



HAYSTACK PROJECT

The Voices of Rare & Ultra Rare

JANUARY RECAP

JAN 2024

DUES, DUES, DUES! Pay your 2024 dues [here](#). Please help us keep up all the good work you've come to count on us for!

ACCOMPLISHMENT

Haystack filed comments on Senator Cassidy's RFI. Read them [here](#).

FDA AND RARE

Haystack has had two follow-up meetings with the FDA since the December 14 Duke Margolis meeting on rare diseases. We appreciate the Agency's willingness to continue the conversation, and look forward to moving toward a concrete tangible change to improve rare disease patients' experiences! *We will continue to report on progress at our February monthly meeting.*

CMMI MODELS

Kay Scanlan, Consilium Strategies, will lead a meeting for Haystack Project patient groups with CMS' Innovation Center related to their Cell and Gene Therapy Model. Kay prepped a background document and outlined themes for members so patient groups could compare their experiences against those principles in preparation for the discussion with CMS. *We'll report on the meeting at our February monthly meeting.*

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HR 6094

Haystack Project patient groups kicked off a series of meetings in January with Representatives who sit on the House E&C and W&M committees, the Rare Disease Caucus, and supporters of the Safe Step bill. The number of groups taking an active role grows every day! Please reach out to us if you'd like to help. Groups have been training each other and doing meetings together.

IRA AND RARE

Haystack conducted an extensive discussion with member groups about Congress' Orphan Exemption and CMS' interpretation of "Qualified Single Source Drug (QSSD)." Groups discussed the impact of each on rare, and the need to address one or both for the rare community.

We discussed NORD and ELF's leadership on the Orphan Cures Act and concluded Haystack should explore a complementary approach with QSSD to (i) avoid duplicating efforts and (2) work on an approach that we believe addresses some important aspects and trends in orphan drug development. We discussed political feasibility, potential 'score,' legal interpretation, working with NORD/ELF, and more -- Really robust discussion!! The feedback was very positive from the groups, so Haystack will begin sharing material on a QSSD approach. Ask us about this! Kara.Berasi@haystackproject.org or Saira.Sultan@connect4strategies.com

RATING RARE DISEASE EVIDENCE

Haystack is hosting a meeting in March on stratifying and grading evidence in small populations. Groups discussed the expertise, timing, goals, and focus... Interested in learning more? Kara.Berasi@haystackproject.org

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RDLA RARE DISEASE WEEK

Haystack responded to patient group calls to highlight H.R. 6094, the Protect Rare Act. Information on our webinar for patients attending Hill meetings is [here](#).

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.

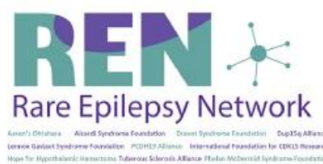
3rd Wednesdays 1-2 pm ET - Member calls with patient groups

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

Any questions, contact Tiara.Logan@haystackproject.org

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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.