

HAYSTACK PROJECT

The Voices of Rare & Ultra Rare

FEBRUARY RECAP

FEBRUARY 2024



CELEBRATE

*everything we've accomplished
already in the first two months
of the year!*

JANUARY

Accomplishments

- 17** Monthly “Member Meet-Up” on pending/new government proposals/ bills.
- 18** Haystack joins NHC’s Government Relations Meeting.
- 22** Haystack **meets with FDA** on patient-friendly PFDD improvements
- 22** Haystack hosts member prep call for groups meeting with CMS’ Innovation Center
- 25** Haystack **meets with FDA** on the START pilot

- 22** Haystack files comments to Sen. Cassidy’s RFI on “Improving and Protecting Access to Gene Therapies”



[READ HERE!](#)

- 29** Haystack **meets with NCCN** on rare disease evidence reviews

- 30** Haystack raises awareness on IRA concerns at Ovarian Cancer Research Alliance’s Industry Council meeting

FEBRUARY

Accomplishments

- 5** **Pink Sheet** reports on Haystack participation on Duke Margolis’ meeting on Rare



[READ HERE!](#)

- 1** Haystack responds to E&C office requests for topics/Qs for their briefing with FDA

- 6** Haystack delivers 50 rare group signatures to E&C leadership to ban use of QALYs

FEBRUARY RECAP

FEBRUARY 2024

FEBRUARY *Accomplishments*

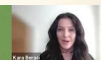
6

Haystack CEO Kara Berasi on NASEM panel on “Evaluating Safety and Efficacy of Drugs for Rare Diseases in US and EU



[WATCH HERE @1:40:30](#)

Meet Blaise!



8

Haystack **meets with CMS’ Office of the Administrator**

8

Haystack participates in RDDC’s Policy Work Group

13

Haystack CEO, Bd Member and members **meet with CMMI** on their CGT and AA Pathway models

29

Protect Rare Act = 16 cosponsors! Ask your Congressperson to cosponsor **HERE**.

22

Kara Berasi, CEO, and member groups host webinar on H.R. 6094 for patients attending Rare Disease Week Hill meetings



[REQUEST COSPONSOR](#)

29

House E&C HEARING included Protect Rare Act! Big milestone!

A Note from the CEO

We are so proud of the work we do on behalf of the almost 150 ultra-rare patient groups that participate with us – your ideas, requests, and suggestions become our projects. Join us and contact me CEO and rare disease mom, **Kara Berasi**, if we can help. We are here for you!

Check out our “[2023 Year in Review](#)” for an impressive lineup of accomplishments in 2023!

DUES, DUES, DUES! Pay your 2024 dues [here](#). Please help us keep up all the good work you've come to count on us for!

FDA AND RARE

Shout out to the FDA for two follow-up meetings and counting since the Dec. 14th FDA/Duke Margolis meeting. We appreciate the time and, more importantly, the effort to find common ground and tangible changes to improve patient engagement. We will continue to report on progress in this space.

CMMI MODELS

Haystack led both a pre-meeting and meeting with our member patient groups interested in the work of CMS' Innovation Center on the Cell and Gene Therapy Model and the Accelerated Approval Pathway model. The CMMI team was gracious and acknowledged some new ideas we raised as well as insights in areas they were already thinking about.

Our prep meeting, background document, and presentation deck meant our patient groups could not just share their stories, but relate those stories to the changes they wanted from CMS. Watch this space for follow-up on the meeting.

HR 6094

Haystack Project patient groups kicked off a series of meetings in January with Congressional offices on the House E&C and W&M committees, the Rare Disease Caucus, and supporters of the Safe Step and Prior Authorization bills. The number of groups taking an active role grows every day! Please reach out to us if you'd like to join the effort. Groups have been training each other and doing meetings together.

IRA AND RARE

Haystack conducted an extensive discussion with member groups about Congress' Orphan Exemption and CMS' interpretation of "Qualified Single Source Drug (QSSD)." Groups discussed the impact of each on rare, and the need to address one or both for the rare community.

We discussed NORD and ELF's leadership on the Orphan Cures Act and concluded Haystack should explore a complementary approach to (1) avoid duplication and (2) work on an approach that would address other important aspects of rare drug development. We discussed political feasibility, potential 'score,' legal interpretation, working with NORD/ELF, and more -- Really robust discussion!! The feedback was very positive from the groups, so Haystack will begin working on materials for a new approach ASAP! Ask us about this!

CMS AND RARE

Three Shout Outs!!! Thank you to:

1. CMS' Center for Medicare
2. CMS' Clinical and Quality Standards' Coverage and Analysis Group
3. The Administrator's Office!

All three held separate meetings with Haystack in February to discuss rare disease patient engagement and priorities.

RARE DISEASE WEEK AND HILL MEETINGS

Haystack responded to patient group calls to highlight H.R. 6094, the Protect Rare Act. Our member groups hosted a webinar for patients attending Hill meetings, handed out one- pagers and more!

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HOUSEKEEPING

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Do you have calendar invitations for our standing monthly calls?

- **3rd Tuesdays 1-2 pm ET** - Speaker Series – Invite includes required registration link.
- **3rd Wednesdays 1-2 pm ET** - Member calls with patient groups

Follow us on social media – [LinkedIn](#), [Facebook](#), and [Twitter](#).

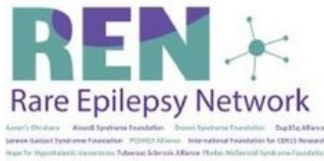
Any questions, contact Tiara.Logan@haystackproject.org

THANK YOU TO THE SPONSORS

Please check the website for the growing list of Haystack sponsors, whose support allows us to do what we do!



All Copays Count Coalition



Haystack Project is grateful to all of our Alliance Partners for lending their insights and perspectives, as well as for combining their efforts with ours to better serve the rare and ultra-rare communities.