

HAYSTACK PROJECT

The Voices of Rare & Ultra Rare

OCTOBER RECAP

OCTOBER 2023

DUES, DUES, DUES! Pay your 2023 dues [here](#). Please help us keep up all the good work you've come to count on us for!

PROTECT RARE ACT INTRODUCED

H.R. 6094 has been introduced!

PROTECT Act (previously the Access to Rare Indications)

H.R. 6094 WILL MAKE IT EASIER FOR RARE DISEASE PATIENTS TO GET THE TREATMENTS OUR DOCTORS SAY WE NEED. IT'S NOT ABOUT 'SPECIAL TREATMENT' FOR RARE DISEASES. IT'S ABOUT EQUAL FOOTING WITH THOSE LIVING WITH MORE COMMON CONDITIONS.

The **P**roviding **R**ealistic **O**ppportunity **t**o **E**qual and **C**omparable **T**reatments for Rare or PROTECT RARE is the old Access to Rare Indications Act. New name but the same great bill! Introduced by Rep. Dunn (R-FL) on the Energy and Commerce Committee, along with previous leaders, Rep. Matsui (D-CA), Kelly (R-PA), and Thompson(D-CA) to introduce Haystack's bill!!!



PROTECT RARE ACT

Providing Realistic Opportunity To Equal and Comparable Treatment for Rare



Find EVERYTHING you need to learn more and advocate for this bill is [HERE!](#)

SENATE UPDATE: A bipartisan group of Finance Committee members is awaiting CMS feedback before introduction... We have been waiting over 18 months to hear from them! PLEASE EMAIL US IF YOU CAN HELP WRITE TO CMS! Tiara.logan@haystackproject.org

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COMPENDIA



Rare Disease Compendia

Haystack Project discussed the lack of compendia in rare diseases and interest in developing one. Groups discussed the expertise needed for further the discussion and took assignments in recruiting interested clinicians to discuss feasibility. Haystack will host a half-day workshop to explore further.

HEART ACT

FDA MEETING

FDA has been completely unresponsive and has turned the meeting planning over to Duke-Margolis, who knew nothing of Haystack's work in passing the Heart Act. We are fighting to get on the agenda for this meeting. Please email us if you would like to help. Tiara.Logan@haystackproject.org. We need you!

GAO Study in Rare Diseases Underway

Haystack reviewed the upcoming GAO study currently underway.

We discussed the GAO's specific draft questions, and Haystack's input to GAO to get the questions refocused on the Heart Act's intent. Groups noted that GAO's initial questions seemed to sidestep the goal of the study by asking about problems groups were having with industry, when in fact they saw the focus as intended to be on challenges with FDA. As one group noted, the focus should be on the accountability of the government agency!

Groups outlined experiences to be shared with GAO and planned who could speak to variations of the rare disease experience at the FDA.

Groups covered: lack of enough patients, lack of meaningful endpoints, inevitable unblinding in small cohorts, lack of natural history studies, inevitable conflicts of interest, variations, and subtypes of rare diseases, lack of certainty/appropriateness of review division assignment, etc. are critical to conveying, as is lack of CPIM/PFDD/Listening Session effectiveness/responsiveness in spite of their expense. Eight Haystack groups will represent Haystack members at the GAO meeting at the end of November.

October Senate Aging Committee Hearing

Haystack provided and prepped a witness for this hearing to highlight the need for increased FDA flexibility in reviewing rare disease applications! We also discussed the pros AND CONS of Sen. Braun's Promising Pathways Act... and the request Haystack has pending with the Senator to improve the bill before supporting it. Ask us for details if you're interested! Groups asked for contact information in Sen. Braun's office to share our concerns. Tiara.logan@haystackproject.org

STATE ADVOCACY

AUGUST was a great time for the groups to undertake a “planning session” about their need/interest in state-level advocacy, and how Haystack might support them. The ‘experiment’ started with Haystack’s “HP50” Work Group, and the planning session made clear that the appetite for more is urgent.

SEPTEMBER 28 Haystack Project hosted a call with companies to discuss rare disease patient advocacy at the state level.

OCTOBER will bring patients and companies together to hear from Matt Salo, who recently retired from the National Association of State Medicaid Directors (NAMD). He'll discuss the gaps and opportunities in state-level advocacy in the rare community.

NOVEMBER will allow Haystack to consider specific goals, resource needs, and an implementation timeline to serve our patients. goals, resource needs, and an implementation timeline to serve our patients.

MISCELLANEOUS UPDATES



UPDATE

Haystack provided an update on...

- Copay Accumulators ruling, and the especially relevant case for rare diseases where there are rarely any generics. We reviewed the history and impact on rare diseases.
- Recent GAO and OIG reports re: Medicare Advantage plans’ prior authorization, denials, appeals, and related Congressional investigations.
- PBMs, the recent PhRMA report on shifting from rebates to more complicated fees, a recent GAO report on what patients vs plans paid for drugs, and pending legislation. We also reviewed the role of PBMs, etc.
- CMMI’s recent announcement on focusing on rare diseases and accelerated approval payments, shifting their focus from oncology once they found confirmatory trials were in fact happening in oncology. Rather than asking the FDA how oncology confirmatory trials are happening and sharing that experience with divisions handling rare diseases

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1:1 Listening Sessions have begun please email Tiara with availability after the Thanksgiving holiday. Look out for the email with Haystack Project's availability



WORK GROUPS

Updates were provided on the several work groups underway, including:

PROTECT Rare/Access to Rare Indications – This workgroup announced the upcoming introduction of the newly named PROTECT Rare Act in the House.

First Look Project – This workgroup is focused on phenotypes/"tell-tale signs" of rare diseases.

HP50 – This workgroup has a pilot underway to improve advocacy at state Medicaid departments – three states and two patient groups with recent/near approvals.

Do you have these on your calendar?

Please email Tiara Logan at tiara.logan@haystackproject.org.



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

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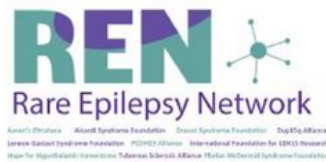
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Haystack Project is grateful to all of our Alliance Partners for lending their insights and perspectives, as well as for combining their efforts with ours to better serve the rare and ultra-rare communities.