This was my first year as CEO, and I am honored to have played a part in Haystack Project’s ongoing success. During a challenging legislative and policy environment, we have made much progress, largely due to the wonderful patient organizations that participate with us. We continued our advocacy on the HEART Act and made progress on educating policymakers on why the Access to Rare Indications Act is so critical for the rare community.

I am pleased to see another idea from our members taking hold – the workgroups that bring multiple stakeholder perspectives together for deeper dives on specific issues – informing our work and making us stronger together. I’m looking forward to another year -- to grow our resources to match all the work our patient groups have come to count on us to provide and support their increasing trust in us to both learn and educate about the rare experience.
OUR BOARD

...and Staff Continue to Grow

Deanna Darlington  Jenn Wappaus  Bela Sastry  James Caro

Christina Nyquist  Marc Yale  Lisa Steelman  Susan Thornton

Kay S.
CMS Policy Director

Saira S.
Policy Director

Cara T.
Policy Director

Misty O.
Manager

Tiara L.
Administrative Asst

Kate G.
Researcher/Analyst

Rebecca B.
Website Support

Joe C.
Finance/Operations

Andrew B.
IT Support

Lydia B.
Graphic Design

haystack@haystackproject.org
HAYSTACK PROJECT’S 2022 Goals

Haystack Project starts each year with clear guidance from its patient groups. In 2022, our focus was a mix of new & existing goals:

1. **Multi-Stakeholder Collaborations**
   - Create multi-stakeholder forums for ‘deep dives’ into specific policy issues
   - Grow our Educational Speaker Series

2. **Continue to Drive Thoughtful, Well-Received Policy Solutions**
   - Promote enactment of HEART Act (HR 6888/S 4071)
   - Progress the Access to Rare Indications Act (HR 6160)
   - Support FDA use of Accelerated Approval Pathway in rare diseases, and coverage for AA-approved products
   - Educate and Refine Advocacy for ICD-10s
   - Execute Approps strategy on inadequacy of DRGs

3. **Raise Haystack Project’s Profile and Collaborations**
   - Increase Industry partnerships and Engage Alliance Partners
   - Rare Disease Week Hill Briefing
   - Trade Press: STAT News & Inside Health Policy
   - Virtual Finance Fly-In

**Dedication**
- 125+ Rare/Ultra-Rare Groups
- 4 Multi-Stakeholder Work Groups
- 14 Alliance Partners
- 100% DEDICATION
- 2 Bills
- 4+ Policy Priorities
- 50+ Listening Sessions
FIRST LOOK WORKGROUP
Determining which conditions can, at least initially, be referred out for further testing based on visual observation by a health care provider as signaling a rare condition.

MEDICAL NECESSITY
Developing policy solutions, including legislation, that help patients with rare diseases access off-label medications that are medically indicated.

HEART ACT AND AA PATHWAY
Ensuring that the FDA’s Accelerated Approval pathway takes into account the needs of patients with rare diseases, where large clinical trials are not feasible.

HP 50
Advancing access-related policy solutions in states and coordinating, empowering, supporting groups to approach state Medicaid agencies.

NEWLY FORMING...
Gauging interest for 2023 workgroups on:
- Rare Cancer Access
- IRA Implementation
Education: A Two-Way Street in Rare!

Seemingly daunting topics made more approachable for our patient groups! And coming full circle, our speakers say they gathered as much as they shared! In many ways, this is **the hallmark of Haystack!**

Sarah Shapiro
Legislative Director for Representative Swalwell

Hemi Tewarson, J.D.
M.P.H., Executive Director, National Academy for State Health Policy (NASHP)

Sana Raoof, M.D., PhD
Radiation Oncologist, Memorial Sloan Kettering Cancer Center

-AND-

Anna Howard
Principal, Policy Development, Access to and Quality of Care, American Cancer Society Cancer Action Network

Joni Rutter, PhD
Director at NIH’s National Center for Advancing Translational Sciences (NCATS)

-AND-

Eric Sid, M.D., M.H.A.
Program Officer at NIH

Beth Roberts, J.D.
Health Partner with Hogan Lovells

Aimee Diaz Lyons
Attorney with Metz, Husband, and Daughton

-AND-

Brian Henderson
Director of State Government Affairs for Hart Health Strategies

Lewis Fermaglich
M.D., M.H.A., Medical Officer, Office of Orphan Products Development, FDA

Tim Mooney, Senior Counsel with the Bolder Advocacy Program at Alliance for Justice

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Bipartisan HELP and E&C Leaders, Sens. Casey & Scott and Reps. Tonko and McKinley never gave up on the Heart Act and rare patients during the long and tortuous UFA reauthorization process!

The Heart Act requires regular reporting on FDA’s use of external experts when reviewing rare disease treatments;

It requires the National Academies to study and share learnings from the European Union’s approach to safety and efficacy reviews of rare disease treatments;

It allows FDA to consult with patients and patient groups when evaluating rare disease treatments.

It encourages FDA to consult with communities of color or other historically underrepresented and vulnerable populations if a product relates to a rare disease or condition that disproportionately affects those communities.
HEART Act’s Journey Through Congress

HR 6888/S 4071
The convoluted, “stop-start,” politics-over-substance journey our HEART Act has taken through Congress and eventual passage frustrated our patient group but gave Haystack a chance to teach how a “bill becomes a law” in 2022 – not exactly like School House Rock!

1. Introduced in House
   FEBRUARY 18, 2021

2. Re-Introduced in House
   After much negotiations
   MARCH 1, 2022

3. Introduced in Senate
   APRIL 7, 2022

4. Included in Senate Use Fee Act
   Reported to Senate
   JULY 13, 2022

5. Included in House User Fee Act
   PASSED HOUSE JUNE 8, 2022

6. Different language in House and Senate

7. “Clean” User Fee Act
   Enacted as part of CR Resolution
   SEPTEMBER 30, 2022

8. New Health-Related Package
   ENACTED BY
   DECEMBER 16, 2022

9. User Fee Act Expires
   DECEMBER 16, 2022

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The Helping Experts Accelerate Rare Treatments (HEART) Act of 2022
S. 4071

The HEART Act of 2022

The HEART Act seeks to further strengthen the FDA review process for drugs that treat rare and ultra-rare diseases by increasing direct involvement of patients, doctors, and scientists with specialized expertise throughout the review process. The goal is to ensure that the agency is including the appropriate subject-matter experts in those regulatory decisions and enhancing transparency into how the FDA makes regulatory decisions for drugs or treatments under certain conditions. The bill includes four key provisions:

1. Requires the FDA to report annually on its activities related to orphan drugs.
2. Establishes a Science Plan to support the agency’s review of orphan drug applications.
3. Increases the FDA’s staffing and funding for the Office of Orphan Products Development.
4. Creates a new grant program to support research on orphan diseases.

The HEART Act was introduced by Senator Bob Casey and Representative hut whisk and was enacted into law on December 16, 2022.
H.R. 6160 “Medical Necessity” Legislation

Access to Rare Indications Act Recognizes Rare Patients Need What Congress Once Did for Cancer Patients …

This bill builds off a cancer precedent to expand the definition of “medically necessary” care for rare patients in Medicare Part D and Medicaid to include peer-reviewed journal articles and clinical guidelines, and provide expedited appeal and reconsideration for private insurers.

Bipartisan lead sponsors in the House represent both W&M and E&C membership.

Hill feedback is consistent - Haystack proposals are tangible, incremental, credible, and politically feasible.

This is not a coverage mandate, and applies only to treatments for rare conditions.
Orphan Indications Provision in IRA
Price Negotiation Exemption Hurts Ultra Rare Indications

House Committee deliberations leading to introduction of H.R. 6160 questioned patient groups’ ability to drive more on-label indications for rare diseases, a daunting and largely unrealized goal by many for decades.

The IRA orphan exemption for price negotiations took a giant leap in the wrong direction, incentivizing developers to choose one large orphan indication rather than multiple ultra-rare ones.

Haystack Project, as the voice of ultra-rare patients, knows all too well that the rarer the condition, the less likely an indication. We will continue to educate and hopefully evolve the IRA provision to meet our goals as well as those of Congress....

This IRA exemption makes passage of H.R.6160 more urgent than ever!!!
Haystack education highlighted:

- necessary FDA enhancements to use the AA pathway more often in rare disease, and do so more consistently and transparently;
- much-needed collaboration across review divisions, with cross-divisional support for rare reviewers;
- critical education to reduce efforts to diminish coverage of treatments approved under the AA pathway;
Haystack Strives To *Crack the Code...*
**ICD-10 Coding**

**WHAT REIMBURSEMENT AND RESEARCH EXPERTS ARE SAYING:**

“Amen to Joni on ICD-10 codes or 9 codes not being sufficient for research....An ICD-10 code for each rare disease will not assist in research or identifying patients. ...ICD codes are not reliable...we need to be able to use data where the definitions are the same and they’re used consistently.”

~ Paula Shireman, Executive Associate Dean, Texas A&M College of Medicine

“ICD-10 codes are used to process insurance claims, to guide an insurance company’s decision whether to cover/pay for a drug.... A doctor’s decision to prescribe ....is not the decision an insurance company relies on....”

~ Beth Roberts, Partner, Hogan Lovells

“All health care providers must code to the greatest degree of specificity available to them. Putting a more general code on a claim to get something covered for a patient when a very specific rare disease code exists could make a doctor liable under the False Claims Act.

“There is tension between diseases not yet well understood, getting a specific ICD-10 code, and the downstream impact on a patient later seeking care when a new treatment comes to market. The code we wanted for electronic health record tracking, research, finding patients, etc. may be helpful or not helpful when the insurance company relies on it to guide a coverage decision. From a coverage/payment perspective, it can be a concern.”

~ Beth Roberts, Partner, Hogan Lovells

“ICD-10 codes are clinical classification systems... intended at supporting reimbursement....These are not meant to be disease terminologies as they lack the details and granularity needed for all of the types of research needed in many rare diseases, such as for natural history studies. ICD-10 codes are not the only way that rare diseases may be documented..... For individual rare diseases, this speaks to the need for a patient registry.”

~ Joni Rutter, Acting NIH NCATS Director
Haystack Strives To Crack the Code...

ICD-10 Coding

**WHAT PATIENT ADVOCATES ARE SAYING:**

“An ICD-10 code for each rare disease will not assist in research or identifying patients.”

“I don’t think there was any intention behind all of this ICD 10 push, but it’s almost a disservice, unless there’s some education. [W]e’re all here getting smarter together, but there’s 10,000 other rare diseases. And there’s podcasts and social media and there’s a lot of loud voices that are saying one thing. And they’re not hearing the [Haystack Speaker Series]. There really is not an appreciation for coding. So it’s a disservice unless the people who are learning these things share it with others.”

~ Patient Group Leader

“My concern with ICD 10 codes have been expressed by others. But I want to [add] - ...the other 2-3,000 advocacy organizations out there need to hear this message. [Y]ou can’t use one thing [like ICD-10 codes] to do everything. You have to think about research, genealogy, the epidemiology of the disorder .... these are entirely different things than recruiting patients, than finding patients. And it’s entirely different from approval, and it’s entirely different from reimbursement. ICD 10 comes out of a reimbursement history, and if you use [them] for all of these things, it starts to pick up all of this baggage.”

~ Patient Group Leader

“I think I started out as: We need to get on the bandwagon, we need to do this. And it’s the sessions that you’ve had and the cautions and then the broader questions that are on the table have really opened my eyes to certainly not rush with this.”

~ Patient Group Leader
Rare Diseases — The Committee notes the limitations of bundled payments for rare and ultra-rare diseases and urges the HHS Inspector General to review payment systems and quality reporting methodologies across settings of care that use bundled payments for barriers to accessing treatments, diagnostics, clinicians, and specialists, as appropriate.

The Senate language is stronger – it clearly assigns the much needed report to the OIG as patients intended;

Rare Diseases — The Committee recognizes patients with rare and ultra-rare diseases experience significant challenges, and such challenges are likely to impact marginalized communities and communities of color disproportionately. The Committee requests a report within 180 days of the date of enactment of this Act on barriers to accessing treatments, diagnostics, clinicians, especially specialist, in both conditions affecting fewer than 20,000 patients, and in communities of color affected by diseases with fewer than 200,000 patients. The report should include an assessment of any legal, improper payment, and fraud implications of any denials of care for these patients, as well as recommendations for addressing any barriers to accessing treatments for such patients.

Haystack Led the Way!!
We also continue to evaluate and sign on to coalition letters on topics such as telehealth flexibilities and copay accumulators.
Our “Library” is Growing! Check out our Patient Oriented Value (POV)™ Report on Uveal Melanoma and our latest one on Choroideremia.

Do you want to partner on a POV?

Haystack continues to seek support and partnership for initiatives envisioned by our patient groups based on their needs. We hope to partner and (re)launch these in 2023.

ICD-10 codes are a perfect example of the bi-directional education coming out of our speaker series. Deep dives into rare disease implications of arcane concepts taught us that ICD-10s don’t help with research or with finding patients like we thought they did. Even our speakers were grateful to learn: They’re not really the panacea for research!
“Rare disease advocacy groups hope the new Congress will prioritize investment in the development of rare disease treatments by passing bills that “ensure patient input is counted throughout the development process.”

“{Some are] hopeful that bills like the Helping Experts Accelerate Rare Treatments (HEART) Act have the bipartisan support needed to [pass]....”

“... the HEART Act would require FDA to consult external experts and stakeholders while also providing reports on how FDA is handling applications for a drug to be designated for the treatment of a rare disease.”

“There’s just a lack of expertise in general in [the area of] rare disease, so we really advocate for the FDA making sure they’re bringing people with the most timely and relevant knowledge into the process,” Malakoff told Inside Health Policy.

“Anything that can be done to streamline the process at FDA will help give that confidence back to investors on rare disease and biotech, and [this bill is an] example of steps Congress can take to help make that happen, Malakoff added.”
We took advantage of all the *Rare in the Air™* that February brings. **HOSTING** a Congressional briefing with esteemed panelists ahead of Rare Disease Day....

**THEN** *STAT News* published our panelists’ op-ed on the Access to Rare Indications Act...

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**Haystack Project is hosting a Rare Disease Week Panel Discussion**

*Tuesday, Feb 22, 2022 from 3-4pm ET/12-1pm PT*

Come join us to learn more about leveling the playing field for rare disease patients seeking medically necessary care.

**Speakers:**

- Christina McCauley: healthcare policy, Congresswoman Matsui
- Marc Yale: past research & advocacy lead, IPPF
- Dr. Robert Carlson: CEP, NCCN
- Dr. Darcy Krueger: University of Cincinnati College of Medicine
- Dr. Emanuel Mavarkis: University of California Davis

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The term “medically necessary” is the yardstick by which insurance companies, including Medicare and Medicaid, decide if they will pay for a particular treatment. For the millions of Americans living with rare diseases, most of which do not have FDA-approved treatments, identifying a treatment as medically necessary can be a lifeline — or the end of the line.

**AND** patients hit the Hill to talk about the ‘medical necessity conundrum’ rare disease patients need to defeat...

*Haystack Project is grateful to NORD and EveryLife Foundation for hosting Rare Disease Day and Week in February*
Targeted Senate Finance Fly-In with patients from Delaware to South Dakota and Wyoming!

Patients came together to share their insurance woes, the nightmare of denials and appeals, and the impact delayed access has when conditions relentlessly deteriorate patients’ lives each day.

Cancer groups shared how helpful similar Congressional intervention has been for oncology, not just directly for patients but for the field.
Ad hoc and regular communication with these partners allows us to support their work and vice versa. Reducing duplication of effort allows Haystack to maximize efficiency and minimize wasted resources.

JOIN OUR NETWORK OF ALLIANCE PARTNERS SO YOU HAVE:

- 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

haystack@haystackproject.org
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!

**OPPORTUNITY:** Leveraging social media network with participants

**TWITTER**
[twitter.com/HaystackProject](https://twitter.com/HaystackProject)
Approximately 20 posts in 2022

**FACEBOOK**
[facebook.com/HaystackProject](https://facebook.com/HaystackProject)
More than 25 posts in 2022

**LINKEDIN**
[linkedin.com/company/haystack-project](https://linkedin.com/company/haystack-project)
Approximately 25 posts in 2022
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.

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

- Amicus Therapeutics
- bridgebio
- mirum
- AVROBIO
- agios
- BeiGene
- Takeda
- Incyte
- Genentech
- Boehringer Ingelheim
- Intercept
- ACADIA
- biocryst
- Homology Medicines, Inc.
- anavex
- Neurocrine Biosciences
- santhera
- Arrowhead Pharmaceuticals
- Spark Therapeutics
- Novartis
- PTC Therapeutics
- IOVANCE Biotherapeutics
HAYSTACK PROJECT
If You’re Not Yet a Partner ...

JOIN
Haystack if you haven’t...

SCHEDULE
A meeting to learn more...

SHARE
Our posts on social media...

COMMUNICATE
With your Congressional leaders ...
Ask us how!

TELL
Patient groups and industry about us (120+ groups strong)