



HAYSTACK PROJECT

The Voices of Rare & Ultra Rare

2021
YEAR IN
REVIEW

FROM THE CEO



Welcome to the 2021 Haystack Project Year in Review!

Although we discovered “Zoom fatigue,” we also found that digital communication could benefit health care for those with rare and ultra-rare conditions. In 2021, Haystack fought to maintain telehealth flexibilities put in place during the COVID public health emergency. We also made hard-won progress on the HEART Act and Access to Rare Indications Act with virtual Hill visits, navigating Committee input and Agency assistance. Based on member input, we also developed new educational / outreach initiatives.

Haystack cannot do any of this work without our Member Groups and Industry and Alliance Partners. Thank you for a productive 2021!

Thank you for the opportunity to serve as Haystack’s CEO. I have enjoyed seeing the organization grow to new heights and am honored to have played a part. I will still be a part of this great group as a Board member. I’d like to welcome Deanna Darlington to the CEO role and look forward to seeing the amazing places she will take Haystack Project and its members.



Deanna Darlington
Incoming CEO and Chairman of the Board

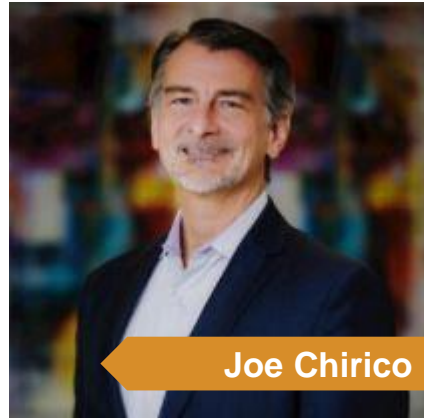

Jim Caro

OUR BOARD

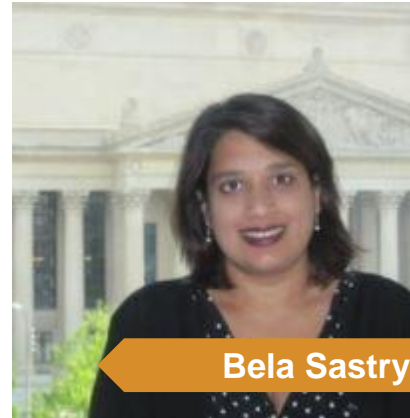
Has been growing



James Caro



Joe Chirico



Bela Sastry



Christina Nyquist



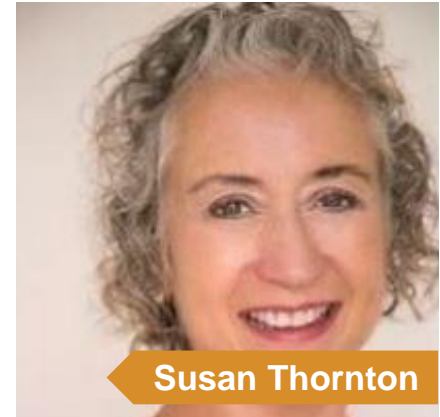
Marc Yale



Chevese Turner



Lisa Steelman



Susan Thornton

Kay Scanlan
Senior Policy Consultant

Saira Sultan
Senior Policy Consultant

Cynthia Rothblum-Oviatt, PhD
Science Advisor / Member Engagement

Cara Tenenbaum
Policy Consultant

Andreas Barth
IT Support

Lydia Szanyi Boudreaux
Graphic Design

Rebecca Balliet
Website Support

Misty Osburn
Administrative Assistant

HAYSTACK PROJECT'S

2021 Goals

As usual, Haystack Project started the year with clear guidance from our member patient groups. In 2021, our focus was to:

Raise Haystack Project's profile and collaborations

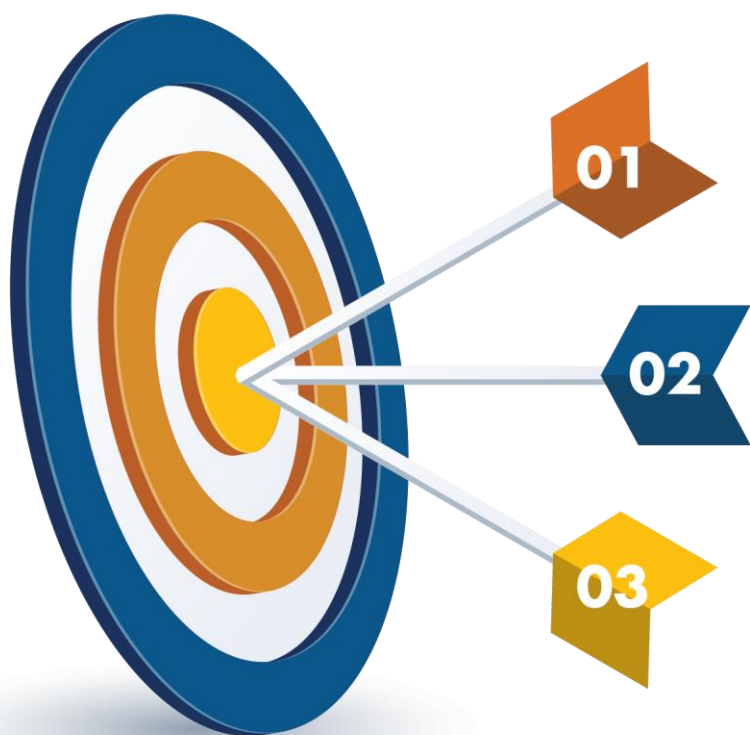
- » Improve profile, including social media
- » Increase Industry partnership and establish Alliance Partnerships

Continue to drive Policy Solutions

- » Re-introduction of HEART and Access to Rare Indications Act
- » Support Telehealth Flexibilities post COVID
- » Advance policies on Telehealth, Out of Network Care and Value Assessments

Increase Educational Programming

- » Advocacy Empowerment Institute (AEI)
- » Health Equity in Access to Treatment (HEAT)
- » Rope Bridge Collaborative
- » ICER



BY THE NUMBERS

90+

Rare / Ultra-Rare
Groups

2 bills in Congress
4+ policy priorities

1500+ letters to
Congress

13

Industry
Partners

7

Alliance
Partners

100%

DEDICATION
to our participants

01

APRIL 2021

MACPAC April Public Meeting

Five Haystack groups - APF, TSA, IPPF, CDG Care and IFOPA - spoke at MACPAC's April Public Meeting to highlight the disproportionately harmful impact on ultra-rare innovation of a policy to impose additional rebates on FDA Accelerated Approval drugs.

Haystack educated groups about the policy, submitted comments to MACPAC, and worked with these groups on their talking points.

MACPAC's recommendations penalize companies before they have become "bad actors" and disincentivize the use of this FDA pathway to speed therapies to those who likely have *no other treatment options*.



Kara Berasi, Board Member with CDG Care and Mom of a child with a CDG or Congenital Disorder of Glycosylation



CONGRESSIONAL FOCUS: *carveout for rare and/or for initial treatment; and/or post-FDA deadlines*

haystack@haystackproject.org

01

SUPPORTING GROUPS

In All Ways



Rep. Tonko Virtual Rare Roundtable with Melissa Goetz, Co-Founder, FCS Foundation in support of HEART Act. May 2021 --Albany, NY

Melissa Goetz meeting with Rep. Tonko, November, 2021 -- Washington, DC



01

HAYSTACK PROJECT'S Alliance Partners

In 2021 we extended our Alliance Partnerships and welcomed several new members.

We regularly connect with these partners about their policy work and ours, supporting each other's work where we can, and lending insights and perspective.



The opportunity to suggest topics for webinars and other initiatives at Haystack



Regular 1:1 calls with Haystack Project to align and discuss opportunities to partner



Recognition on our website



AMERICAN
BRAIN
COALITION



BLACK WOMEN'S
HEALTH IMPERATIVE



Global Genes®
Allies in Rare Disease



INNOVATION AND
VALUE INITIATIVE

All Copays Count Coalition



01

HAYSTACK PROJECT'S Traditional & Social Media

We have been more active with our social media, but have a ways to go ... Be sure to follow, like, share, & post what you see!



TWITTER
twitter.com/HaystackProject



FACEBOOK
facebook.com/HaystackProject



LINKEDIN
linkedin.com/company/haystack-project



OPPORTUNITY: Partnering on traditional media.

Senate Introduction in December '21



Ad Coms

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



Division Experience Transparency

Annual report setting out, by review division, number of rare applications accepted or rejected for filing, the number pending, approved, and disapproved, and the size of the affected population;



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;



Benefit-Risk Assessment

For rare disease applications, require FDA to consult with patients, patient group, and at least one expert identified by the patient group, throughout the benefit-risk assessment and post approval safety monitoring;

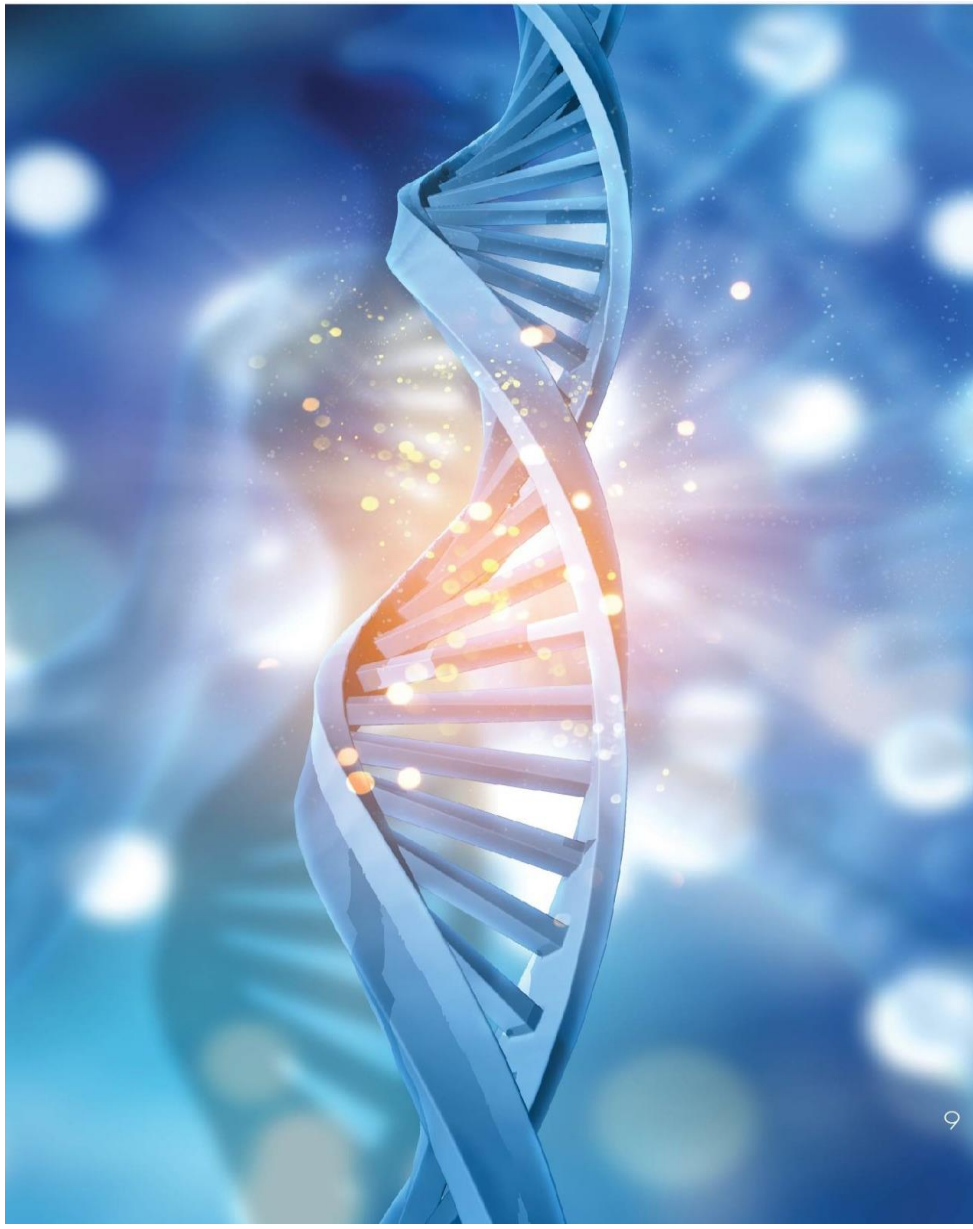


EU Experience

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

ACCESS TO RARE INDICATIONS ACT

Re-introduction Dec '21



Access to Rare Indications Act of 2021

- Expands the definition of “medically necessary” care in Medicare Part D and Medicaid to include peer-reviewed journal articles and expert opinion (in addition to on label and compendia).
- Provides for an expedited appeal and reconsideration process for private insurers for rare patients

This is not a coverage mandate, and applies only to treatments for rare conditions

*Bipartisan W&M and E&C Members to Introduce
Early discussions with Committee staff positive*

VALUE FRAMEWORKS LEGISLATION

Hill Discussions Underway

Prohibits CMMI from applying model tests to rare conditions *UNTIL*:



02 TELEHEALTH FLEXIBILITIES For Rare Patients

Support Permanency of PHE Telehealth Flexibilities for Rare Patients

Looking beyond the COVID PHE



Rare disease is the “poster child” for maintenance of telehealth flexibilities post COVID. Rare needed telehealth prior to COVID and will need it once the PHE is past.

The ongoing examination of making those flexibilities permanent continues. We’d love to convey how those flexibilities have worked for rare patients and how they didn’t.



Haystack will develop a questionnaire about telehealth use during COVID for distribution to your patients and then generate a white paper to share with legislators.



HAYSTACK PROJECT

Comment Letters

MACPAC
Accelerated
Approval Rebate
proposal

MARCH 2021

Cures 2.0

JULY 2021

CMS MCIT and
Reasonable & Necessary
comments (2)

OCTOBER 2021

JANUARY 2021

Health Care
Innovation Caucus'
Modernization of
Stark and Antikick for
VBE *and*
Most Favored Nation
(MFN) Comments *and*
BERA+VBE+Request
for Input

APRIL 2021

CMS MCIT and
Reasonable &
Necessary
comments
and
Support HEART Act

SEPTEMBER 2021

Joint Letter-Part D OOP
(Haystack signed-on)

NOVEMBER 2021

RFI for new
Congressional **Caucus**
for **Social Determinants**
of **Health**

HAYSTACK PROJECT Initiatives



Established
2019



Initiated Speaker Series
Feb 2021



Began collaborations
Apr 2021



Began seeking partners
May 2021



Rare Cancer Policy Coalition
Focused exclusively on patient access and value across the rare cancer community

Advocacy Empowerment Institute
Daunting topics made approachable for participant groups

Rope Bridge
Collaborative cross-communication and education with payer groups; Establish Payer Council

HEAT
Identifying racial inequities in access to providers and treatment to improve health outcomes



Patient Oriented Value
Inserting the patient voice in value frameworks


Uveal Melanoma 2020
Choroideremia 2021
Inquiries pending for 2022



*Haystack participants requested increased educational programming on a range of topics for 2021, and Haystack is responding with the **Advocacy Empowerment Institute (AEI)**.*

AEI is designed to cover a range of topics, recognizing that rare and ultra-rare organizations are often required to be experts in many things, from governance and fundraising to translating complex healthcare policies into patient-centric advocacy concepts. Seemingly daunting topics will be made both approachable and more readily integrated into the operations and mission of the participant groups.

AEI 2021 has hosted:

-  **Carly Paterson Khan, PhD, RN, MPH**, PCORI Program Officer
-  **Matt Salo**, National Association of Medicaid Directors
-  **John Coster**, Director, Division of Pharmacy, Center for Medicaid, CMS
-  **Anne L Schwartz, PhD**, Executive Director, MACPAC
-  **Christina McCauley**, Senior Policy Advisor, Rep. Matsui (D-CA)
-  **Sarah Shapiro**, Legislative Director, Rep Swalwell



The HEAT program aims to create a “best practices” guide for our patient advocacy organizations

Haystack Project works every day on solving for a health system not designed to fully consider the lived experience of rare and ultra-rare patients. As Covid-19 cast a spotlight on the profound impact that race and racial inequities have on health outcomes, Haystack’s participating advocacy organizations asked for support in illuminating and addressing the needs of non-white patients in their communities.

Haystack and the HEAT program will enable each of our 90+ advocacy organizations to leverage their learnings to proactively drive initiatives toward reducing racial inequities in access to treatment and health outcomes.



We are actively seeking companies / patient groups as partners!!

Connecting Rare & Payor Expectations

To connect our patients with payors, Haystack launched **ROPE BRIDGE**. We envision this initiative as a collaborative, bi-directional communication and education effort to enable both sides to better understand rationales for access barriers and their impact across rare and ultra-rare disease stakeholders.

Rope Bridge will take two approaches:

- » Regular exchange with **Payer Associations** on policy issues;
- » A **Payer Advisory** for continuous and deep insights to help strengthen our understanding of utilization management approaches and how to work with and support our providers to meet documentation and other requirements for medical necessity.

“In this doctrinal relationship, honest differences can be bridged, if by no other means than by the swaying rope bridge of patience and friendship.”

~ Hansard archive



HAYSTACK PROJECT Initiatives



PAYER ASSOCIATIONS

INVITATIONS



INTRODUCTIONS



HAYSTACK PROJECT

Payer Council Underway



Cheryl Avants

Formerly with both Arkansas Children's Hospital and Arkansas BlueCross and BlueShield

Cheryl Avants comes from Arkansas BlueCross and BlueShield, where she worked with large national account employers and implemented a pharmacist consulting program for care management efforts, initiated medical benefit UM and created care management education, including care gaps for high-cost rare diseases. Cheryl also expanded internal efforts around medication policy, pipeline strategies, and rebates. Of particular note, Cheryl represented the Plan in efforts to address specialty medication concerns within BlueCross BlueShield Association and Consortium and with manufacturers on coverage strategy and UM.

Before coming to BlueCross Arkansas, Cheryl managed and ran drug utilization review programs for 8 years, including as Director of a Retrospective Drug Utilization Review (RDUR) for Arkansas Medicaid for 5 years and Colorado Medicaid program for 2 years. She was also a clinical analyst in information technology at the University of Arkansas for Medical Sciences, overseeing pharmacy efforts to implement an integrated software system combining pharmacy and medical ordering and billing for a hospital.



Susan McClendon

30-Year Pharmacy Manager at Accredo Specialty Pharmacy

A pharmacist by training, Susan McClendon brings decades of experience and insights in specialty pharmacy. She worked on numerous rare disease medications in her career at Accredo Specialty Pharmacy, as well as other complex intravenous therapies requiring high touch clinical expertise and follow up. Many of the therapies under her purview had special clinical requirements patients and/or their physicians had to meet before the product would be dispensed.

In her role as pharmacy manager, Susan oversaw the process of obtaining product and clinical consultation with prescribers, patients and benefit providers, arming her with a close working relationship with prescribers and specialty clinics.

Susan also worked closely with private and government payers to answer questions regarding the clinical aspect of the patient's billing or prior authorization. This was also a critical role, since some of the rare and ultra-rare therapies are not well understood by payers and require clinician follow-up to ensure patient starts and uninterrupted coverage.

Susan holds a BS in pharmacy from the University of Arkansas School of pharmacy.



FIRST 2 MEMBERS

of a group of 6-8 who will consult regularly with us.

INTRODUCTIONS WELCOME.

03

HAYSTACK PROJECT POV

PATIENT ORIENTED VALUE (POV)[®] Reports provide insight into the patient journey, articulate disease burden from the patient perspective, reveal real-world care gaps and communication deficiencies, and better understand treatment priorities and perceived value from the patient perspective.



In partnership with the Melanoma Research Foundation and with support from **Amgen**, we completed a Patient Oriented Value[®] (POV) Report in Uveal/Ocular Melanoma (UM/ OM).



In partnership with the Choroideremia Research Foundation and with support from **Biogen**, we are nearing completion on a POV report in Choroideremia, a rare inherited disorder that causes progressive vision loss and ultimately leads to complete blindness.



Uveal Melanoma POV Report Findings


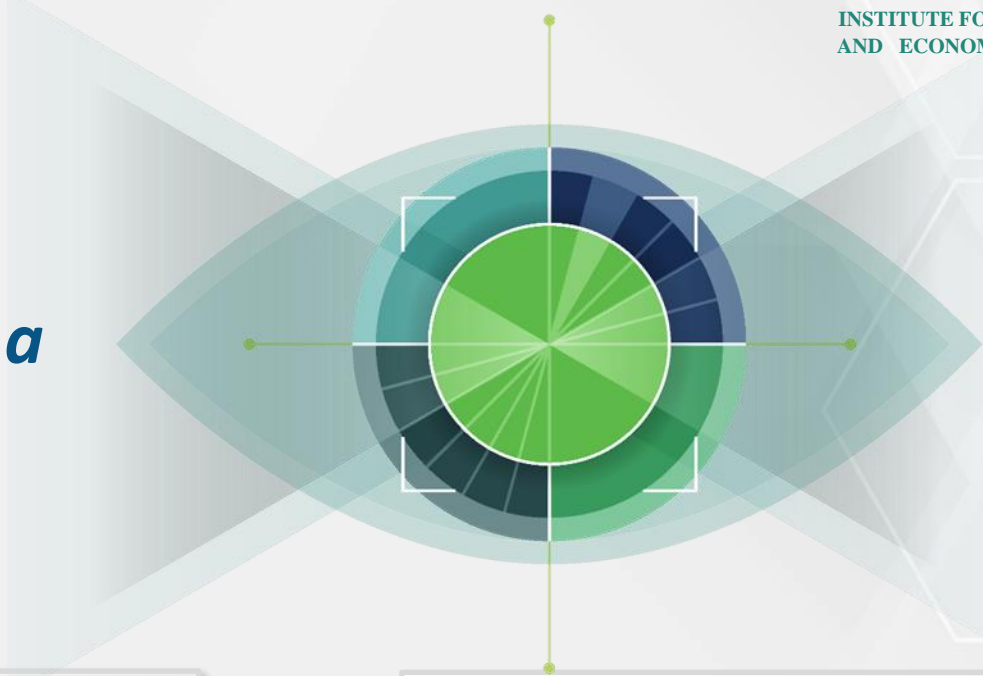
“Although there is no current consensus on a [SOC] for adjuvant treatment in high-risk patients, 14 of the 35 high-risk respondents (40%) indicated that they have received adjuvant treatment, either within or outside of a clinical study. This contrasts sharply with the reported experience of patients with “unknown” risk of metastatic disease. None of these patients have received adjuvant treatment or have enrolled in a clinical trial.”

»» “High-risk patients potentially benefiting from adjuvant treatment in the years, or even decades, after their primary treatment are closed out of these clinical trials and may find that they have limited (or no) access if a treatment is approved but its label aligns with the clinical trial population.”


»» “[C]overage for genetic or molecular prognostic testing may vary from payer to payer and require UM patients to go through multiple appeal processes to secure coverage. Medicare covers the DecisionDX-UM test but requires that billing providers utilize a registry and implies a level of oversight on subsequent care, including referrals and follow-up surveillance intensity, which could deter utilization.”

»» “[N]ewly diagnosed UM patients are not always informed that genetic testing for metastatic risk is available. In addition, a subset of respondents indicated that their metastatic risk was “unknown” despite reporting that genetic testing was performed. ...suggesting a gap in communication to be addressed by augmenting clinician-led discussions on diagnosis, treatment planning and follow-up”


Haystack is active in a number of ways:




Educational webinars




Access to ICER Analytistics™




Comment Letters




Fair Access Project




ICER Listening Session



Voting Panel Member for Rare Perspective



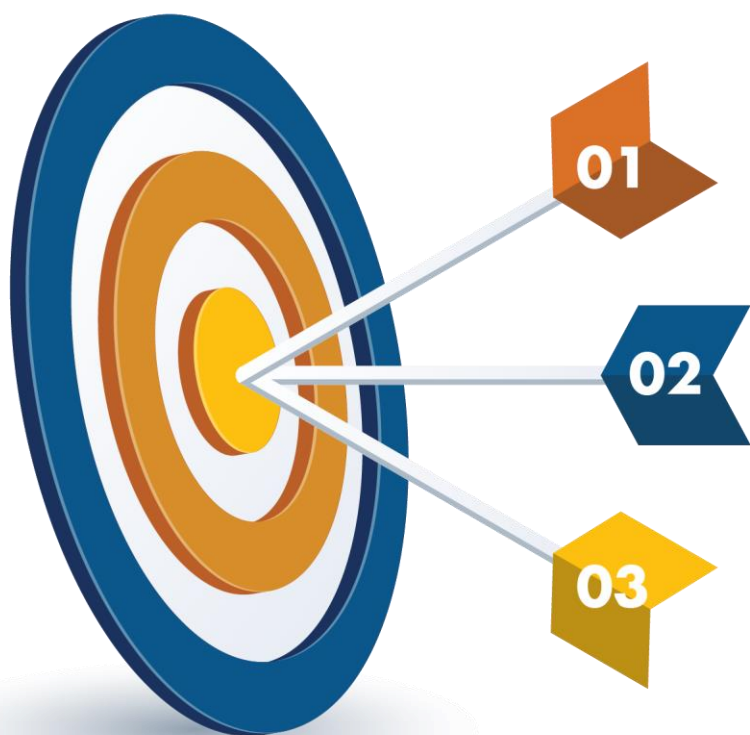
Rare Disease Week panel discussion



Coordinating groups for upcoming reviews

HAYSTACK PROJECT'S 2021 *Achievements*

As usual, Haystack Project started the year with clear guidance from our member patient groups. In 2021, our focus was to:



Raise Haystack Project's profile and collaborations

- » Improve profile, including social media
- » Increase Industry partnership and establish Alliance Partnerships

Continue to drive Policy Solutions

- » Re-introduction of HEART and Access to Rare Indications Act
- » Support Telehealth Flexibilities post COVID
- » Advance policies on Telehealth, Out of Network Care and Value Assessments

Increase Educational Programming

- » Advocacy Empowerment Institute (AEI)
- » Health Equity in Access to Treatment (HEAT)
- » Rope Bridge Collaborative
- » ICER

BY THE NUMBERS

90+

Rare / Ultra-Rare
Groups

2 bills in Congress

1500+ letters to
Congress

13

Industry
Partners

7

Alliance
Partners

100%

DEDICATION
to our participants

INDUSTRY Participation



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 access and appropriate 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
- Market Access
- Payer Marketing
- Health Economics
- Outcomes Research
- 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.

POTENTIAL PARTNERS

On Our Radar



HAYSTACK PROJECT

If You're Not Yet a Partner ...

