HAYSTACK PROJECT

*The Voices of Rare & Ultra Rare*

# YEAR IN REVIEW

· 2023 ·

# FROM THE CEO

*Welcome to the* **2023 HAYSTACK PROJECT**

# *YEAR IN REVIEW*

**Chevese Turner**
***Interim CEO***

Reflecting on 2023, I am struck by just how much Haystack Project has accomplished on behalf of rare disease patients. The collaboration with our 140 incredible patient groups has allowed Haystack to work hard to:

- Ensure the strongest possible implementation of the Heart Act, our signature bill that was enacted by Congress last year. Our presentations to GAO, NASEM, and FDA are examples of this work.
- Introduce the PROTECT Rare Act, to ensure rare disease patients can access medically necessary care.

As the interim CEO this year, I also worked closely with the team to identify and onboard a new CEO that I'm excited to have lead the organization in 2024. I will be returning to my Board role, ready to support Kara Berasi, a tireless rare disease mom who has brought so much energy to Haystack already and is excited to take on an even greater role. Her passion for Haystack's work is undeniable, and I expect you'll all soon see her shaking things up!

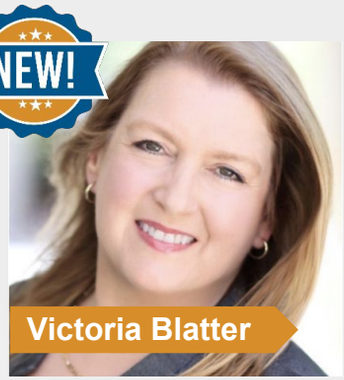
# WELCOME HAYSTACK PROJECT'S
## New CEO, Kara Berasi


Kara Berasi started working with Haystack Project in 2020 as a patient advocate after her son was diagnosed with a rare disease called PMM2-CDG, or congenital disorder of glycosylation. Over the past three years, Kara has been a very active member across a number of Haystack Project initiatives, including the HEART Act, the PROTECT RARE Act, Work Groups, grassroots efforts and more! She has a personal passion to change the landscape of care for patients and families living with rare diseases, and will bring that tenacity and focus, as well as her personal experience, to her role as CEO.

Before becoming a rare disease mom and advocate, Kara completed a residency in Pharmacy Health System Administration at Nationwide Children's Hospital in Columbus, OH and started her career as the Chief of Pediatric Pharmacy at University of Florida Health Shands Hospital. She went on to become the Assistant Director of Outpatient Pharmacy, and oversaw the hospital's four retail pharmacies, outpatient infusion center, and investigational drug service pharmacies.

Kara holds a Masters degree in Pharmacy Health System Administration from The Ohio State University and a Doctor of Pharmacy degree from the University of Toledo.

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












## Our Board continues TO GROW

Both Lynn Taylor and Victoria Blatter joined Haystack Project this year. Each bring more than 20 years of leadership experience in policy and public affairs and a wide breadth of knowledge around healthcare innovation, business, government, and a wide breadth of knowledge around patient access to care.

**Rebecca B. & Brad H.**
*Website Support*

**Andrew B.**
*IT Support*

**Lydia B.**
*Graphic Design*

***Joe C.***
*Finance/Operations*

***Tiara L.***
*Administrative Asst.*

**Kate G.**
*Researcher/Analyst*

**Misty O.**
*Manager*

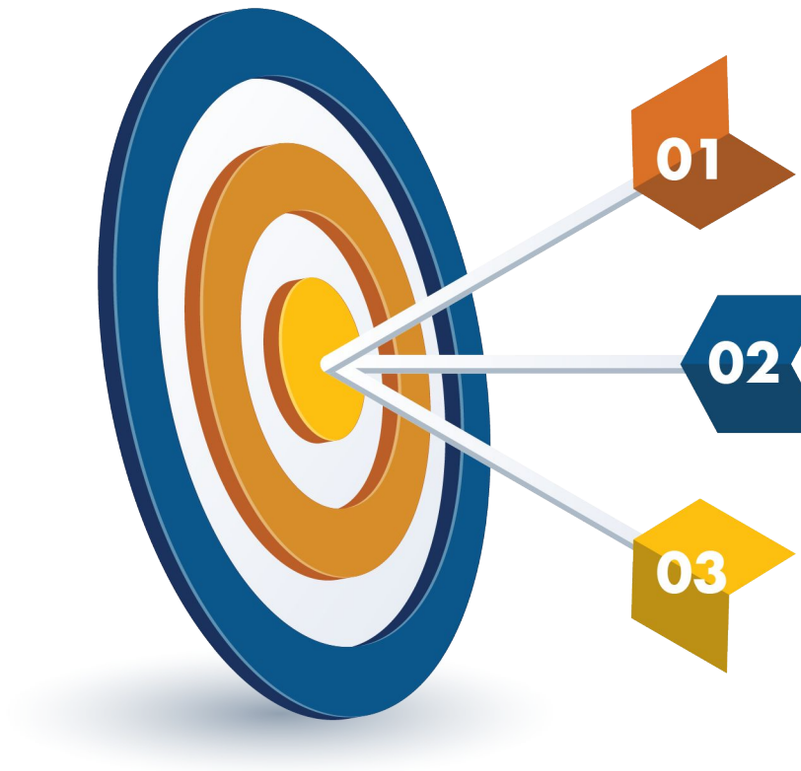
**Kay S.**
*Policy Director*

**Saira S.**
*Policy Director*

***Cara T.***
*Policy Director*

*haystack@haystackproject.org*

# HAYSTACK PROJECT'S
# 2023 *Goals*




## DRIVE
**Thoughtful, Tangible, Politically Feasible Policy Solutions**

## SUSTAIN
**Multi-Stakeholder Collaborations**

## RAISE
**Haystack Project's Profile**

**140+**
Ultra Rare Groups

**4**
Multi-Stakeholder Work Groups

**16**
Alliance Partners

**∞**
Hill Meetings

**2**
Bills

**4+**
Policy Priorities

**50+**
Listening Sessions

**100%**
**DEDICATION**

01

# HEART Act

*Proud of our bipartisan, bicameral Committee Member Champions*

## HEART ACT

**Haystack's first bill enacted in 2022!**

Considerable Haystack patient group engagement continued on these provisions in 2023, with Sen. Casey and Rep. Tonko's support…

- **FDA public meeting** to discuss approaches and opportunities for engaging patients, patient groups, rare disease experts, and experts on small population studies during the drug development process for rare diseases;
- **National Academies study** on US vs EU processes, flexibilities and authorities for evaluation of rare disease applications;
- **GAO report** on the effectiveness of FDA policies and practices re: PFDDs, patient experience, and patient views, consulting/engaging external experts, and training reviewers for rare disease reviews;
- **FDA report** on the number of rare disease applications submitted, approved, and for which the Agency consulted experts, as well as the size of the affected population;
- FDA *encouraged* to consult w/ patients, patient groups, & an expert selected by the group, to meet with FDA prior to application submission, and consult with small population studies expert if a disease-specific expert is unavailable;

# 01 POLICY SOLUTIONS

## Evaluating US vs EU Flexibilities and Authorities for Rare Diseases

***NASEM Study on Processes to Evaluate the Safety and Efficacy of Drugs for Rare Diseases or Conditions in the United States and the European Union …***

***From Patient Group Leaders:***

*"I was literally applauding during [Haystack's] presentation to the NASEM committee this morning"*

*"Fantastic presentation, thanks for speaking up for us!"*

*"Haystack hit the nail on the head on so many of the issues plaguing the intersection of ultra-rare diseases and regulatory affairs. Thank you!"*

NATIONAL ACADEMIES Sciences Engineering Medicine

Haystack Project was invited to speak at NASEM's inaugural meeting on the study Congress requested in the HEART Act -- evaluate flexibilities and authorities. at the FDA and its EU counterparts. Visit https://haystackproject.org/heart-act to watch.

01

# POLICY SOLUTIONS
## Duke Margolis Hosts Meeting for FDA

DECEMBER 14

### Duke-Margolis Center for Health Policy

*Advancing the Development of Therapeutics Through Rare Disease Patient Community Engagement*


## BILL TEXT

**(d) PUBLIC MEETING.—** In 2023, FDA shall convene one or more public meetings to solicit input from stakeholders regarding approaches to increasing & improving engagement w/ rare disease patients, groups, and experts to improve the understanding with respect to rare disease — (A) patient burden, (B) treatment options, and (C) side effects of treatments, including understanding risks of side effects relative to the health status of the patient and the progression of the disease or condition.

**REPORTS.—**180 days after each public meeting, FDA shall develop & publish a report on (A) the approaches discussed at the public meeting; and (B) any related recommendations.

## WATCH NOW

Visit
https://haystackproject.org/heart-act

*Select the first video and tune in at 3:43:49 to watch Haystack's panel, and at 3:56:51 when Haystack begins.*

Duke MARGOLIS CENTER for Health Policy

FDA U.S. FOOD & DRUG ADMINISTRATION

01

# POLICY SOLUTIONS

## Meeting Feedback Was Loud … and Frustrated

*Why are they presenting case studies that are successful? That's not the point!*

*Maybe we can find some next steps to collaborate on to move FDA forward [outside] of this meeting?*

*None of [Duke Margolis'] questions ask the FDA what we want.*

*How is everyone stuck on heterogeneity. It's insane. Some of us aren't even big enough to even consider that.*

*We still have patient groups with issues who do have PFDDs and listening sessions. They don't think that's the way to get FDA's attention.*

*Unfortunately, we are hearing a lot of excuses and not any solutions.*

*We are hearing a lot of 'I'm just doing my job" … and that's not what we need.*

*We already know the benefit of PAGs. I feel like we are being spoken to like we all just got into the rare disease space and don't know what we're doing. This is not amateur hour over here.*

*Well, this is now the second and third time [today] FDA has said they have no policies or procedures to ensure consistency across divisions.*

*The question isn't what these groups can share with each other that's helpful. The question is how can FDA do things differently. They're deflecting and trying to broaden the tent to who else needs to do better.*

FDA U.S. FOOD & DRUG ADMINISTRATION

Duke | MARGOLIS CENTER for Health Policy

01

# POLICY SOLUTIONS

## More Patient Group Reactions…

*Advancing the Development of Therapeutics Through Rare Disease Patient Community Engagement* | December 2023

Duke MARGOLIS CENTER *for* Health Policy

FDA U.S. FOOD & DRUG ADMINISTRATION

> **FDA has never reached out to our PAG for any of the many clinical trials in our rare and complex disease space – there have been over 10 clinical trials over the years – it's always been a request from the pharma company and usually as a silent partner in case there are any questions related to the patient lived experience. There never have been any.**

> **It seems like after the PFDD is held, FDA is hands off and then its up to the patient group to publish the report, and chase after the FDA to continue the conversation.**

> **It would be worthwhile to have an in-person program that could incorporate more people and a variety of breakouts to cover the variety of rare diseases and small companies as well. It would foster much more robust discussion and no hiding behind computers with no way to actually have a conversation. Need a conversation, not a presentation.**

01

# POLICY SOLUTIONS

## Overwhelmingly positive feedback for Haystack

Duke | MARGOLIS CENTER *for* Health Policy

FDA U.S. FOOD & DRUG ADMINISTRATION

*"I just wanted to thank [Haystack] for your input during the FDA session today."*

*"I feel you very well represented rare disease families!"*

*"Thank YOU, [Haystack]! You lead from the front."*

*"Fantastic presentation today @ the Duke Margolis meeting. Very effective. Thank you!"*

*Out of all the speakers/panelists at the workshop, Haystack's remarks were the most insightful and actionable. Thank you for being a voice for our rare community.*

*"Rock on, Haystack!!!!!"*

*"Mic drop 😊"*

*"Thank you, Haystack! This isn't the time to stroke FDA's ego. This is time to speak up. Thank you, Haystack!"*

*"Haystack did great! Strong and respectful."*

*"Thank you [Haystack] for your involvement today."*

*"GREAT presentation on the Duke Margolis meeting today – really raised some key issues! LOVED it."*

*– Academic medical center*

01

# POLICY SOLUTIONS

## Implementation of the HEART Act

**Haystack Project Led a Two-Hour Presentation and Discussion** with the Government Accountability Office (GAO) staff working on rare disease patient engagement, reviewer training, division assignment, consultation with experts, and more. Haystack groups took the time to learn about each other's experiences, then presented thoughtful solutions we hope GAO will incorporate as recommendation in this report.

**GAO report due** 18 months after enactment assessing effectiveness of FDA policies & practices in considering relevant PFDD data/ information, patient experience data, views of patients, training of FDA personnel, consultations/engagement w/ stakeholders and external experts, consistency of policies/practices across review divisions; Include recommendations to improve effectiveness, consistency, and coordination of policies, practices, and programs.

> ***"Thanks Haystack, for being our powerhouse. Brilliant insights from everyone. I hope the GAO team was as blown away as I was by the knowledgeable, thoughtful insights that everyone shared. Grateful to be a part of this amazing team."***
>
> *~ Participating Haystack Member*

01

# H.R. 6094
## Protect Rare Act

**PROTECT RARE ACT**

Providing Realistic Opportunity To Equal and Comparable Treatment for Rare

- The bill permits Medicare and Medicaid to use clinical guidelines and peer-reviewed literature to allow for coverage of rare disease treatments.
- This bill is supported by more than 60 rare disease organizations.
- The bill does not provide ‘special treatment’ for rare diseases; rather, it levels the playing field for access to those living with more common conditions.
- The bill requires private payers to create an expedited review pathway for formulary exception, reconsideration, and/or appeal of any denial of coverage for a treatment prescribed for a patient with a rare disorder.

01

# POLICY SOLUTIONS
# PROTECT Rare Act

## PROTECT RARE ACT

Providing Realistic Opportunity To Equal and Comparable Treatment for Rare

*Building on an oncology precedent, H.R. 6094 expands the definition of 'medical necessity' to improve access to the treatments treating clinicians say their rare disease patients need.*

**HAYSTACK PROJECT:**

**IDENTIFIED** – – –
the problem after listening to numerous patients and patient groups

**CONCEIVED** – – –
a solution

**RESEARCHED** – – –
precedent

**DRAFTED** – – –
legislation

**SEARCHED** – – –
for champions

# 01 “PROTECT RARE” ACT, H.R. 6094

*Builds on precedent to expand definition of ‘medical necessity’ for rare*

## Haystack Led Targeted Fly-In:

*Well-Received by the Patients that Participated and by Hill Staff for its Actionable Content*

*Introduction Needed*

### SENATORS

*Senators Tills (R-NC) and Cassidy (R-LA) have a “want ad” out for a Finance Democrat*

*Race is on for CMS TA and Chairman Wyden engagement*

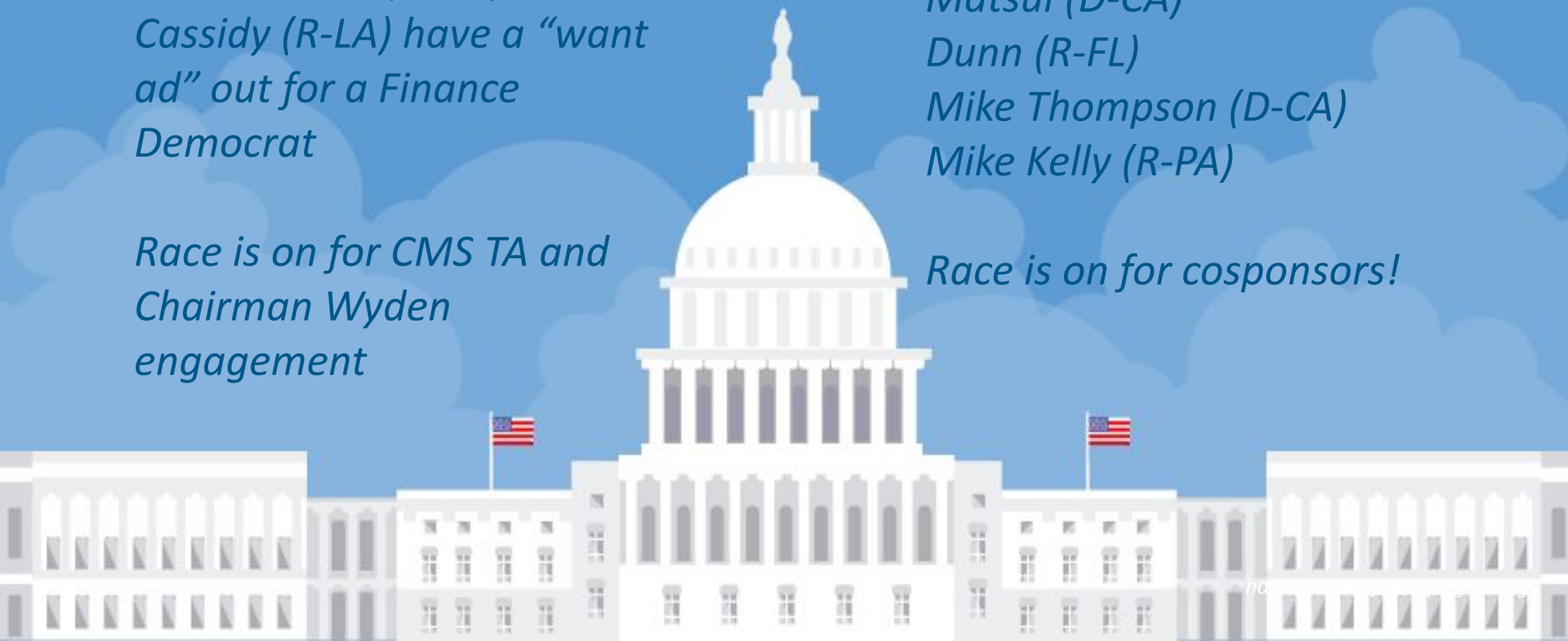
VS.

*Introduced by*

### REPRESENTATIVES

*Representatives Matsui (D-CA)*
*Dunn (R-FL)*
*Mike Thompson (D-CA)*
*Mike Kelly (R-PA)*

*Race is on for cosponsors!*



01

# INFLATION REDUCTION ACT
## All Things Rare

***Focus on Alleviating the Impact of CMS' QSSD Interpretation (IRA) on Rare and Particularly Ultra Rare. We believe this will align with and improve upon existing efforts related to the Orphan Drugs Exclusion.***

***Topics included:***

CMS defines 'qualifying single source drug' proved a complex area as groups understood the impact on initial and future indications or applications

**Drug Shortages:** companies reducing or withdrawing inventory, which could create 'artificial shortages'

**Inflation Rebates**: the impact of inflation penalties in shortage or in the context of value based arrangements

**Small Biotech Exception:** much of the innovation in rare come from really small companies

**Orphan Exclusion:** the distinction between 'indications' versus 'designations.' and CMS's proposal to count 'active ingredient' as one "qualifying single source drug" instead of each NDA/BLA.

The end of the 5% catastrophic out-of-pocket for patients starting in 2024

Advocating for CMS to use its discretion in favor of rare diseases

**Data elements:** value of the treatment, other drugs that treat the same condition. These elements could broaden CMS's thinking

**The Maximum Fair Price (MFP):** how this affects rare disease patients using medications off-label

**Smoothing**: Concern that out-of-pocket cap / smoothing provisions of the IRA "inoculate" patients from concerns about high prices

01

# POLICY SOLUTIONS
## Inflation Reduction Act

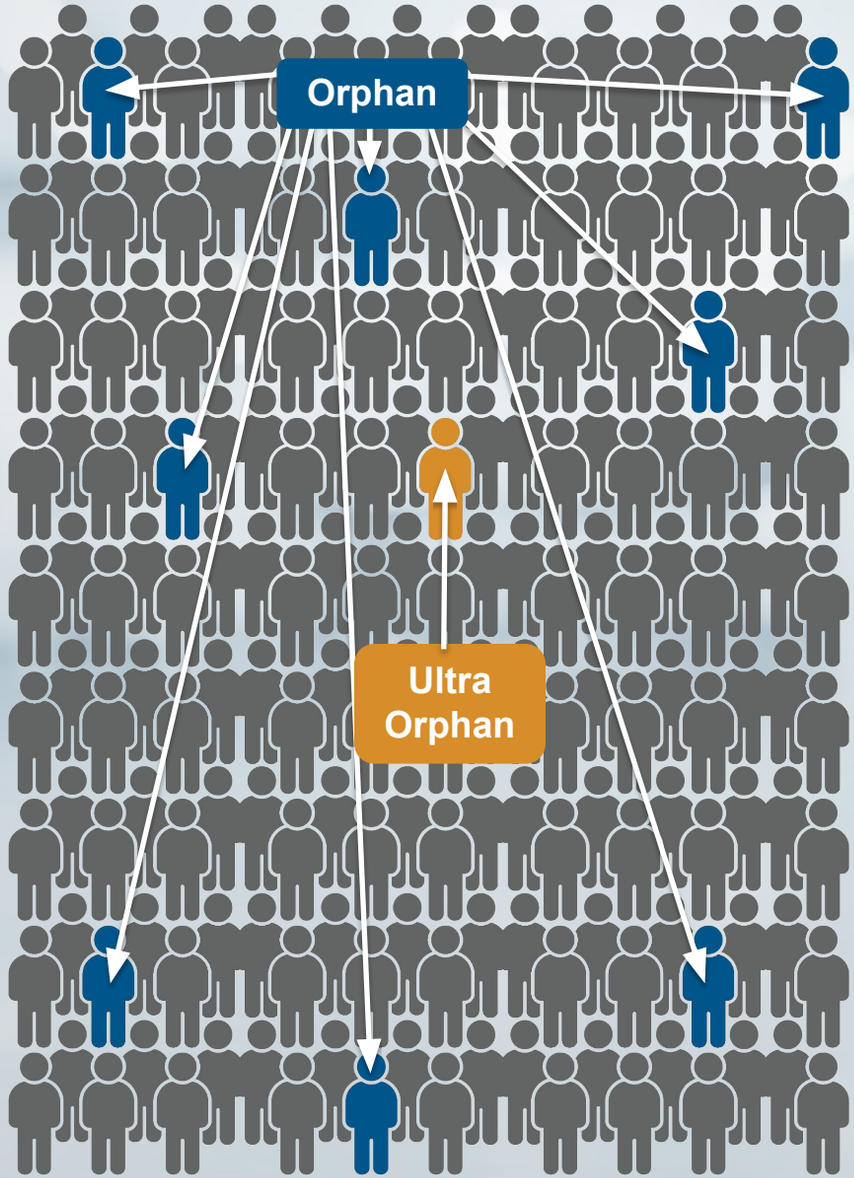
- *Prepared groups for* **LISTENING SESSIONS**
- *"Staffed" groups at* **CONGRESSIONAL MEETINGS**
- *Educated groups about possible changes in access and future* **DRUG DEVELOPMENT**
- *Galvanized grassroots support to communicate concerns about* **PROTECTING THE "*NEXT* TREATMENT OR CURE**,*" increasing likelihood of off-label use in rare diseases, especially ultra rare*
- *Helped groups navigate the dichotomy of* **"OPPOSING LOWER DRUG PRICES"** *versus being vested in their next treatment*

***. . . Haystack has been educating our patient groups, bringing in key speakers, commenting to CMS, and meeting with Congress about all things IRA related to rare disease drug development, commercialization, and access . . . .***

01

# RARE AT RISK IN THE IRA

*Price Negotiation Exemption and Active Moiety Interpretation Risk Damaging Gains in Rare*




- ***House Committee deliberations leading to introduction of the PROTECT RARE Act 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 and CMS' interpretation of "eligible drug" 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 CMS interpretation of active ingredient…***

***CMS' Interpretation of IRA 'eligible drug' makes passage of H.R.6094 more urgent than ever!!!***

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01

# VALUE FRAMEWORKS LEGISLATION
## CMS and Hill Discussions Underway

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


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# 01 Continue to Drive Policy Solutions
## Comment Letters

### JANUARY

**AHRQ CED criteria:** Mandating research participation to access medically accepted treatments requires central IRB review and other protections for human subjects..

**Senate dual-eligibles RFI:** Care coordination models must consider ultra-rare patients.

**EHB RFI:** Urged greater focus on reduced time to diagnosis, access to the right treatments, and newborn screening.

### FEBRUARY

**Medicare Advantage:** Quality measures and risk adjustment mechanisms must fully incorporate quality care for rare patients.

**PCORI:** Contributed ultra-rare perspective on "Components of 'Patient-Centered Value' in Health and Health Care"

### MARCH

**Prior Authorization:** Rare disease patients face a labyrinth of claim denials, PA and inappropriate step therapy protocols to access the care they need.

**IPPS:** CMS must develop a mechanism to ensure inpatient access to rare disease treatments. MS-DRG system fails rare patients.

**Part B & D Inflation Rebate Guidance:** CMS should not penalize manufacturers facing ingredient shortage or adopting value-based pricing.

**IRA Small Biotech Exemption:** Since rare treatments are frequently developed by clinical stage biotech, CMS should clarify its processes and streamline eligibility mechanisms to minimize burden and uncertainty.

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

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# 01 Continue to Drive Policy Solutions
## Comment Letters

### APRIL

**IRA Negotiation Initial Guidance.** Urged CMS to preserve innovation in ultra-rare by defining eligible drug based on NDA/BLA and fully implementing orphan exemption.

### MAY

**IRA data elements ICR.** Emphasized that limited options in ultra-rare must be considered in assessing treatment value.

### JUNE

**IPPS:** Re-emphasized the need to account for orphan drug costs in reimbursing for low volume inpatient stays. MS-MRG system disfavors rare.

**IRA counteroffer ICR.** Urged CMS to reconsider applying attestation penalties to non-commercial commenters and maintain transparency on stakeholder input.

**Medicaid, CHIP Managed Care.** Expressed support for CMS proposals to improve access and quality of care.

**Part D Redesign RFI.** Urged CMS to engage patients throughout implementation and monitor impact of UM tools on access.

**Dual eligibles bill.** Supported Senator Cassidy's draft on Integrated Care Programs and asked that the bill address the longstanding hardship rare patients face when unable to access an out-of-state disease-specific specialist.

**Ensuring access to Medicaid services.** Urged CMS to require beneficiary advisory groups include rare patients, and pmt adequacy inquiries consider rare, including need for in-home nursing care.

### JULY

**2ND IRA counteroffer ICR.** Applauded CMS for including our requests in 2nd IRA negotiation data elements.

**340B RFI.** Urged adoption of covered entity requirements to improve access for ultra-rare.

### AUGUST

**NCD evidence review guidance.** Opposed a one size fits all approach to coverage due to unintended consequences for ultra-rare, including off-label use.

**CED guidance.** Opposed CED for FDA-approved drugs; cautioned CMS on ethical requirements for human subject research.

### SEPTEMBER

**Part D "smoothing" guidance.** Offered CMS insights from rare disease community; suggested improvements to further the Program's goal of ensuring that beneficiaries can afford the treatments they nee

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02

# MULTI-STAKEHOLDER COLLABORATION

## Deep Dive Workgroups


### MEDICAL NECESSITY / HR 6094

» Blocking and tackling towards passage of the PROTECT Rare Act

» **Compendia Strategy kicks off in 2024** NEW!

### HEART ACT IMPLEMENTATION

» NASEM, GAO, FDA, Duke Margolis….

### HP50

» Pilot

» Gap filling in state advocacy efforts

### FIRST LOOK WORKGROUP - *HOLD*

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.

### INNOVATION MODELS

» The Cell and Gene Therapy Access Model

» The Accelerating Clinical Evidence Model

02

# MULTI-STAKEHOLDER COLLABORATION

## Joined National Health Council

### NHC MISSION

The mission of the National Health Council is to provide a united voice for the 160 million people living with chronic diseases and disabilities and their family caregivers.

### NHC VISION

We envision a society in which all people have access to quality, affordable health care that respects personal goals and aspirations to promote their best possible health outcomes.

### NHC VALUES

Integrity, collaboration, and transparency guide all aspects of the National Health Council's interactions among the diverse sectors of the health community. We are patient-focused and forward-thinking, ever mindful of our mission.

02

# MULTI-STAKEHOLDER COLLABORATION

## Collaboration

**PROTECT RARE ACT**
Providing Realistic Opportunity To Equal and Comparable Treatment for Rare

Supported by **60+** **rare disease** organizations.

02

# MULTI-STAKEHOLDER COLLABORATION

## Educational Speaker Series

*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!*

**COURTNEY JOHNSON**
Principal, Alpine Group
One of the Hill's Top Lobbyists of 2023

**LISA GOLDSTEIN**
Health information Privacy Specialist with the US Department of Health and Human Services Office for Civil Rights

**STEVE USDIN**
Senior Editor at BioCentury Publications

**JODI G. DANIEL**
Partner, Crowell & Moring; Managing Partner, Crowell Health Solutions

**JULIETTE CUBANSKI**
Deputy Director of the Program on Medicare Policy at KFF

**PRISCILLA PARRILLA**
Director, Vendor Drug Program Office of Medicaid and CHIP Services Texas Health and Human Services

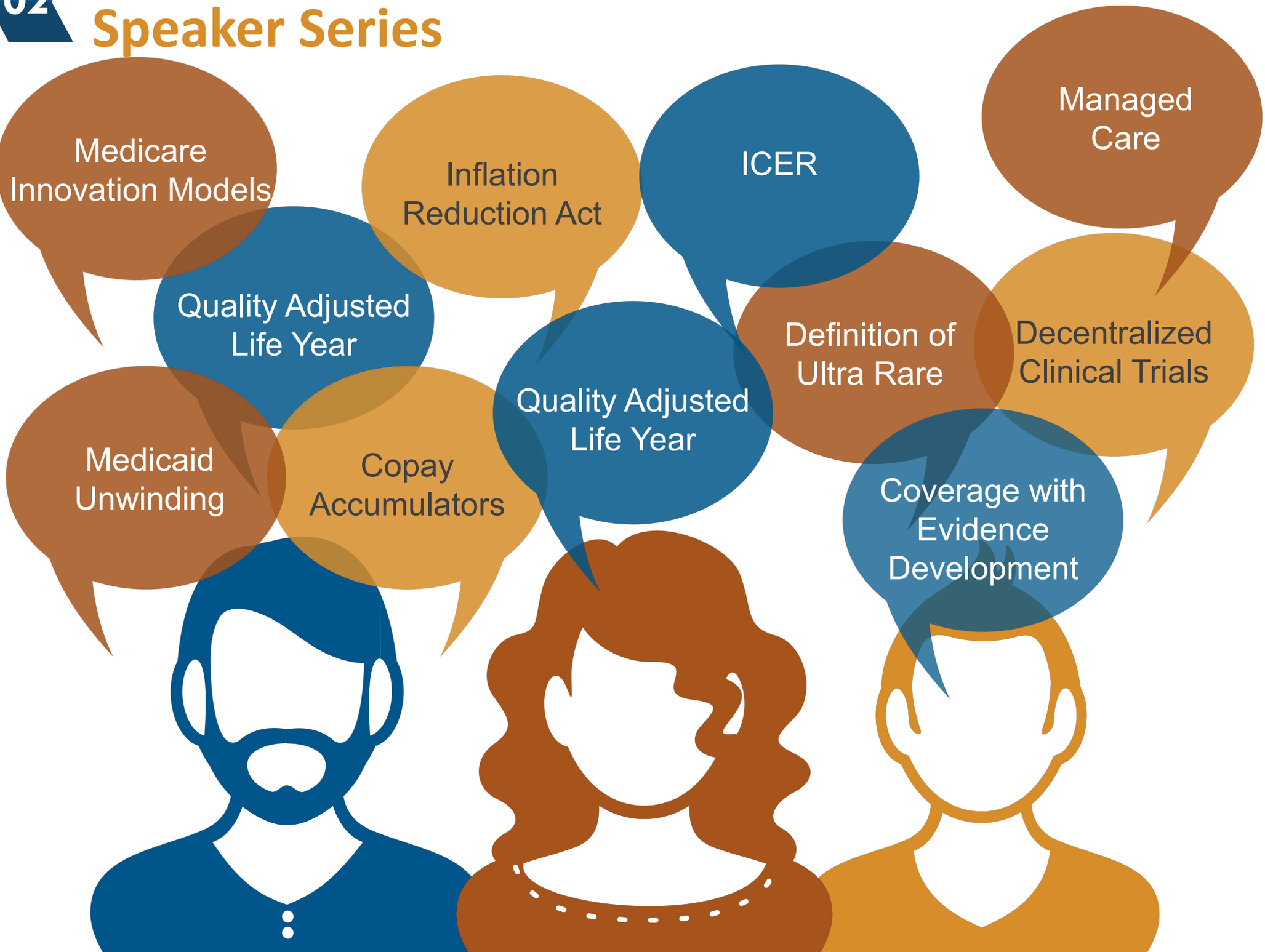
**MATT SALO**
Founder and CEO of Salo Health Strategies

**MICHAEL BAGEL & NISSA SHAFFI**
Associate Vice President and Associate Director of Public Policy for the Alliance of Community Health Plans

02

# MULTI-STAKEHOLDER COLLABORATION

## Speaker Series



02

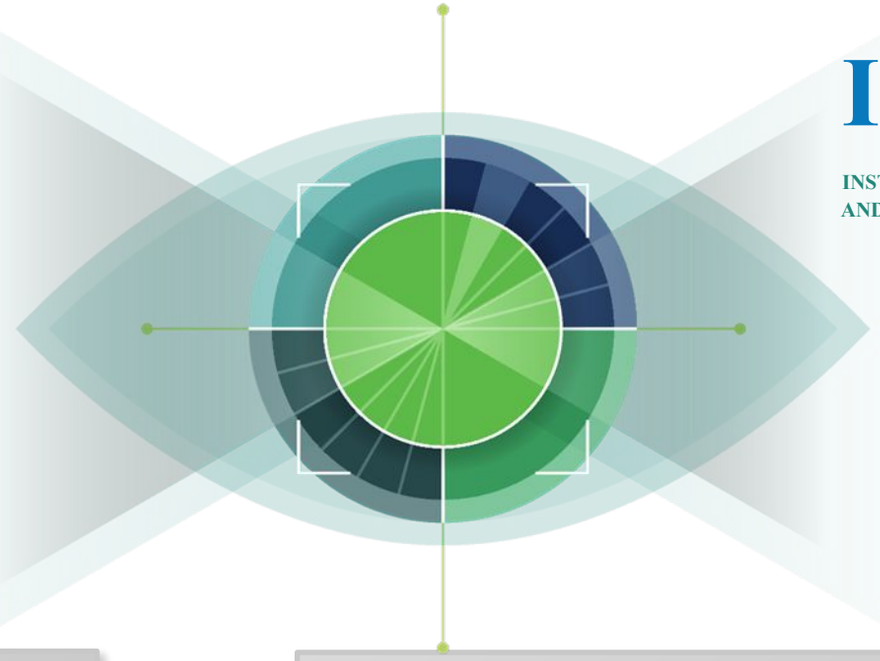
# MULTI-STAKEHOLDER COLLABORATION

## Eyes on ICER

ICERi

INSTITUTE FOR CLINICAL AND ECONOMIC REVIEW

***Haystack is active in a number of ways:***



- Educational webinars
- Comment Letters
- ICER Listening Session
- Rare Disease Week panel discussion
- Access to ICER Analystics™
- Fair Access Project
- Voting Panel Member for Rare Perspective
- Coordinating groups for upcoming reviews

# 03 RAISE PROFILE

## ISO: Senate Finance DEMOCRAT

Targeted HOUSE AND SENATE COMMITTEE Fly-Ins continued this year!

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.

Successes = REP. DUNN and SENS. TILLIS and CASSIDY

03

# RAISE PROFILE & COLLABORATIONS

## State Level Advocacy


### AUGUST

Groups came together to undertake a “planning session” about their need/interest in state-level advocacy, and how Haystack might support them. Clear takeaway = appetite for more is urgent.

### SEPTEMBER

Haystack hosted a call with industry to discuss rare disease patient advocacy at the state level.

### OCTOBER

In October, patients and companies came together to hear from Matt Salo, who recently retired from the National Association of State Medicaid Directors (NAMD). He discussed the gaps and opportunities in state-level advocacy in the rare community

### NOVEMBER

In November, Haystack considered specific goals, resource needs, and an implementation timeline to serve our patients.

### DECEMBER

In December, we regrouped with the most active of Haystack’s patient groups to discuss feasibility, resources, likelihood of success, and next steps

### JANUARY

January – kick off with identification of initial target states and engagement with HP50 Work Group members on ‘fanning out!”

# 03 RAISE PROFILE


*A new ad hoc committee of the National Academies of Science, Engineering, and Medicine (NASEM) has been tasked with conducting a study on the processes for evaluation of drugs to treat rare diseases or conditions in the U.S. and the E.U.*

One particularly insightful session described the series of events which culminated in the NASEM study. This background information was shared by Saira Sultan, a policy consultant at the Haystack Project, who led the charge on getting the HEART Act passed last year. Started in 2016, the Haystack Project is a nonprofit coalition of 140+ ultra-rare disease patient organizations working together to advance patient access to novel treatments, specialists, and diagnostics for extremely rare conditions.

According to Sultan, it all started with a patient group outside of Haystack that had been shopping the legislation around the Hill. The group was encouraged by Congressional staff to move forward with the bill; however, the group recognized that they needed partners to gain traction. As Sultan described, the group pitched it directly to the 100+ organizations which belong to Haystack and was met with "resounding interest." The groups are hopeful that this study will shed light on the root cause of these issues, and how they can be addressed moving forward.

The patient groups who were pushing the HEART Act forward last year are eager to see the NASEM study touch on several key issues. First, they want committee members to carefully inventory the flexibilities, authorities and/or mechanisms available to regulators in the U.S. and the E.U. which could be used in the context of rare diseases. Secondly, the groups are requesting that the NASEM study assess how both regulators consider supplemental data—including data associated with open label extensions studies and expanded access programs specific to rare diseases or conditions—during application review. Interestingly, a third point that did not actually originate from the patient organizations but was instead promoted by the FDA, is an assessment of the "collaborative efforts between U.S. and E.U. regulators on: product development programs under review, policies under development and/or recently issued, and scientific information related to product development /regulations."

# 03 RAISE PROFILE

Patient groups would like the committee to carefully consider not just which flexibilities regulators have, but if, how and when they are actually using them. “I can't tell you how often in the back and forth with FDA and Congress and Haystack Project as we were negotiating the language for each of these provisions, that we were repeatedly told by FDA that they have all the flexibilities and authorities they need, and that this legislation was not needed. This study was not needed,” Sultan said. For this reason, Sultan said it’s key that the study not just assess which flexibilities FDA already has but how they are using them, whether they are being used consistently, whether some divisions are using them while others aren’t, and more.

Patient groups see many of the current activities performed by FDA as performative exercises that lack meaningful impact, said Sultan. Putting it bluntly, she described FDA’ s Patient-Focused Drug Development sessions as “dog and pony shows.” While a few organizations in the Haystack Project have had positive experiences with these meetings, the overwhelming majority have not. “It’s not helpful for them to know that the Office of Patient Engagement is attending or that the Office of Orphan Drug Products is attending because they in no way affect the decision on the actual application,” she reinforced. Furthermore, patient groups don’t know whether any reviewers attend these meetings, and if they do, Sultan said they’re not actively participating or asking questions, “How is it possible that a disease you have never heard of—that affects 500 people in the United States—that you have not a single question or follow-up clarification for them?” Yet preparing for and attending these meetings is burdensome for patient groups, not just in terms of the logistics but also in terms of monetary investment. So, she clarified, it’s frustrating for patients to feel as though the meetings are not actually being used to make decisions on applications.

Sultan also stressed the importance of a comparison between what is being done in the U.S. versus the E.U., although she noted that it wasn’t included in the committee’s current “statement of work.” She referred to an example where a product was approved by the EMA but not by the FDA. In this case, the European regulators were willing to accept additional data that had been gathered after the marketing application was submitted, but prior to the final decision on the application. In contrast, the FDA was unwilling to consider the additional year of data. She remarked, “It doesn’t mean that they didn't have the flexibility or authority to do so, but did they use that flexibility or authority? Or did they simply not have it? What they told the patients was that ‘they simply didn't have it,’ and so, the comparison I think is really important.”

Patients and patient advocates are also hoping for a review of the remit and composition of FDA advisory committees. “We were told repeatedly during negotiations on the HEART Act that they [FDA] had the flexibility and did not need help—or did not need legislation—to be able to ask expert clinicians in the actual disease to participate in an advisory committee meeting in a non-voting fashion, for example,” said Sultan. But she said patient groups want to see these experts in actual voting roles. She continued, “And we're often told, ‘Oh yes, we have expertise on the advisory committee’ but when the patient group asks who that is, it's a generic geneticist. That is not the same as a clinician with actual experience treating an extremely rare disease.”

03

# RAISE PROFILE & COLLABORATIONS

## Health Equity in Access to Treatment (HEAT) Project

***HEAT*** *seeks to understand variabilities in access, disease burden, and lived experience of the patient and caregiver communities. Haystack Project undertook its first HEAT project this year in partnership with SRNA, an active Haystack member.*

HEAT
Health Equity in Access to Treatments

- Focused on Neuromyelitis Optica Spectrum Disorder (NMOSD)
- Appears to be more prevalent in those of African and Asian descent.
- While people can be diagnosed with NMOSD at any age, the average age of onset is about 40.
- Women are at least four times as likely to have the recurring form of the disorder.

**THROUGH PROACTIVELY IDENTIFYING:**

**Barriers to diagnosis, specialists, & treatments**

*that shape a Black, Latinx, Asian, Native American patient's journey (and may be further differentiated between male and female patients)*

+

**Experiences of Minority Patients**

*And how they might differ from the patient journey in populations captured in registries, natural history studies, and clinical trials*

=

***The HEAT initiative is designed to better equip patient groups to address the needs of the full set of communities they seek to represent and drive advocacy initiatives toward reducing racial inequities in access and health outcomes.***

SRNA
connect. care. cure.
Siegel Rare Neuroimmune Association

Genentech
A Member of the Roche Group

HORIZON®

# 03 POV IS BOTH
# Patient Oriented Value & Point Of View

POV
Patient Oriented Value

*While value frameworks would assign marginal value to preserving vision in patients who've already lost much of their visual function, CHM patients worked with Haystack to articulate their perspective in the* ***Patient Oriented Value (POV)© Report for CHM****. This work underscores how important it is to be proactive as a community if we want the patient voice to guide how payers value treatments. Haystack brings together experts in science, economics, biostatistics, survey creation, data analysis, and reimbursement policy to support patient groups like CRF with grants from our partners.*

## *Also of* NOTE …

Our **Patient Oriented Value (POV)© Report for Uveal Melanoma** was released before FDA approved Immunocore's Kimmtrak for select patients with unresectable or metastatic uveal melanoma. Uveal melanoma can be fully resolved by treating or removing the primary tumor in approximately 50% of patients; the other 50% will develop metastatic disease, which had been considered universally fatal. The POV Report on UM assessed, among other things, patient need and access to the test establishing which side they were on and for the very real need of enrolling in clinical trials for Kimmtrak. The oncology community has echoed our POV recommendation now that an effective therapy has been approved for metastatic disease.

Our UM POV report also emphasized the need for a biomarker to identify measurable residual disease (MRD) so patients can start anti-cancer treatment before metastatic disease is evident. One clinical trial studying the effectiveness of an investigational blood test to detect MRD and another investigating adjuvant use of Kimmtrak in uveal melanoma for high-risk UM patients have been initiated in Europe, but not in the U.S. We hope that US studies will be initiated soon and that our POV findings on the long and variable time period from initial UM treatment to emergence of metastatic disease will inform U.S. clinical trial design. The 6-month timeframe outlined in the European studies appears grossly insufficient to capture the potential real-world benefit of adjuvant Kimmtrak on UM patient survival.

03

# RAISE PROFILE
## Senate Aging Committee Witness

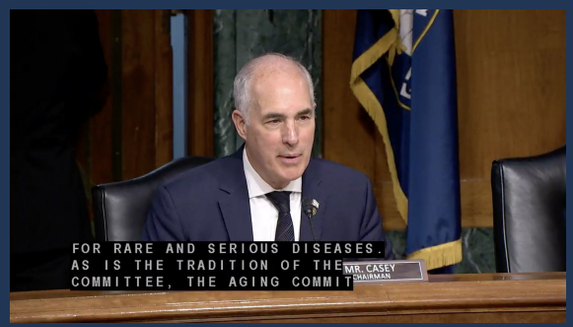

### Chairman Robert Casey

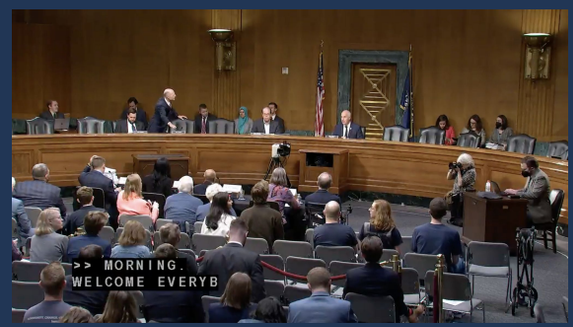
Senate Aging Committee Hearing
October 2023

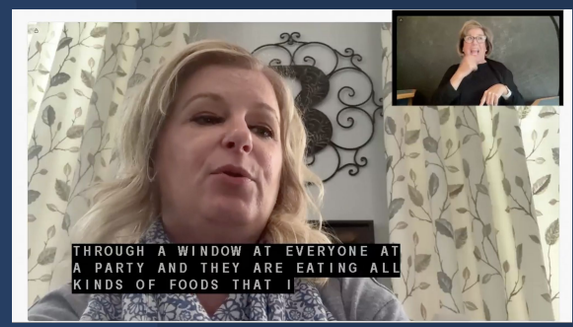
Haystack has continued to engage **Senator Casey's** help in highlighting FDA progress on rare diseases.

We continue to share examples of where FDA must exercise its authority/flexibility in review of NDAs and BLAs for rare diseases.

We prepared a Pennsylvania are disease patient to testify – allowing her story to give weight to our educational efforts on the need for FDA to exercise its flexibilities when it comes to rare.



# 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

Putting Patients First® National Health Council

American Brain Coalition

Black Women's Health Imperative

Partnership for Innovation and Empowerment

Southern Christian Leadership Conference GPI Global Policy Initiative

Alliance for Connected Care

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

EveryLife Foundation for Rare Diseases

ata™ Health. Virtually. Everywhere.

*haystack@haystackproject.org*

03

# RAISE HAYSTACK PROJECT'S PROFILE

## Social Media

UNDER CONSTRUCTION!
We are revising our approach to social media. Please continue to follow us at the links below as we update things!

### X (Formerly TWITTER)
twitter.com/HaystackProject

### FACEBOOK
facebook.com/HaystackProject

### LINKEDIN
linkedin.com/company/haystack-project


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

# POTENTIAL PARTNERS
## On Our Radar

# 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 (140+ groups strong)