

# HAYSTACK PROJECT

## The Voices of Rare & Ultra Rare

JULY RECAP

JULY 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!

## LEGISLATION



Even as we were implementing HEART Act, 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 messages, and more. As a result, over a dozen Hill offices reached out to FDA, making sure 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 (aka previously the Access to Rare Indications)**

**Name Change!** Providing Realistic Opportunity to Equal and Comparable Treatments for Rare or PROTECT RARE is the new name for the last year’s Access to Rare Indications Act. In this new Congress, the House bill will be introduced by Rep. Dunn (R-FL) on 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:**

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



**SENATE UPDATE:** Bipartisan group of Finance Committee members are awaiting CMS feedback before introduction. We’ve made progress in identifying Finance

JULY RECAP

JULY 2023

## Education of IRA Provisions continued this month...

1. Haystack Project highlighted the end of the 5% catastrophic out-of-pocket for patients starting in 2024. We also discussed the possible logistics around ‘smoothing’ – sharing specifics of the letter Haystack signed onto and the upcoming comments on the Explanation of Benefits (EOB). We discussed the estimated savings calculated by HHS and others for each of these provisions.
2. The conundrum for patients of ‘not supporting the IRA’ which is trying to reduce drug prices for patients is an interesting challenge. The discussion focused on how the OOP cap/smoothing provisions of the IRA “inoculate” patients from concerns about high prices, freeing them to turn their attention to protecting innovation.
3. Groups acknowledged that some of the rhetoric around ‘killing innovation’ might be overblown, but that in rare diseases, where 95% of diseases don’t yet have treatments, anything that can throw off the balance of incentives to innovate – on what is already a tightrope – is problematic. We are grateful for the enactment of the OOP cap/smoothing provisions for giving our community a chance to focus more on the rare disease innovation tightrope.
4. Haystack Project thanks CMS for revising IRA Negotiation Data Elements to include our requests for:
  - (i) expanding the concept of unmet needs related to patient subpopulations and to therapeutic alternatives when they exist, but are inadequate for subpopulations;
  - (ii) more specific questions focused on soliciting input on off-label uses (in add-on to unmet medical needs and health equity considerations);
  - (iii) elements re: side effects and adverse outcomes with alternative treatments; and
  - (iv) removing fraud and abuse penalty references in the certification statement so that patients, caregivers, and researchers aren’t discouraged from providing input on selected drugs.

Haystack Project remains concerned the overall negotiation framework will inevitably compromise efforts to align on value and a fair price for rare and ultra-rare treatments. We also reiterate that CMS’ interpretation of the statute will further deter ultra-rare innovation. [Click here](#) to read more.

## IRA Timeline Discussion focused on:

1. The Sept. 1 deadline for CMS announcement of the 10 selected drugs for negotiation.
2. The 10 selected drugs likely for more common conditions, the off-label use for unmet needs.
3. How the groups might work together and through Haystack to engage CMS in the upcoming window to discuss valuation and inform the agency’s negotiations.
4. The Maximum Fair Price (MFP) implications for on- and off-label uses, the Negotiation/Offer/Counter-offer process.

## Initial List of Selected Drugs for Price Negotiation

Groups discussed how patient groups can engage. We recirculated the predicted list of 10 after our member call. We discussed Haystack's resources to support member groups in engaging the CMS portal for input

### Patient Groups' "TO DO" List Discussion:

1. Orphan Drug Exemption education
2. CLL Society Rare Cancer Coalition
3. Upcoming Rare Disease Coalition
4. Review the List of 10 Initial Selected Drugs
5. Prepare for input to CMS on/off-label uses



### Other Topics"(yes, there's more going on than the IRA!)

**1.** We reviewed recent comment letter content, themes, and positions vis-à-vis rare impact (e.g., very real threats to rare diseases in CMS' efforts to improve patient access in Medicaid)

**2. Coverage with Evidence Development** – upcoming comments due, implications for rare disease coverage/access given how CMS reviews evidence in CED:

- a. Accelerated approval trials
- b. Single-arm trials
- c. Too fee patients
- d. Rare disease proposal on wastage in CMS' physician fee rule – exemption for extremely small population drugs; We covered the history/evolution of wastage policy – what it is, why it matters – and the policy changes proposed

**3. ICER** – We discussed:

- a. Recently proposed changes – e.g., reduction of QALY from \$150-200K to \$50K – in the context of ICER's work
- b. Measuring value from the perspective of getting patients back to perfect health when rare disease patients value halting disease progression
- c. High cost drugs a s rule because of small population size
- d. Lack of data on utility values like productivity, reduction of caregiver needs
- e. Implications of ICER weighing in on drug price negotiations at CMS
- f. Groups commented on the need to engage ICER again in spite of having to watch budgets of IRA-related work

## REGULATORY CONTINUED

**4. 340B Reform** -- Our 340B RFI comments raise a uniquely rare circumstance – Our innovators face very high 340B exposure given the potential concentration of rare disease experts in a limited set of “centers of excellence.” The percent of sales subject to 340B discounts can easily, and overwhelmingly, exceed standard expectations, and diverge quarter to quarter, reaching well above the already high 25% toward half or more of total sales. The rarer the disease, the more complete care, all or nearly all patients will be treated in teaching hospitals with 340B covered entity status. [Click here](#) to read more.



## 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](mailto:tiara.logan@haystackproject.org).

## HOUSEKEEPING

**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!

Do you have calendar invitations for our standing monthly calls?

**3rd Tuesdays 1-2 pm ET** - Speaker Series – Invite includes required registration link.

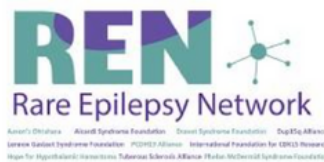
**3rd Wednesdays 1-2 pm ET** - Member calls with patient groups

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

Any questions, contact [Tiara.Logan@haystackproject.org](mailto: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!



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.