# HAYSTACK PROJECT

## The Voices of Rare & Ultra Rare

AUGUST RECAP

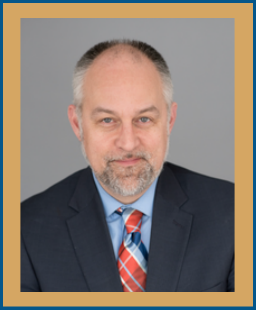
AUGUST 2023

**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!

## SPEAKER SERIES

On **September 19, 2023, at 1 p.m. ET**, Priscilla Parrilla will discuss Texas Medicaid and patient access to orphan drugs.

REGISTER HERE!



On **October 17, 2023, at 1 p.m. ET**, Matt Salo will discuss gaps in state-level patient engagement and how Haystack Project and its 140+ participating patient groups can work together to effectively address them.

REGISTER HERE!

On **November 21, 2023, at 1 p.m. ET**, Michael Bagel and Nissa Shaffi will discuss Medicare Advantage and Medicaid managed care plans – their evolution and policy priorities.

REGISTER HERE!

Who would you like to see Haystack host next on our Speaker Series? Send your suggestions HERE!

## 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 will host a call with companies to discuss rare disease patient advocacy at the state level. RSVP HERE!

**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.

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# IRA INFLATION REDUCTION ACT

**IRA's "10 drugs"** – We talked in detail about what to expect when CMS releases the list of 10 and reminded patients to let Haystack know if there are off-label uses of any of the drugs CMS lists.

Walked through **IRA's drug negotiation process**, including what constitutes alternative therapies for CMS, and their relevance. We fielded questions about combination treatments we often see in cancers and implications for alternative therapies. We noted CMS' focus on on-label and compendia to identify alternative therapies, which are less helpful resources for rare disease patients.

Alliance Partner – PIRC -- Haystack highlighted the Protecting Innovation in Rare Cancers – rebalancing the IRA. Rare cancer-specific effort… Haystack deferring rare cancer IRA work so as not to duplicate efforts.

# FEDERAL LEGISLATION

Even as we were implementing the HEART Act, the Galactosemia Foundation alerted Haystack about the lack of engagement they were facing with the FDA. Another "poster child" for the Heart Act. Haystack worked with the Foundation's leadership and patients to help them share their experience in ways that would be most impactful with Hill offices. Haystack scheduled and participated with them in 40+ Hill meetings helped draft/edit their follow-up messages, and more. As a result, over a dozen Hill offices reached out to the FDA, making sure the FDA knows Congress is watching and is not happy about what's happening yet again with a rare disease group. Heart Act leaders are pushing the agency to course correct here! REMINDER: PLS LET US KNOW ABOUT YOUR FDA ENGAGEMENT EXPERIENCES (GOOD AND BAD….)

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

**PROTECT Act (previously the Access to Rare Indications)**

**Name Change!** **P**roviding **R**ealistic **O**pportunity **t**o **E**qual **a**nd **C**omparable **T**reatments for Rare or PROTECT RARE is the new name for the last Access to Rare Indications Act. In this new Congress, the House bill will be 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!!!

**Patient Group "TO DO" List on PROTECT Rare focused on:**

a. Sign On letter thanking Reps Dunn, Matsui, Thompson, and Kelly.
b. These Representatives will list your group in their press release if you sign onto the letter in (a)…
c. Bill text to use with your own meetings with Representatives asking they cosponsor.
d. Ask other coalitions you belong to sign on to the letter in (a):
e. One-pager targeting Rare Disease Week to gain cosponsors and other fly-in events
f. Groups highlighted prior criticizing this bill at PAC events they are invited to attend.

**SENATE UPDATE**: A bipartisan group of Finance Committee members is awaiting CMS feedback before introduction. We've made progress in identifying the Finance

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# PBM REFORM AND SAFE STEP

**Ahead of ELF's Rare Across America** meetings, we took a deep dive into legislation on copay accumulators and Safe Step. We shared rare disease perspectives and answered questions to support their upcoming meetings. Interesting questions about documenting fail first, annual fail firsts, and the incentives plans and/or PBMs have to force repeated stepping policies.

We took the 'long road' on this topic, starting with their advent, their evolution, and the current situation. We continued down detours and curves to current legislation, why it is needed, the nuances between the bills, and what is missing from the bills. We covered vertical integration, delinking compensation from drug cost, data collection, fiduciary duty, and more – all in layman's terms with analogies to everyday situations to get everyone to a baseline understanding. Questions focused on several aspects of the bills. Some groups were hearing about PBMs for the first time, others appreciated the history and deeper dive. Interesting to consider unique rare/ultra-rare community….
And infographics to help drive home the discussion.

# 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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***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.***