December 10, 2021

The Honorable Cheri Bustos
1233 Longworth House Office Building
Washington, DC 20515

The Honorable Tom Cole
2207 Rayburn House Office Building
Washington, DC 20515

The Honorable G.K. Butterfield
2080 Rayburn House Office Building
Washington, DC 20515

The Honorable Markwayne Mullin
2421 Rayburn House Office Building
Washington, DC 20515

Dear Ms. Bustos, Mr. Cole, Mr. Butterfield and Mr. Mullin,

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 unique to rare diseases or particularly pronounced in extremely rare diseases, including rare cancers. 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 all Americans living with or caring for someone with a rare or ultra-rare condition. We strive to amplify the patient and caregiver voice in disease states where unmet need is high, and treatment delays and inadequacies can be catastrophic. Haystack Project is thankful for the opportunity to submit these responses to the questions outlined in the Request for Information from the Congressional Social Determinants of Health Caucus.

What specific SDOH challenges have you seen to have the most impact on health? What areas have changed most during the COVID-19 pandemic?

Haystack Project is a non-profit organization enabling rare and ultra-rare disease patient advocacy organizations to highlight and address systemic obstacles to patient access that are unique to, or particularly pronounced in, extremely rare diseases. In early- to mid-2020, the COVID-19 pandemic initiated social distancing requirements that presented SDOH challenges in:

- Access to affordable and safe transportation options to access health care;
- For pediatric rare and ultra-rare disease patients, and adult patients with significant caregiver requirements, access to viable daycare and respite care options for families;
- Availability of in-home infusion of needed medications;
- Diagnostic delays already exacerbated by differential access to technology, specialist care, diagnostic services, and preventive care screenings were further impacted by social distancing as well as the impact the pandemic has had on employment;
- Challenges in initiating or maintaining participation in clinical trials;
- Constricted access to patient, caregiver, and family support services due to social distancing; and
- Financial challenges leading to increased insecurity in food, housing, and affordable insurance.

As the pandemic extended, patients and their families have struggled with social isolation. Families living with immune response vulnerability pre-pandemic are accustomed to and have "expertise" in maintaining tight control of social contacts, but still faced significant stress, uncertainty, and even poorer outcomes due to "clinical" distancing that resulted in delayed access to basic clinical care and specialty services. Although remote visits permitted a level of care continuity, and should remain available beyond the pandemic, the uniqueness and subtleties associated with many rare diseases can make loss of observation or care within an in-person visit essential. A patient with a rare, progressive disease could find that even a slight downward trajectory could result in symptom exacerbation or functional losses that cannot be returned to their former baseline. Availability of reliable broadband wireless technologies have also made it difficult for low-income families and those in rural areas to take advantage of the expanded availability of telemedicine.

In addition, patients experiencing acute crises associated with their rare or ultra-rare conditions faced uncertainties on availability of hospital services as well as concerns that a visit to the ER or inpatient admission presented a significant risk of COVID-19 exposure. When patients required care in an inpatient setting, families experienced increased stress because hospital policies often curtailed or prohibited family members from staying with or visiting their child or other loved ones.

Haystack Project did, however, hear from several patient groups that the COVID-19 flexibilities enabled access to out-of-state specialists through telemedicine. Patients with extremely rare diseases often find that there are just a handful of disease-specific specialists in the entire country. Individuals in rural, low-income, and other underserved areas do not have meaningful access to these experts unless the telehealth flexibilities remain in place. In addition, Medicaid patient access to out-of-state experts has been a longstanding barrier to timely diagnosis and appropriate care for individuals with rare diseases. The COVID-19 pandemic introduced a

streamlined approach to out-of-state Medicaid provider eligibility that should be a permanent pathway for Medicaid providers treating individuals with rare and ultra-rare conditions.

In addition to the interplay between the pandemic and social determinants of health on access to quality care for rare disease patients, COVID-19 cast a spotlight on the profound impact that race and racial inequities have on health outcomes. Our member organizations represent a diverse set of rare and ultra-rare disorders, some of which have known disparate impacts on communities of color. Unfortunately, unless registry participation, outreach, and engagement is sufficiently representative of the total patient population, advocacy organizations remain uninformed of disparate disease burdens, treatment response, and access to care, and cannot advocate on behalf of ***all*** patients impacted by a rare condition. Haystack believes that patient advocacy organizations can play a strong role in narrowing care gaps due to social determinants of health and systemic perpetuation of racial inequities. Our member advocacy organizations have asked for support in illuminating and addressing the needs of non-white patients in their communities.

What types of gaps in care, programs, and services serve as a main barrier in addressing SDOH in the communities you serve? What approaches have your organization, community, Tribal organization, or state taken to address such challenges?

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.

Haystack Project conducted several webinars to inform patient advocacy organizations on the telemedicine flexibilities and how their patients might use those refinements to continue their treatments. We have also reached out to our member organizations to assess the impact COVID-19 had on access to care.

In addition, Haystack supports the rare and ultra-rare advocacy community in efforts to capture the patient voice in "value" determinations through its Patient Oriented Value Reports, and is initiating a project to guide member organizations in assessing the representativeness of their patient community and improving their outreach and education initiatives. These initiatives will empower advocacy organizations with the information and insights they need to ensure that their policy priorities align with patients of all races and ethnicities, regardless of socioeconomic status.

Are there other federal policies that present challenges to addressing SDOH?

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 fall 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.

Is there a unique role technology can play to alleviate specific challenges (e.g. referrals to community resources, telehealth consultations with community resource partners, etc.)? What are the barriers to using technology in this way?

As the pandemic emerged, rare disease patients and their families faced significant challenges in accessing care. This meant that patients and caregivers were, of necessity, taking on more demanding and active roles as the hands and eyes of clinicians. The pandemic has demonstrated that, with guidance, tools and support, families can take on proactive and impactful roles and responsibilities that optimize patient care.

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. 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.

Are there any non-traditional partners that are critical to addressing SDOH that should be better aligned with the health sector to address SDOH across the continuum from birth through adulthood? What differences should be considered between non-health partners for adults' social needs vs children's social needs?

Patient groups can play a critical role in ensuring that their membership reach is representative of the relevant disease population, and that all voices are included in patient registry efforts, natural history studies, and clinical trial recruitment. Since patients with very rare diseases are unlikely to have multiple FDA-approved treatment options, there should be greater flexibility

for patient organizations to receive funds from industry and utilize those funds to provide underserved patient communities with what they need to overcome logistic challenges to care, including within clinical trials.

Patient advocacy organizations should have a seat at the care, research, therapeutic, and treatment coverage table. Although the data and contact information, registry data, and collected experience of patients are of vital importance in treatment development, patient groups can and should play a larger role as n early and primary point of contact, information, education, and support for families as well as local medical and caregiver teams. Patient organizations, particularly those within the rare and ultra-rare disease community, provide extensive support to guide the entire family of affected patients and are a key sounding board and resource as families consider the many day-to-day decisions required to optimize quality of life with or without therapy.

What opportunities exist to better collect, understand, leverage, and report SDOH data to link individuals to services to address their health and social needs and to empower communities to improve outcomes?

See above.

What are some programs/emergency flexibilities your organization leveraged to better address SDOH during the pandemic (i.e., emergency funding, emergency waivers, etc.)? Of the changes made, which would you like to see continued post-COVID?

Please see the above comments on telehealth flexibilities and COVID-19 challenges.

Which innovative state, local, and/or private sector programs or practices addressing SDOH should Congress look into further that could potentially be leveraged more widely across other settings? Are there particular models or pilots that seek to address SDOH that could be successful in other areas, particularly rural, tribal or underserved communities?

Several states have implemented programs for children with complex care needs. For the most part, these programs utilize a coordinated care model to facilitate timely diagnosis, access to disease experts, including out-of-state providers, support for caregivers, informational resources, and transportation to providers. These types of programs can improve care for all individuals with rare and ultra-rare diseases and their families. In addition, individuals in underserved communities face particularly long diagnostic journeys.

Symptoms that start in early childhood may not be recognized, addressed, or followed-up to achieve diagnosis until the patient reaches adolescence or early adulthood. For these individuals, disease progression can be substantial, and families with limited financial resources are unlikely to be equipped to absorb the costs of medical care and caregiver responsibilities.

Given the evidence base about the importance of the early years in influencing lifelong health trajectories, what are the most promising opportunities for addressing SDOH and promoting equity for children and families? What could Congress do to accelerate progress in addressing SDOH for the pediatric population?

See above.

Alternative payment models help to measure health care based on its outcomes, rather than its services. What opportunities exist to expand SDOH interventions in outcome-based alternative payment models and bundled payment models?

Haystack Project and its member organizations have significant concerns that APMs and other "value-based" care models can have unintended consequences for individuals with rare and ultra-rare diseases. Incentives in these programs 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 APM carve-outs applicable to rare disease patients as well as incentives to reward timely diagnosis, treatment planning, and care coordination.

A critical element of transformation, particularly for new models of care, is measurement and evaluation. With SDOH in mind, which are the most critical elements to measure in a model, and what differences should be considered when measuring SDOH outcomes for adults vs children?

Haystack Project remains concerned that establishing a fixed set of measurement and evaluation parameters will almost certainly yield unintended consequences for individuals with rare and ultra-rare diseases. We strongly believe that evaluation should be driven by outcomes that are most important to patients with the specific disease state.


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