

HAYSTACK PROJECT

The Voices of Rare & Ultra Rare

SEPTEMBER RECAP / NEWSLETTER

SEPT 2022

SPEAKER SERIES

In September, we were joined by Lewis Fermaglich, MD, MHA, from the Office of Orphan Products Development at the FDA. He shared with us his office's process for designating an Orphan Product and the benefits of that designation.

Dr. Fermaglich clarified that the Office is *NOT* involved in any decisions re: use of the Accelerated Approval Pathway, the review of products, or determining assignment of the review decision. A recording of the discussion can be found [HERE](#), and some resources he provided for patients are also on our website.

Who would you like to see Haystack host on our next Speaker Series? Send ideas [HERE!](#)

LEGISLATIVE

DISCUSSION THIS MONTH CENTERED AROUND:

The Heart Art -- We continue to monitor the potential for an additional year-end package for the HEART Act, working with our lead sponsors.

We also continue to educate members about the need for the Access to Rare Indications Act (H.R. 6160), and how the IRA provision limiting the exemption to drug price negotiations to one orphan indication makes H.R. 6160 more necessary than ever.

We review a "Top 10" list of Inflation Reduction Act provisions and implications for rare patients, and it was a *lively discussion!*

Here are the [KFF slides](#) we used to facilitate the discussion, and for even more reading, here is the [Congressional Research Service report](#).

VALUE & ACCESS



Haystack is looking for patient groups whose patients use multi cancer detection blood tests. We are assessing experience with these test to consider value from a patient's perspective. Please email us [HERE](#).

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WORKGROUPS

Updates on our existing workgroups

This FDA- focused work group is holding its breath on HEART Act (“policy rider”) making it into a year end package since Congress passed a “clean UFA” without any policy riders.

We have also been trying to clarify to Sen. Kaine that the AA Pathway Council should NOT include FDA’s Office of Orphan Product Development (see Dr. Fermaglich's comments).

After our Sept call, CMS approved Oregon’s 1115 waiver. We didn’t get a chance to discuss, but the good news is CMS’ approval deleted the state’s request to exclude coverage of treatments approved under the Accelerated Approval pathway. [HERE](#) is the CMS letter for your late night reading pleasure....

HEART & ACCELERATED APPROVAL

led by Kara Berasi, CDG Cares and Dottie Caplan, Applied Therapeutics)
Meets second Mondays at 3pm ET



HP50 / STATE ACTIVITIES

led by Dean Suhr, MLD Foundation
Meets second Tuesdays @ 3pm ET



We are finalizing an exciting new pilot to work with some of our patient groups who have **significant #s of Medicaid patients** and have treatments getting approved and becoming available to patients **in the next 6 months**.

Email us if you’re (i) expecting a new treatment for your disease to launch early next year, and (ii) if you have a good number of Medicaid patients. See if you qualify to participate in what we hope will be the first of several pilots. Under the pilot, Haystack Project will support patient groups to learn more about how to advocate at the state level for Medicaid coverage and set up meetings for the groups among key policymakers. We will then share learnings with all Haystack Project groups.

NEW WORKGROUPS

Forming now!

NEW WORK GROUP
FORMING!

RARE CANCER ACCESS
BARRIERS

As we think ahead to next year, and our Heart Act work drawing to a close (hopefully!), we are considering a new work group dedicated to **Access Barriers Facing Rare Cancers**. The long-standing **Rare Cancer Policy Coalition** seeks to kick off a discussion of existing and upcoming access threats and a plan forward.

Patient groups, companies, and medical societies are all welcome to [email us](#) if interested in starting/joining a Rare Cancer Access Work Group.



Again, as we think ahead to next year, there will be a lot to do – responding to guidances and proposed rules, and educating policymakers.

Rare patients are often left behind when reimbursement policies are written. Here's our chance to be "in on the ground level" as new groundbreaking law is implemented. Patient groups, medical societies and companies are all welcome to [email us](#) if interested in starting/joining a new IRA Work Group.

NEW WORK GROUPS
FORMING!

IRA IMPLEMENTATION
IMPLICATIONS FOR RARE



REGULATORY

We discussed the Agency for Healthcare Quality Research's (AHRQ) very short comment period on recommendations to CMS on clinical trial design for Coverage with Evidence Development (CED). It was quite the deep dive into CED, CMS' use of the tool, and AHRQ's role.

We filed comments asking AHRQ to recommend CMS:

- Submit the clinical design to an IRB;
- Include an informed consent statement that clearly states the drug is FDA approved and commercially available;
- Ensure coverage continues outside of CED for patients who benefitted from the drug during the clinical trial.

We discussed the chilling effect CED has on uptake of a new treatment – and the clear and present danger to rare disease treatments. See our letter [HERE](#) for more details.

MEDICAID: We highlighted rare disease implications of CMS' new Proposed Rule seeking to amend Medicaid enrollment processes. We'll be reaching out to groups to get some feedback from their patients on some of the elements in the proposal.

Haystack submitted comments to CMS on the Physician Fee Schedule proposed rules, focusing on the needs of rare disease patients. Here's the [FINAL VERSION](#) we submitted in case you would like to review.



REGULATORY CONTINUED

Request for Information: CMS also released an RFI on four topics, each of which have implications for rare patients. We are soliciting insights/suggestions now, and plan to draft comments, due Nov. 4. Drop us a note [HERE](#) if you are interested. We already discussed patient' interest in including discussion of off-label use and lack of clinical guidelines and quality measures. Here is an outline of the RFI:

1. Impact of PHE flexibilities

- the impact of waivers and flexibilities/any that should be made permanent
- what was helpful & inadvertently harmful to patients
- any unintended consequences
- how to address any health disparities exacerbated by the PHE

2. Access to health care

- challenges patients face “understanding, choosing, accessing, paying for, or using health care services” -- including medication
- how beneficiaries access different types of care (e.g., primary care, acute care, long-term care, and care for mental health conditions and substance use)
- extent to which rural and underserved areas access care, and obstacles (e.g., access to broadband, culturally competent care, linguistic support, etc.)
- improvements needed in communication about health care

3. Workforce

- how to support the workforce
- how to address uneven distribution of health care workers
- strategies to help protect providers’ mental, physical, emotional health, combat burnout and improve providers’ access to support, either face-to-face or using digital health technology
- suggested improvements to payment policies and quality programs to help providers’ serve their communities

4. Health equity

- suggested program improvements to reduce health disparities
- social determinants of health
- what health care target areas should be high-priority
- how to reduce bias in technologies or clinical tools that rely on algorithms, including how to mitigate potential bias with clinical tools that have included race and ethnicity, sex/gender
- what policies should be implement to keep biases in check for future technologies and clinical tools

HOUSEKEEPING

*Please pay your 2022 dues today
and help us reach our 100% participation goal!*

Have you shared how many patients you have in each state? Simply knowing the number of patients in each state has helped Haystack work on your behalf to reach policymakers more effectively!

Learn more about why this is such a critical tool for all our patient groups and share your data! Email Kara [HERE](#).

AGGREGATE DATA

ARTICLES

There was a great article [HERE](#) from the Niemann-Pick (NPC) community on FDA's complicated approval process for rare disease treatments. What works in common diseases doesn't necessarily work in trials for rare diseases. We have two NPC members in Haystack Project.

Haystack Project continues to support the work of other organizations taking the lead on issues important to rare patients. We have evaluated and signed onto letters this month related to both copays and telemedicine beyond the PHE.

TELEMEDICINE & COPY ACCUMULATORS

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.

All Copays Count Coalition



AMERICAN
BRAIN
COALITION

