



HAYSTACK PROJECT

The Voices of Rare & Ultra Rare

YEAR IN
REVIEW **2020**

FROM THE CEO



Welcome to the 2020 Haystack Project Year in Review. To say this year has been tumultuous could be considered an understatement. The COVID-19 pandemic touched all of us in one way or another. With heartfelt sorrow, Haystack recognizes that some felt the effects of the pandemic acutely with the loss of loved ones. For the rare and ultra-rare disease communities, the pandemic brought critical medical and healthcare challenges and often the loss of essential support systems.

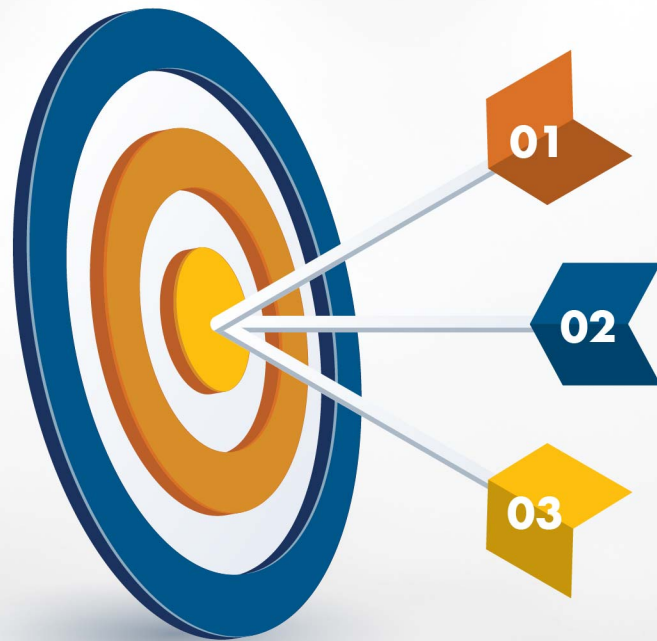
Even in this craziest of years, Haystack has worked hard to represent the issues raised by our patient groups and industry partners. And we made tremendous progress! In July and October, several of our policy priorities were introduced as legislation – identified by our participating patient groups as vital to their and our community. We also responded to the COVID-19 pandemic on several fronts.

As we leave a turbulent 2020 behind and look ahead to a promising New Year, we hope that this Year in Review brings you much encouragement, since we could not have accomplished all this without you.

James Caro
CEO and Chairman of the Board

HAYSTACK PROJECT'S 2020 GOALS

Haystack Project started the year with clear guidance from the participating patient groups. In 2020, our focus was to:



01 Raise Haystack Project's profile and collaborations

02 Initiate Policy Solutions

03 Formalize Industry Partnerships

BY THE NUMBERS

70+
Rare / Ultra-Rare
Groups

7 Policy Initiatives =
2 bills in Congress;
491 Letters to Congress
from **39** states in **2** months

10
Industry
Partners

53
1:1 Listening Sessions
with each of our participating
groups

100%
Dedication
to our participants

CONGRESSIONAL Roundtables

We were initially invited to present our policy priorities and solutions to the staff of the Congressional Rare Caucus in the House. A successful and robust Roundtable led to a second even more widely attended Roundtable in the Senate. Senators Bob Casey (D-PA) and Tim Scott (R-SC) invited staff from every Senate Finance and HELP Committee office to a wide-ranging discussion of Haystack priorities in early 2020.

Both events kicked off a series of meetings by patients and patient groups that led to two pivotal bill introductions in spite of the sometimes insurmountable distractions of COVID-19!



RARE DISEASE Week



RARE ACCESS FORUM, FEBRUARY 2020

As advocates came together during Rare Disease Week, Haystack Project hosted an interactive Panel Discussion on Access and Value in Rare. Melanoma Research Foundation led the discussion for rare cancers and the American Porphyria Foundation represented ultra-rare conditions.

The panel included Congressional staff as well as representatives from academia and diagnostics.

Topics ranged from the study of small populations, access to early diagnosis, and the ability of value frameworks to capture the patient perspective.

RAISING OUR Profile

As the pandemic took hold, our community confronted the impossible choice of risking COVID-19 exposure or the potentially catastrophic consequences of delaying or stopping treatment. Haystack asked others to join us in urging HHS to **immediately let patients receive Medicare-covered Part B infused medications from qualified practitioners in the safety of their homes**. We were fortunate to have a very early heads-up from our community, allowing us to engage CMS and HHS in a timely way.



March 27, 2020

The Honorable Alex Azar
Secretary
Health and Human Services
200 Independence Avenue, SW
Washington, DC 20201

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

Secretary Azar and Administrator Verma,

The undersigned patient advocacy organizations are committed to ensuring that individuals with serious chronic conditions, including rare and ultra-rare conditions and rare cancers, have access to the health care services that are critical in treating and/or managing their diseases. We appreciate CMS' efforts to ensure that the nation's most vulnerable patients receive the care they need throughout the COVID-19 pandemic. We write to call your attention to a serious care gap that has emerged from the COVID crisis that, without your urgent attention and action, will place our patients at significant risk.

Secretary Azar's leadership in writing to the nation's governors on March 24, urging an "all hands on deck" approach was a critical step toward ensuring that our health care system quickly adapts to meet the challenges of this national emergency. The letter highlighted the importance of relaxing settings of care to permit broader access to care in patients' homes, and this should be of great help to those covered by Medicaid and commercial insurers. Unfortunately, Medicare payment rules and mechanisms do not align with the flexibilities Secretary Azar urges among states and providers. Medicare beneficiaries are at greatest risk for severe and fatal COVID-19 illness, yet without CMS' immediate action they will remain unable to receive their care within the safe social distancing required to minimize COVID transmission.

Throughout the pandemic, patients with rare and serious chronic conditions who rely on Medicare Part B settings of care for their infusion treatments are confronted with an impossible choice between the high risk of COVID-19 exposure within a provider setting and the known, potentially catastrophic consequences of delaying or stopping the treatments they need to avoid disease progression or exacerbation. We desperately need your help, and urge you to issue the necessary program transmittal(s) or other policy directives required to align Medicare



identified. CMS must permit Medicare infused medications from qualified Part B benefit. We expect that this could result in increased payments between physician offices (and/or other settings) and qualified health care providers in the home setting. The attached draft outlines the mechanisms patients and providers need to ensure access.

For patients with serious and life-threatening conditions, the risk of illness and that of their caregivers, but it is also overwhelming by gravely ill COVID-19 patients throughout this public health emergency, with the greatest vulnerability to COVID-19.

On this critical issue. Please contact Saira Choudhury at scho@nhc.org or reproject.org with any questions or to help us to impact specific patient communities. We appreciate your attention.



INITIATE Policy Priorities

2020

We began by listening to each of our patient groups. All 70+ participating groups were asked to collect – from their patients, caregivers, medical advisors, industry partners, board members and anyone else they care to ask – what barriers to access pose the biggest hurdles for them.

We then worked on parsing out how often the same issues cropped up, in what settings of care, among which payers. We looked for opportunities to write to CMS and others but also to create tangible, meaningful solutions from which we could start conversations with policymakers.

The ideation stage, and the extensive research, analysis, and education that followed, gave Haystack the credibility to socialize barriers and solutions, with examples and data where possible.

RESULTS FOLLOWED

Even in a daunting year with many distractions, H.R. 7567 (the HEART Act) and H.R. 8467 (Access to Rare Indications Act) were both introduced!

Haystack's hard work, thoughtful problem solving, and credible approach accelerated our ability to make a mark and propel us to a level of success it takes years to achieve!



THE HEART ACT

H.R. 7567

WHAT IS IT?

A new bill introduced in Congress calls for important changes in the FDA rare disease drug review process.

The HEART Act would require rare disease experts, including patients living with the condition, to be involved throughout the FDA's process for determining if a new drug is safe and effective for ultra-rare conditions.

ADVISORY COMMITTEES

Require a rare/ultra-rare expert in the science of small population studies at Advisory Committee meetings when the application under review is for a low prevalence condition;

DIVISION EXPERIENCE TRANSPARENCY

Require a rare/ultra-rare expert in the science of small population studies at Advisory Committee meetings when the application under review is for a low prevalence condition;

REVIEW DIVISION SUPPORT

Require Rare Disease Program staff in review team when reviewing a first drug/biologic or first disease modifying agent for a particular indication associated with orphan condition with very low prevalence;

RISK EVALUATION AND MITIGATION STRATEGIES

For any very low prevalence orphan applications, require FDA to consult with patients in devising/reviewing any Risk Evaluation and Mitigation Strategies elements that require patient action/participation;

EU EXPERIENCE

Require study of how the European system reviews ultra-rare applications and its applicability in the US.



THE HEART ACT

Helping Experts Accelerate Rare Treatments

What is it?

A new bill introduced in Congress calls for important changes in the FDA rare disease drug review process.

The bill, called **The HEART Act (Helping Experts Accelerate Rare Treatments)**, will require rare disease experts to be involved throughout the review process, including FDA staff, patients and experts in the study of small populations.

For more information about the bill, visit: <https://livingwithfcs.org/the-heart-act/>.

For additional ways to support The HEART Act, email contact@livingwithfcs.org or info@haystackproject.org.



How can you help?

Congress needs to hear from you in support of The HEART Act. Make your voice heard!

- Find your Congressperson. Enter your zip code at <https://www.house.gov/representatives/find-your-representative> to locate your Congressman and get their contact information.
- Send them an email and ask them to support **The Heart Act (Bill# H.R. 7567)**.
- Your message should include your name and the city where you live. Share a personal message about your family's experience with a rare disease and end with a request that your Congressperson sponsor H.R. 7567.
- Below is a draft email that you can customize to reflect your family's experience and your personal feelings about the need to support the Rare Disease Community.

My name is (insert name) and I live in (city/state). I am reaching out to urge you to co-sponsor a new bill, H.R. 7567, also known as The HEART Act. This bill requires that experts who really understand rare diseases, like patients and physicians, are involved in the FDA's drug review process. By co-sponsoring this bill, you will be helping to make sure that the drug review process in the U.S. reflects the unique needs of all rare disease communities. Thank you.



ACCESS TO RARE INDICATIONS ACT

H.R. 8467

The Access to Rare Indications Act of 2020 ensures indications for rare and ultra-rare treatments are covered to the full extent of FDA's label and extends a precedent that recognizes the limitations of studying very small populations. It also takes a doctor's experience into account in allowing patients to get those new treatments.

The Access to Rare Indications Act of 2020 would:

- » Require payers to cover FDA indications per the FDA approved label rather than limit coverage to inclusion/exclusion data.
- » Expand the statutory definition of "medically accepted use" to include compendia-listed uses, peer-reviewed literature, clinical guidelines, or expert opinion within the National Institutes of Health Rare Diseases Clinical Research Network consortia or its participating clinical centers.



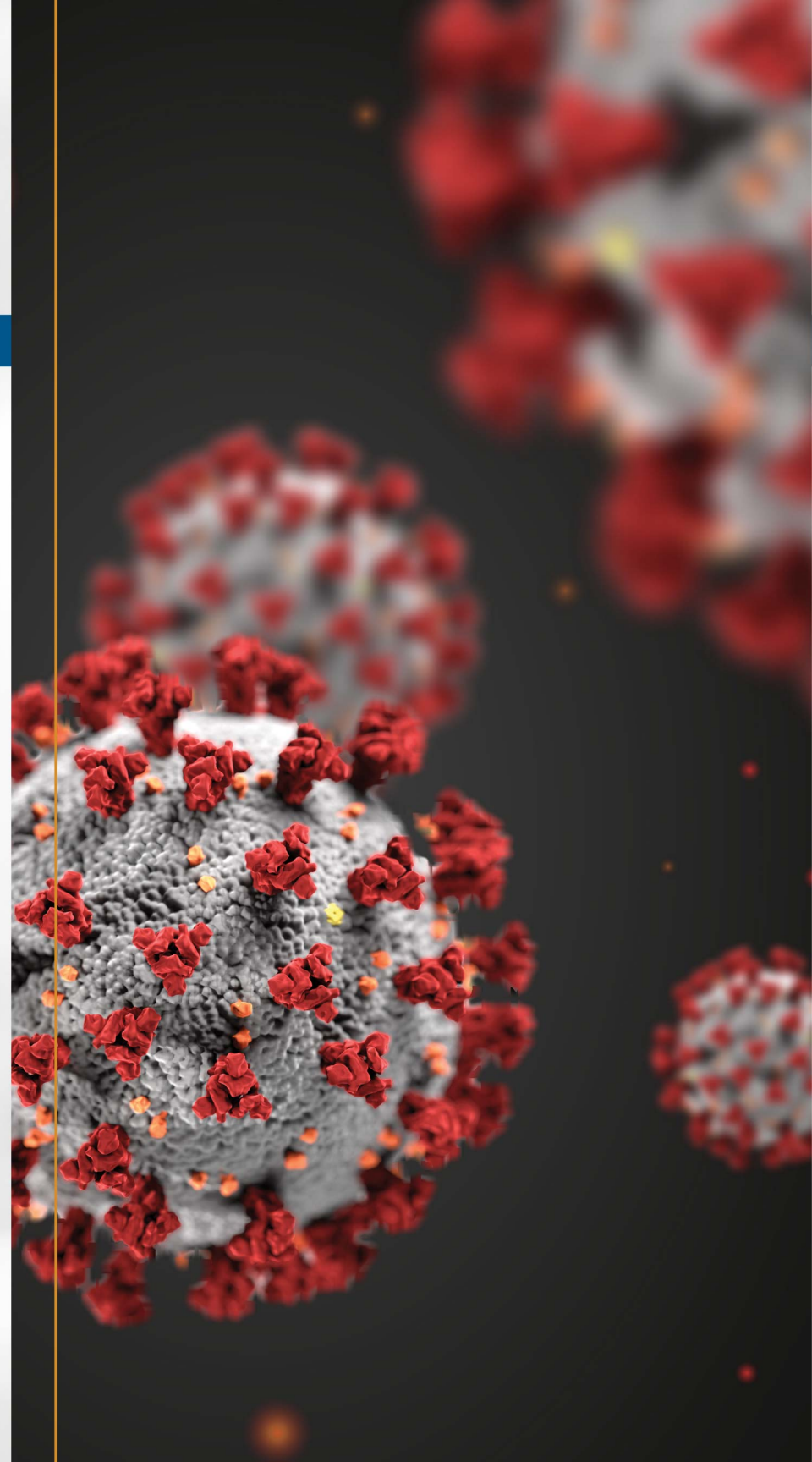
COVID-19 Efforts

MEDICARE HOME INFUSIONS ADVOCACY

Haystack Project played a critical role in advocating for patient needs related to the COVID pandemic. Haystack not only drafted a letter highlighting the problem of immune compromised patients that other umbrella groups signed onto, we led a dialogue with CMS to find the right solution for home infusion.

The fact that we were early in identifying the issues gave Haystack Project exceptional access to the right COVID-response team members at CMS.

There is, unfortunately, more work to be done on this front, and we hope to continue the discussions into 2021.



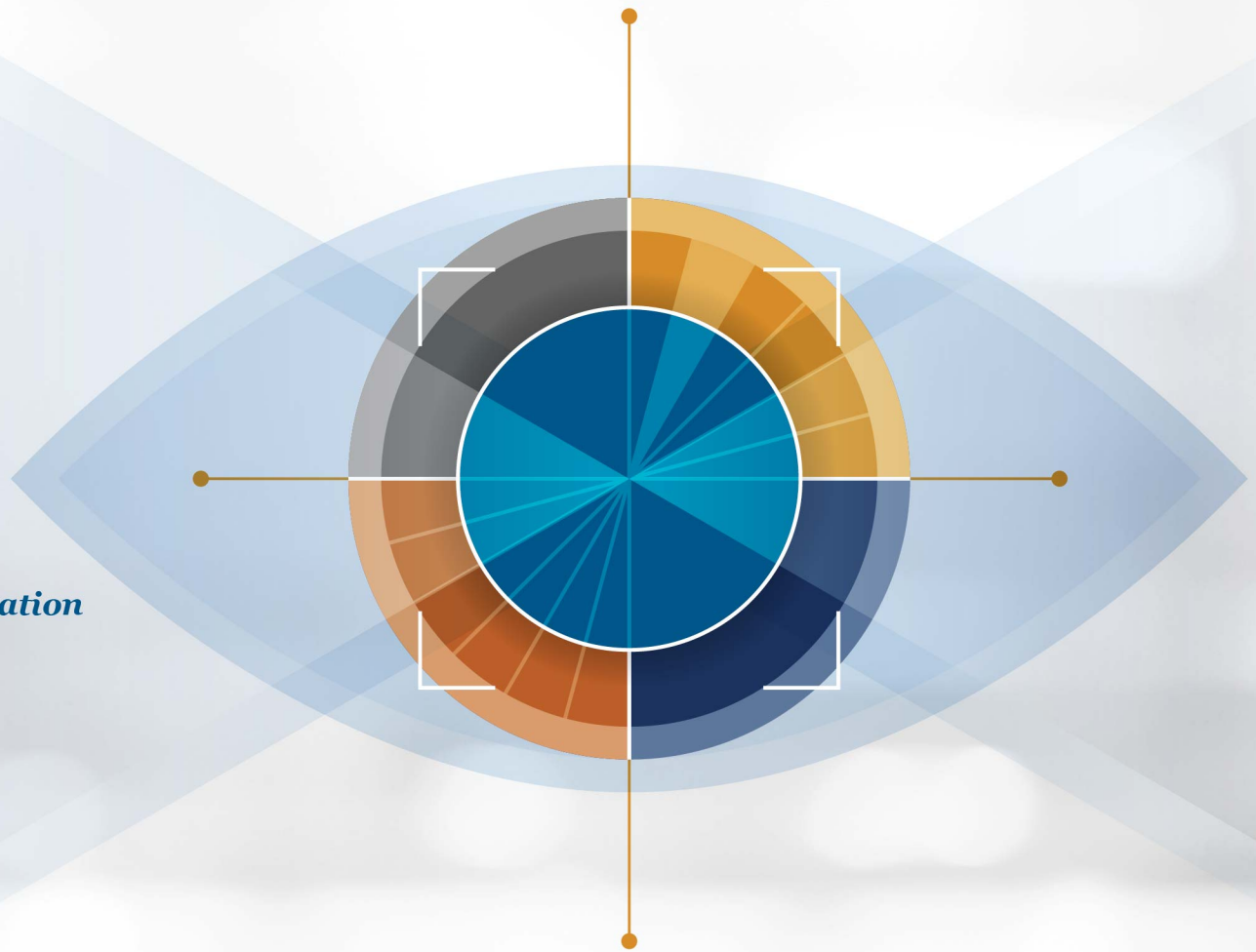


What we've done so far:

- » Educational webinars
- » Comment letters
- » ICER Listening Session
- » Rare Disease Week panel discussion

In 2020, we continued our conversation with ICER. We have:

- » Joined Fair Access initiative
- » Applied to be a Voting Panel member
- » Requested access to ICER Analytics™



PATIENT ORIENTED VALUE Report (POV)[®]



POV

Patient Oriented Value

We are nearing completion of our first Patient Oriented Value (POV)[®] Report in Ocular Melanoma. Ocular

Melanoma is a very rare cancer that is cured through surgery or radiation therapy in about 50% of patients, while the remaining 50% go on to develop metastatic disease and have very poor prognosis. The “wait and see” period can be very stressful for patients, and those developing metastases have few treatment options.

The POV[®] Report assesses the patient perspective throughout the patient journey, including disease burden for patients and caregivers, access to testing for risk of metastasis, preferences on primary treatment, potential adjuvant approaches to address high-risk disease, and treatment for metastatic disease. We believe that this POV[®] Report will enable a better understanding of Ocular Melanoma as it is experienced by patients and their caregivers, and highlight unmet needs and the value of addressing them.

We are kicking off our second POV[®] Report in Choroideremia, a rare inherited disorder that causes progressive vision loss and ultimately leads to complete blindness. ICER’s review of Luxturna[®] was completed in advance of product approval, so we expect ICER is aware that a Choroideremia treatment has reached Phase III. It is critically important that Haystack and the Choroideremia Research Foundation work on articulating the patient perception on value for ICER, the patient and provider community and for payers.



INDUSTRY Participation

Established a Corporate Council and a Value and Access Council



CORPORATE COUNCIL

Haystack Project's Corporate Council will critically affect the lives of rare and ultra-rare patients and their caregivers.

As the only organization focused solely on reimbursement, value, and patient access for the rare and ultra-rare community, our educational efforts continue to grow in size and impact.

The Corporate Council increases the overall capacity of Haystack Project to fulfill its mission. Haystack Project accepts financial support from corporations to increase the education and awareness of systemic barriers to appropriate reimbursement and assessment of value in rare and especially ultra-rare conditions. Haystack Project employs corporate resources to develop, produce, and implement mission related programs, materials, and activities.



VALUE & ACCESS COUNCIL

The Value and Access Council (VAC) brings critical specialized expertise and insight to the sole mission of Haystack Project. Corporate Council members will be integral in identifying internal experts to serve on the VAC.

Patient groups are particularly interested in VAC members who have distinct and deep experience in:

- » reimbursement
- » payer marketing
- » outcomes research
- » market access
- » health economics
- » commercialization

Haystack Project believes these disciplines are strongly aligned to the mission and vision of our organization. Only with really deep, multi-faceted stakeholder experience in these disciplines will we be able to develop and coalesce around priorities that are tangible, credible, and thoughtful for our patients. Join us and make a difference in the lives of rare and ultra-rare patients and their caregivers.

GET Involved

As we complete our patient group listening sessions, we are building out new initiatives for 2021, so keep an eye out for details on HEAT, AEI, and Rope Bridge, which will join our existing RCPC and POV initiatives.



Haystack Project has many opportunities to support our ongoing work. Request a sponsorship brochure today and join the growing ranks of the partners we are so grateful and honored to work with.



Mitsubishi Tanabe Pharma
America

