HAYSTACK PROJECT
The Voices of Rare & Ultra Rare

2022 YEAR IN REVIEW

FROM THE CEO

Deanna Darlington
CEO and Chair of the Board

2022 HAYSTACK PROJECT
Welcome to the Year in Review!

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.

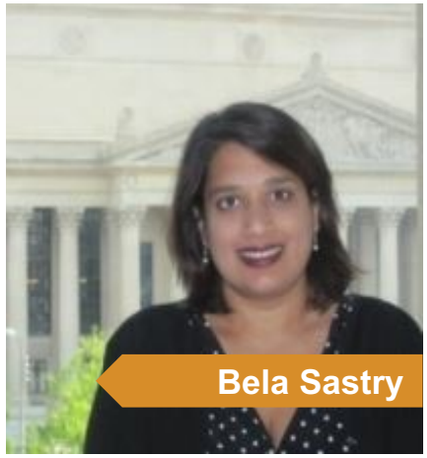
I am pleased to see another idea from our members taking hold – the workgroups that bring multiple stakeholder perspectives together for deeper dives on specific issues – informing our work and making us stronger together. I'm looking forward to another year -- to grow our resources to match all the 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.

RARE Is In the Air™ at Haystack Project

OUR BOARD
...and Staff *Continue to Grow*


Deanna Darlington


Jenn Wappaus

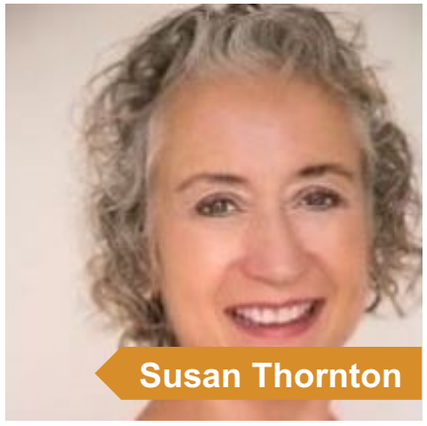

Bela Sastry


James Caro


Christina Nyquist


Marc Yale


Lisa Steelman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Cara T.
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Misty O.
Manager

Tiara L.
Administrative Asst

Kate G.
Researcher/Analyst

Rebecca B.
Website Support

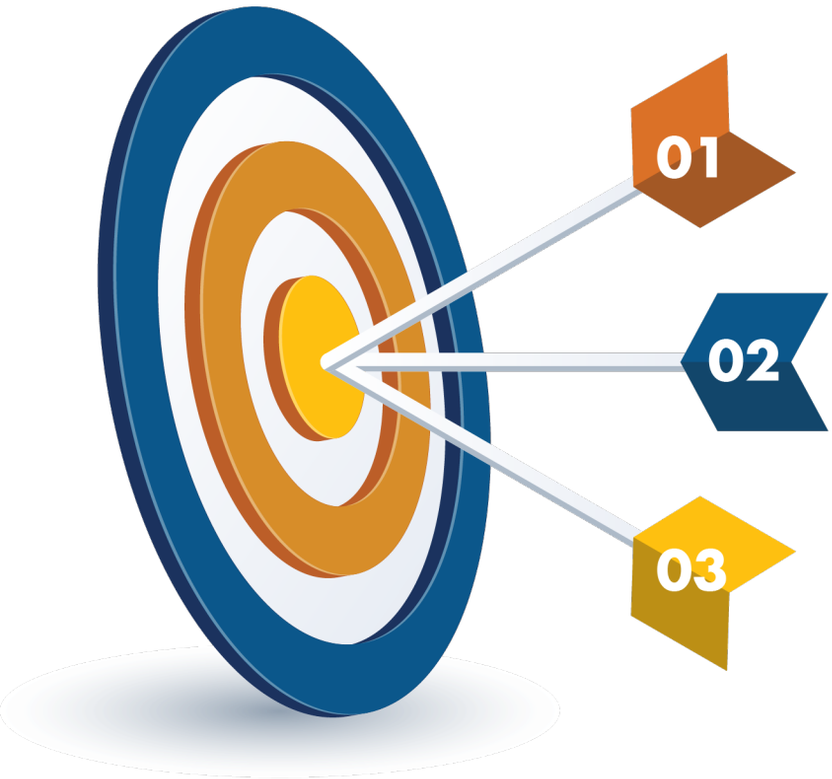
Joe C.
Finance/Operations

Andrew B.
IT Support

Lydia B.
Graphic Design

HAYSTACK 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:



Multi-Stakeholder Collaborations

- Create multi-stakeholder forums for 'deep dives' into specific policy issues
- Grow our Educational Speaker Series

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

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

125+ Rare/Ultra-Rare Groups

2 Bills

4 Multi-Stakeholder Work Groups

4+ Policy Priorities

14 Alliance Partners

50+ Listening Sessions

100% DEDICATION

01 Multi-Stakeholder Collaboration
Deep Dive Workgroups

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**

01

Education: A Two-Way Street in Rare!

AEI Advocacy Empowerment Institute

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)

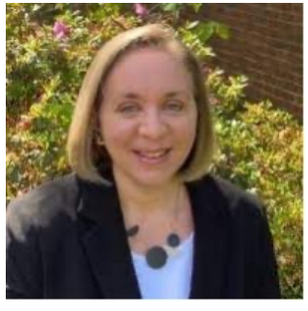
Joni Rutter, PhD
Director at NIH's National Center for Advancing Translational Sciences (NCATS)

-AND-

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

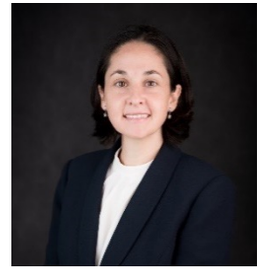
Sana Raoof, M.D., PhD
Radiation Oncologist, Memorial Sloan Kettering Cancer Center

-AND-



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

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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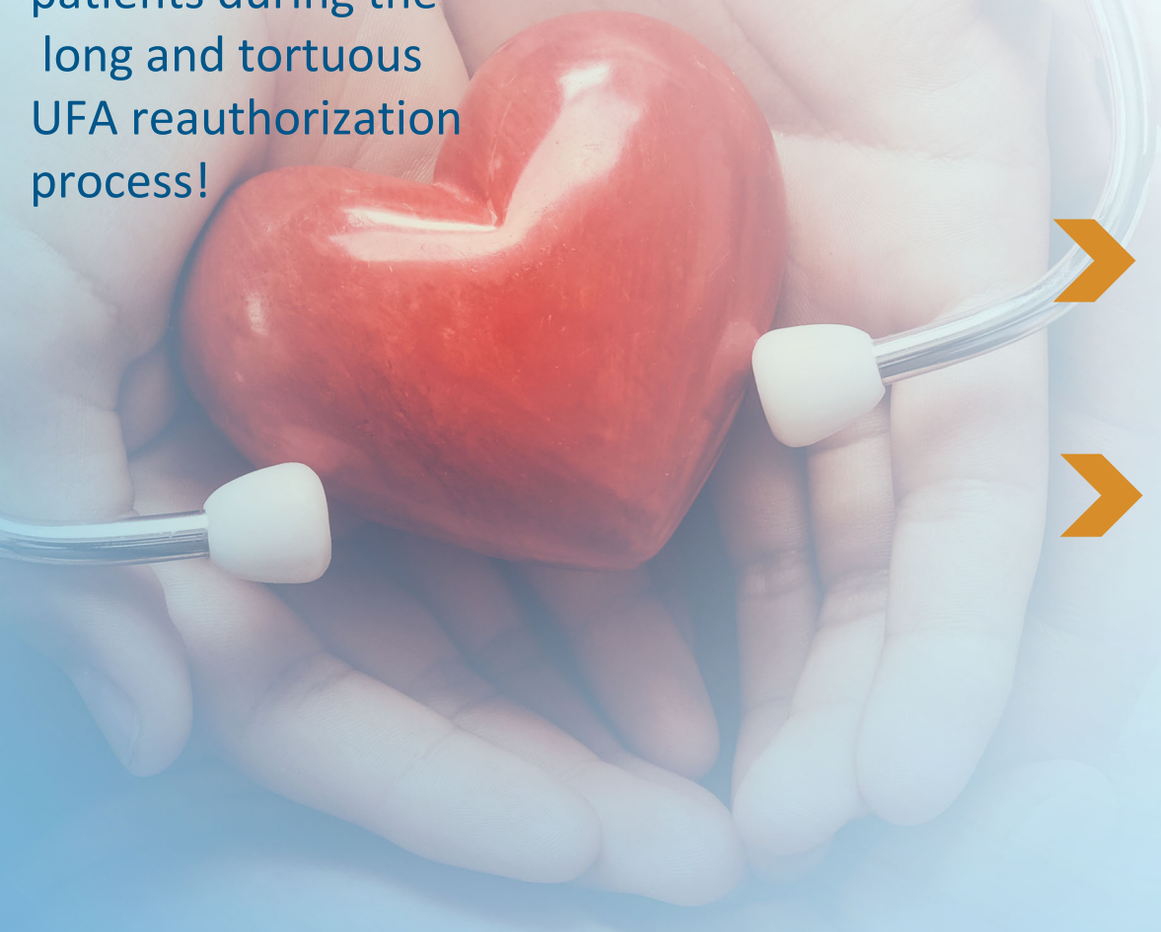
02

HR 6888/S. 4071 HEART Act

Proud of our bipartisan, bicameral Committee Member Champions

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

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02 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 House User Fee Act
PASSED HOUSE JUNE 8, 2022

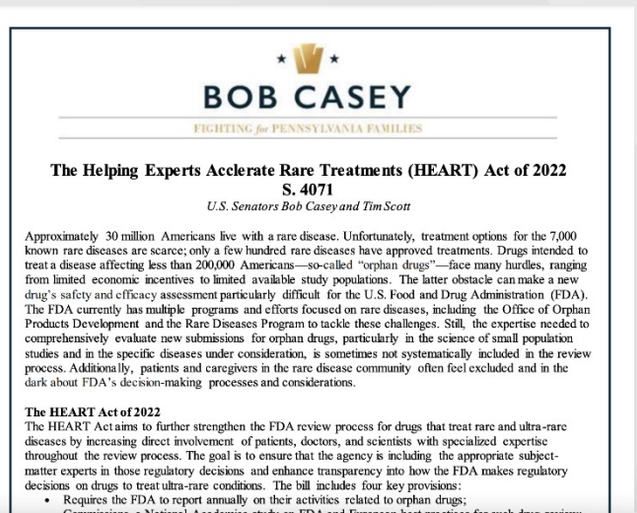
5 Included in Senate Use Fee Act Reported to Senate
JULY 13, 2022

6 Different language in House and Senate

7 "Clean" User Fee Act Enacted as part of CR Resolution
SEPTEMBER 30, 2022

8 User Fee Act Expires
DECEMBER 16, 2022

9 New Health-Related Package
ENACTED BY
DECEMBER 16, 2022



BOB CASEY

FIGHTING for PENNSYLVANIA FAMILIES

The Helping Experts Acclerate Rare Treatments (HEART) Act of 2022
S. 4071
U.S. Senators Bob Casey and Tim Scott

Approximately 30 million Americans live with a rare disease. Unfortunately, treatment options for the 7,000 known rare diseases are scarce; only a few hundred rare diseases have approved treatments. Drugs intended to treat a disease affecting less than 200,000 Americans—so-called "orphan drugs"—face many hurdles, ranging from limited economic incentives to limited available study populations. The latter obstacle can make a new drug's safety and efficacy assessment particularly difficult for the U.S. Food and Drug Administration (FDA). The FDA currently has multiple programs and efforts focused on rare diseases, including the Office of Orphan Products Development and the Rare Diseases Program to tackle these challenges. Still, the expertise needed to comprehensively evaluate new submissions for orphan drugs, particularly in the science of small population studies and in the specific diseases under consideration, is sometimes not systematically included in the review process. Additionally, patients and caregivers in the rare disease community often feel excluded and in the dark about FDA's decision-making processes and considerations.

The HEART Act of 2022
The HEART Act aims 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 enhance transparency into how the FDA makes regulatory decisions on drugs to treat ultra-rare conditions. The bill includes four key provisions:

- Requires the FDA to report annually on their activities related to orphan drugs;

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02

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.

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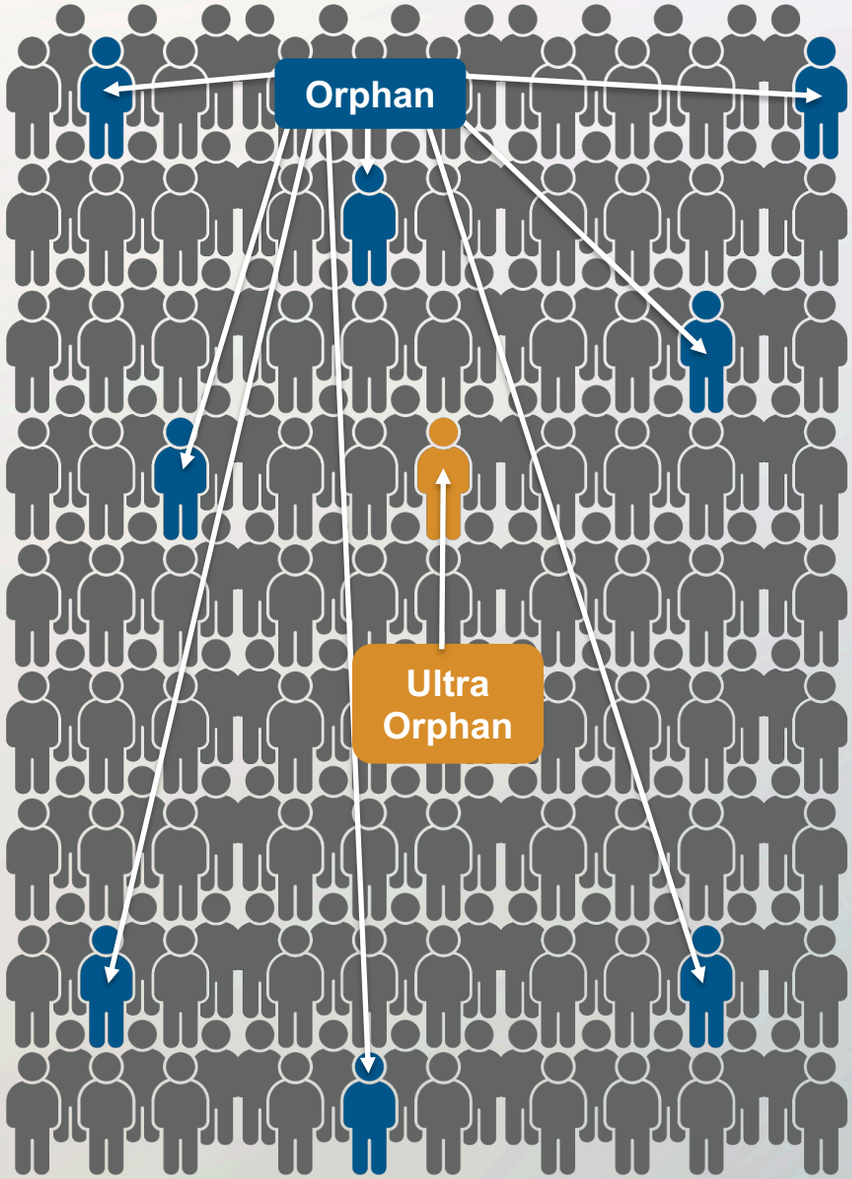
02

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!!!

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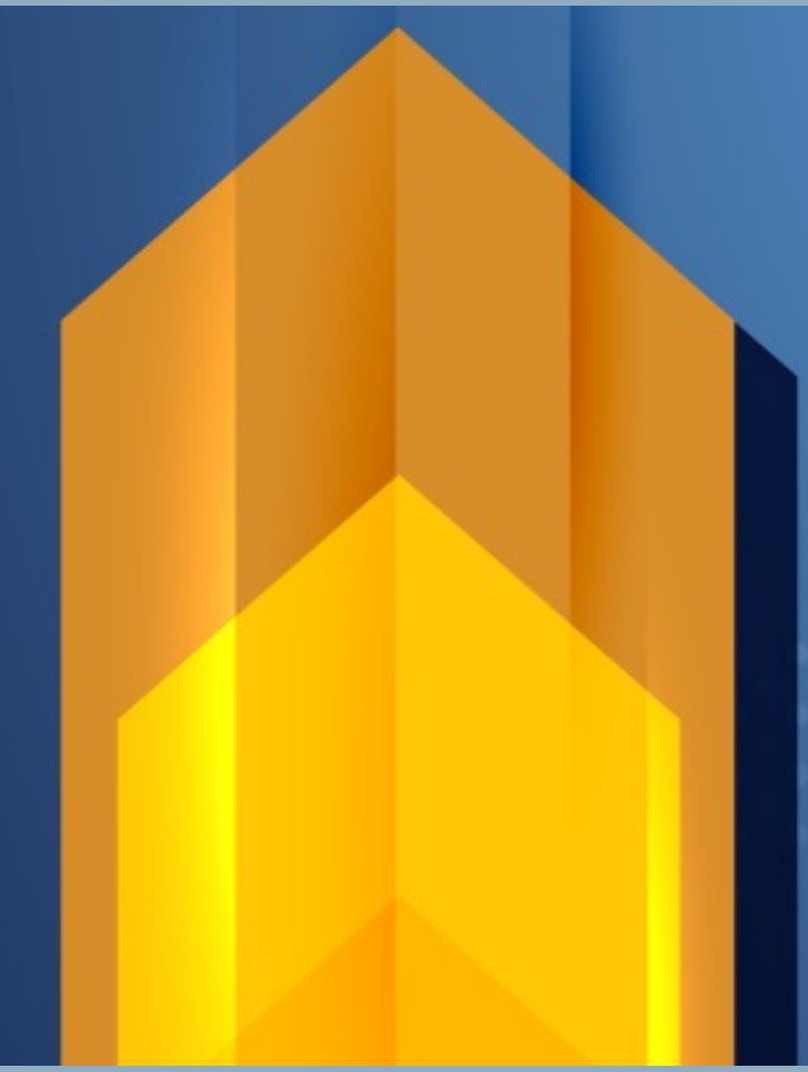
02 Accelerated Approval Pathway

Haystack Fought for More than Manufacturer Improvements



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;

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02 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

"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

"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

02 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

"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

"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

02 Report Language on DRGs

(Haystack Hint: bundled payment don't work for rare!)

Haystack Led the Way!!

The SENATE

Language states:

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;

VS.

Haystack Led the Way!!

The HOUSE

Language states:

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.

02 Continue to Drive Policy Solutions
Comment Letters (11)

JANUARY

NBPP: CMS must consider rare and ultra-rare community as it considers health equity policies

FEBRUARY

National Coverage Analysis: Coverage of novel therapy and importance of covering accelerated approval drugs off-label drugs

MARCH

Congress: Healthy Futures Task Force must consider needs of rare & ultra-rare as it considers future of telehealth

SEPTEMBER

PFS: Ongoing telehealth coverage uniquely critical for rare patients

CED: AHRQ should recommend CMS use informed consent and IRBs for CED research

AUGUST

PRIMARY CARE RFI: HHS can improve newborn screening programs to reduce the diagnostic burden for rare and ultra-rare patients

JUNE

HOSPITAL INPATIENT: DRG-based payment impedes access for rare disease patients

NOVEMBER

MEDICAID: Streamlining enrollment & eligibility will reduce beneficiary burden

EFFICIENCY & EQUITY RFI: Rare circumstances are critical to Medicare and Medicaid reforms

DECEMBER

PROVIDER DIRECTORY: Consider specialties & rare experience in developing directory

CAREGIVER STRATEGY: Consider extent of/unique nature of caregiver burdens i rare community

We also continue to evaluate and sign on to coalition letters on topics such as telehealth flexibilities and copay accumulators.

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03 Raise Profile and Collaborations
Initiatives

01

POV
Patient Oriented Value

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@haystackproject.org

02

AEI
Advocacy Empowerment Institute

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!*

03

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.

RCPC
Rare Cancer Policy Coalition

ROPE BRIDGE COLLABORATIVE

HEAT
Health Equity in Access to Treatments

03 Inside Health Policy & the Heart Act

"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."

03 Congressional Briefing
Rare in the Air ™

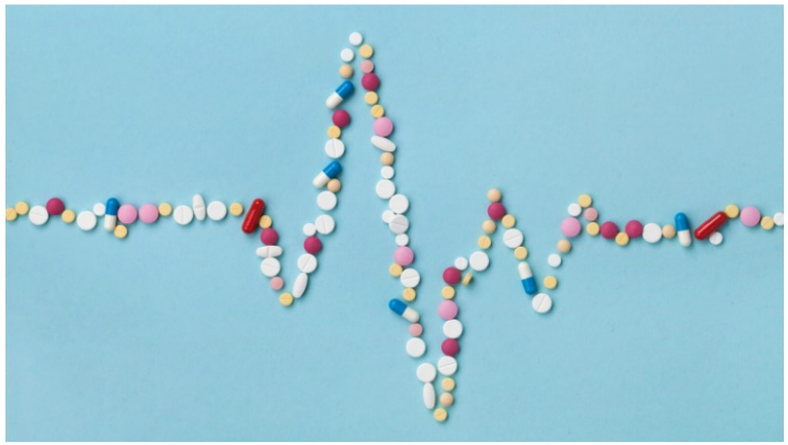
…We took advantage of all the *Rare in the Air* ™ that February brings …. ***HOSTING*** a Congressional briefing with esteemed panelists ahead of Rare Disease Day….

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

FIRST OPINION

The Access to Rare Indications Act could be a game changer for millions of Americans

By Darcy Krueger and Emanual Maverakis June 1, 2022 Reprints



ADOBE

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.

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

…***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

03 Virtual Finance Fly-In Hill Week

ISO: Senate Finance Champions

- ***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.***

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03 Raise Profile & Collaborations
Alliance Partners

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

American Brain Coalition

Black Women's Health Imperative

Alliance for Connected Care

Partnership for Innovation and Empowerment

Southern Christian Leadership GPI Global Policy Initiative

REN Rare Epilepsy Network

RAREX

RDDC The Rare Disease Diversity Coalition

Global Genes® Allies in Rare Disease

All Copays Count Coalition

IVI Innovation and Value Initiative

HAB Habilitation Benefits Coalition

ata™ Health. Virtually. Everywhere.

EveryLife Foundation for Rare Diseases

haystack@haystackproject.org

03

Raise Haystack Project's profile
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
Approximately 20 posts in 2022

FACEBOOK
facebook.com/HaystackProject
More than 25 posts in 2022

LINKEDIN
linkedin.com/company/haystack-project
Approximately 25 posts in 2022

OPPORTUNITY: Leveraging social media network with participants

03 Raise Profile and Collaborations

Sponsor 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 Hay stack 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.

AKCEA THERAPEUTICS · ALEXION · AMGEN · APPLIED THERAPEUTICS · AstraZeneca · Aurinia · Biogen · BIOMARIN · bluebirdbio · GRAIL · illumina · IONIS · IPSEN · Mitsubishi Tanabe Pharma · SANOFI · UCB · ultragenyx · ZOGENIX

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

agios
mirum
bridgebio
Amicus Therapeutics
AVROBIO
TRAVERE THERAPEUTICS
X4 PHARMACEUTICALS
BeiGene
Takeda
Incyte
Boehringer Ingelheim
Intercept
Genentech
A Member of the Roche Group
Orchard therapeutics
blueprint MEDICINES
ACADIA
biocryst
REGENXBIO
HOMOLOGY Medicines, Inc.
saniona
anavex LIFE SCIENCES Corp.
nkarta THERAPEUTICS
KYOWA KIRIN
Neurocrine BIOSCIENCES
santhera THEIR FUTURE - OUR FOCUS
arrowhead pharmaceuticals
Spark THERAPEUTICS
NOVARTIS
IOVANCE BIOTHERAPEUTICS
novocure
PTC THERAPEUTICS
SpringWorks THERAPEUTICS

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)