

HAYSTACK PROJECT

The Voices of Rare & Ultra Rare

FEBRUARY NEWSLETTER

FEB 2023

SPEAKER SERIES

On March 21, 2023, at 1 pm ET, **Lisa Goldstein** will discuss patient privacy. She helps develop policy, regulations, and guidance related to the HIPPA and privacy.

[REGISTER HERE](#) to join this session.

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



REGULATORY

On February 14th, CMS' Center for Innovation released three new models without much detail. We discussed two of them at length: 1. Cell & Gene Therapy Access Model and 2. the Accelerating Clinical Evidence Model.

The first would direct CMS to coordinate/administer a multi-state outcomes-based agreements with manufacturers to ease access for Medicaid beneficiaries. The second would have CMS develop payment options for drugs approved under the Accelerated Approval, presumably in an effort to encourage timely confirmatory trials. We then discussed the two day MedCAC meeting that CMS hosted to discuss National Coverage Decisions with Coverage with Evidence Development. All three topics were the subject of robust discussion.

Email tiara.logan@haystackproject.org to join this work group and learn more.

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REGULATORY CONTINUED



Haystack member patient groups expressed numerous concerns, including:

1. Each patient with even the same rare disease looks different, and outcomes are a moving target and can't be 'one size fits all.'
2. How can we measure outcomes when we don't have natural history information.
3. Progress/outcomes can sometimes take years; how can we measure outcomes against progress that could be driven by 'aging up.'
4. Every rare disease is a spectrum of issues, and the diagnostic journey is a good illustration of that; this makes outcomes-based anything very difficult.
5. How long it takes a patient to respond is only half the story; the other is how long will a patient continue responding. When does progression fade, could the patient go backward? What amount of time is enough?
6. Even within a fairly progressive disease, measuring outcomes gets tied up with measuring value.
7. What is the value of a drug working long enough for a second treatment option to get approved?

The group is considering a meeting with CMMI, CMS, and other policymakers. We will also use the discussion to draft comments and talking points as needed.

WE ARE MOVING

Our standing Thursday calls will **NO LONGER BE ON THURSDAY AFTERNOONS.**

We heard you and responded!

NEW this year – we've sent out calendar invites. You should have pre-populated invites on your calendar for the year.

3rd Tuesdays 1-2 pm ET -- Speaker Series calls -- Registration link is in the pre populated calendar invites.

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



HOUSEKEEPING



Pay your 2023 dues [here](#).

Dues paid early mean you won't receive email reminders for the rest of the year!

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

Haystack Project has posted 20+ times so far this year!

Our first quarter was filled with many sign-on and comment letters.

You can find finalized letters below:

[Haystack's Comment on CMS Proposal to Oppose for Rare Diseases](#)

[Haystack's Sign-On Letter on Proposed Rule on Patient Protection and Affordable Care Act](#)

[Haystack's Comments on CMS RFI related to EHB under the ACA](#)

[Haystack's Comments in Response to CMS' Medicare Part B Inflation Rebate Guidance](#)

[Haystack's Comments on CMS Advance Notice of Methodological Changes](#)

[Haystack's Comments on the PCORI's Draft Inventory of Attributes](#)



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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.

