducing costs after initial face-to-face visits enabling a treatment plan.

State and federal efforts to implement waivers for both telehealth and treatment received outside of state boundaries have proven very helpful during the PHE. While there has been a lot of attention on the need to extend or make permanent the gains made in accessing care through telehealth for rural communities, we want to emphasize the critical need for access to clinicians with very specific expertise when patients face conditions so rare that there are only a few specialists in the country. We strongly urge these changes be made permanent as these challenges will persist for our community. They are a critical lifeline between our patients and the extremely rare disease experts that are few and far between for each condition.

4. Impact of the COVID-19 Public Health Emergency (PHE) Waivers and Flexibilities

CMS wants to understand the impact of waivers and flexibilities issued during the COVID-19 PHE, such as eligibility and enrollment flexibilities, to identify what was helpful as well as any areas for improvement, including opportunities to further decrease burden and address any health disparities that may have been exacerbated by the PHE.

Examples may include, but are not limited to:

- Impact of COVID-19 PHE waivers and flexibilities and preparation for future health emergencies (e.g., unintended consequences, disparities) on providers, suppliers, patients, and other stakeholders.

Response:

Haystack supports continued access to telemedicine as an option that patients can choose in consultation with their clinicians. For rare and ultra-rare disease patients, telehealth services have proven to be a valuable adjunct to in-person visits throughout the COVID-19 pandemic. Audio-only and audiovisual visits initiated from the patient's home have enabled broader access to a continuity of coordinated care that includes disease-specific expertise from local specialists as well as those outside the patient's geographic area without the burden of travel. For many patients and their families, telemedicine has offered increased convenience and incrementally decreased the burden families face in caring for an individual with a serious rare condition.

In the early months of the pandemic, several Haystack member organizations reached out to patients with a survey assessing the patient experience with telehealth services during the Public Health Emergency (PHE). Most patients responding to the survey were able to access telehealth with relative ease and felt that the telehealth service flexibilities were useful in avoiding COVID-

19 exposure. For individuals with rare conditions, the increased ease in accessing specialist care underscores the need to continue many of these flexibilities permanently. For example, one patient noted the care they have received through telehealth during the PHE:

"Medication changes, local tests were ordered, met with neurosurgeon to determine surgery is needed. We live in Alaska and frequently have to fly to Seattle for care. We have been able to visit with specialists via telehealth and it's saved us considerable money and provided us with additional opportunities to see experts regarding care."

Patients responding to Haystack's survey appeared to view telehealth as vital throughout the PHE, but also recognized its value as an adjunct to in-person care thereafter. The PHE has given patients increased flexibility to see their healthcare providers from home or other convenient location, reducing the costs of transportation, missed work or school, child-care, and other expenses associated with in-person appointments. Increased access to telemedicine has also given patients access to disease specialists they may have previously been unable to see due to distance. It is not uncommon for rare disease patients to find that there are just a handful of disease-specific specialists in the entire country. Individuals in rural, low-income, and other underserved areas will lose meaningful access to these experts unless the telehealth flexibilities remain in place.

Last year, the Government Accountability Office (GAO) complied a report to Congress entitled "RARE DISEASES: Although Limited, Available Evidence Suggests Medical and Other Costs Can Be Substantial" in collaboration with EveryLife Foundation for Rare Diseases and the National Organization for Rare Disorders. The report assessed the challenges rare disease patients face accessing diagnostic and treatment services as well as the personal and economic costs associated with treatment delays. Among its many findings, the GAO identified that rare disease patients are unable to access specialists due to geography or failure to receive a referral for follow-up care at initial symptoms, and often progress to more severe disease states by the time they receive an accurate diagnosis. Forty-one percent of rare disease patients also receive at least one misdiagnosis, and many are treated for a condition they did not have, and approximately 7 percent of rare disease patients reported that they were given a false psychological/psychiatric diagnosis that further impeded and delayed their treatment. Making access to appropriate experts through telemedicine could make a meaningful difference in addressing some of these findings.

Haystack has also heard from patients and caregivers regarding the barriers individuals with hearing and/or visual impairments face in seeking care. Usher Syndrome, for example, is a very rare (approximately 25,000 US patients) inherited disease that causes combined hearing loss and vision loss from retinitis pigmentosa. For these patients, it is essential that remote care includes access to an ASL interpreter if they have sufficient remaining vision, or a tactile sign interpreter if they do not. We urge CMS to ensure that providers are sufficiently reimbursed for telemedicine services that are accessible for hearing and vision impaired patients.

Lack of reliable broadband wireless technologies and/or devices with data and video capabilities have also made it difficult for low-income families and those in rural areas to take advantage of the expanded availability of telemedicine other than through flexibilities permitting telephone-only visits. Returning to the pre-pandemic requirements on initiation site and audiovisual

capabilities will disproportionately constrict care that the most vulnerable patients have relied upon.

- Recommendations for CMS policy and program focus areas to address health disparities, including requested waivers/flexibilities to make permanent; any unintended consequences of CMS actions during the PHE; and opportunities for CMS to reduce any health disparities that may have been exacerbated by the PHE.

Response:

Haystack members have expressed gratitude for the flexibilities implemented during the PHE that allowed coverage of injected or infused medications administered in the home. However, the surprise out-of-pocket (OOP) costs for patients as their care has unexpectedly shifted from Medicare Part B to D has been problematic. Part D OOP costs can be significantly higher, and in fact, Congress had been considering a bipartisan proposal to cap OOP costs in Part D prior to the PHE. Our efforts to address high OOP under Part D have been delayed during the PHE, but seniors face increasing economic hardships during this pandemic and need help now. We would strongly encourage any efforts to reduce or mitigate high Part D OOP costs during this and future PHEs. This could be done by:

- requiring coverage of clinician-administered medications under Part B even when the technicalities associated with the entity doing the administration or the site of care would shift it to Part D;
- looking at how Medigap policies could support their patients, given that they contracted to cover their Part B OOP costs and are finding that obligation lifted unexpectedly during this the PHE in patients forced to rely on Part D for their Part B drugs due to in-home administration;

Haystack supports pragmatic and immediate solutions that offer seniors and disabled beneficiaries relief during the PHE or until Congress enacts a cap.