HAYSTACK PROJECT
The Voices of Rare & Ultra Rare

Dues

We need to meet our goal of 100% participation, even if groups have to pay a reduced sum. Please click here today!

REGULATORY

Medicaid "Unwinding" Update – After Haystack staff provided context, the groups discussed unwinding efforts to date in April, data each state has provided in May, Kaiser data on expected insurance losses, and an Urban Institute report on expected shifts to Obamacare or private insurance. We shared and discussed approaches for how groups can educate their own patients, what to share with them, the CMS pilot navigators, etc.

Medicare Part D and MA Plans – We discussed a number of patient protections CMS finalized with respect to Medicare Advantage plans. One group asked about the 'authority' needed for clinical guidelines to be effective and whether groups can deploy their own KOLs to develop them (in response to CMS finalizing need for MA plans to use evidence- based UM criteria, including clinical guidelines). **Seeking speaker on this topic for our Speaker Series.**

NBPP Final Rule on ACA plans– We reviewed CMS' decisions in its ACO final rule, including on brand and generic tiering and patient protections like network adequacy, etc.

REGULATORY

IRA Negotiations, cont'd. – We reviewed CMS' plan to consider alternative treatments and unmet need to inform direct price negotiations. Groups shared their off-label uses of drugs for rare diseases from among the likely Top 10 drugs to be negotiated, and we considered how to support patients and CMS with surveys on value and unmet need for off label uses….as well as implications for reducing access to off label uses. Groups again raised the counter-intuitiveness of pushing against lower prices in the IRA negotiations, while appreciating concerns over future access, future innovation, etc. that have played out in other countries with direct government price setting.

PBM reform/Safe Step Act on the Hill – We updated groups on PBM legislation progressing on the Hill, and the provision related to remitting PBM rebates to the health plans (rather than to patients), and the lack of help this givesto patients. We also noted new progress on Safe Step legislation, how it would help patients with more common conditions, but not rare diseases since Access to Rare Indications bill is almost a 'precursor' bill that would have to pass first in order for rare disease patients to benefit. The groups were interested in Haystack talking with Safe Step cosponsors about this link.

WORK GROUPS

Updates were provided on the several work groups underway, including:

First Look Project – phenotypes/'tell-tale signs"of rare diseases is making some, albeit limited progress, while awaiting additional funding;

HP50 – one of the two groups in the pilot saw their drug approved in May, and Haystack is awaiting one-pagers, etc. from them to get started.

Access to Rare Indications – new 'calls to action' to find a Democratic Finance Senator to co-lead the bill. Our "FLY-INS" worked to secure two Republican Senators! One group hosted Haystack to do a webinar with their patients on the bill. **This webinar** is on our website.

If you'd like to join a work group or start a new one, please email **Tiara Logan** at **tiara.logan@haystackproject.org**.

LEGISLATION

Access to Rare Indications Act – pls watch/circulate to your patients **this webinar** designed to show patients, caregivers, doctors, etc. some EASY WAYS to add their voices in support of the bill to increase insurance coverage for rare disease treatments. If insurance doesn't pay for what we need, we'll likely not get what we need...

HEART Act – So we passed a law, but can we get it implemented correctly?!
Groups discussed their experiences -- both pro and con -- with previous FDA rare disease meetings they've attended. We talked about what would constitute a helpful meeting. Fill out **Google form/survey here** to help us improve the rare disease meetings FDA is required to host under the Heart Act. **Survey results will be kept confidential.** Haystack has already requested a discussion with FDA, and alerted our Hill champions that we may need their support. We are meeting mid-June with GAO and awaiting a response from the National Academy of Sciences. If you fill out the survey and attend the June 9 12-2pm ET meeting, your group will be considered for participating in these FDA, GAO, and NAS meetings (pls let us know if you're interested). See table below for goals of each meeting…

HEART ACT FDA MEETING	HEART ACT GAO REPORT (Government Accounting Office)	HEART ACT NAS REPORT (National Academy of Sciences…)
Haystack to request, attend meeting with FDA to discuss partnering on Heart Act meeting.	Haystack to convene all day roundtable, prepare patient groups and meet with GAO, followed by written submission.	Haystack to convene all day roundtable, prepare patient groups and meet with NAS, followed by written submission.
Not later than December 31, 2023, the FDA shall convene one or more public meetings to solicit input from stakeholders regarding approaches to increasing and improving engagement with: rare disease or condition patients, groups representing such patients, rare disease or condition experts, and experts on small population studies in order to improve the understanding with respect to rare diseases or conditions of— • patient burden; • treatment options; and • side effects of treatments including understanding the risks of side effects relative to the health status of the patient and the progression of the disease or condition. FDA shall establish a public docket to receive written comments related to the approaches addressed during public meeting(s) for 60 days following each meeting. Not later than 180 days after each public meeting, FDA shall develop and publish on the FDA website the approaches discussed at the public meeting; and any related recommendations.	In 18 months, submit report to Congress: (1) assessing FDA policies, practices, and programs with respect to review of applications for approval of rare disease drugs/biologicals ("drugs"); (2) describe FDA activities dedicated to the development/review of rare disease drugs, describe challenges with developing and obtaining approval, e.g., challenges related to designing/conducting trials, recruitment and enrollment, study endpoints, and ensuring data quality, assessing the benefit-risk profile of drugs and biological products intended to treat rare diseases, and meeting requirements for approval; (3) assess effectiveness of FDA policies and practices related to review of drug applications, including— (i) initiatives to support the development/review of drugs, incl. any related to regulatory science, trial design, statistical analysis, etc.; (ii) consideration of relevant patient-focused drug development data and information, incl. patient experience data and the views of patients; (iii) training and other efforts to ensure FDA personnel expertise regarding review of drug applications; and (iv) consultation and engagement with stakeholders and external experts; (4) assess extent FDA applies policies and practices described in subparagraph (3) consistently across review divisions, and the factors that influence the extent to which such application is consistent; and, (5) recommendations to address challenges and deficiencies identified, incl. recommendations to improve FDA's effectiveness, consistency, and coordination of policies, practices, and programs;	STUDY ON EUROPEAN UNION SAFETY AND EFFICACY REVIEWS OF DRUGS FOR RARE DISEASES AND CONDITIONS. — The Secretary shall enter into a contract with the National Academies of Sciences, Engineering, and Medicine (NAS) to conduct a study on processes for evaluating the safety and efficacy of rare disease drugs in the US and EU, incl.— (a) flexibilities, authorities, or mechanisms available to US and EU regulators specific to rare diseases or conditions; (b) the consideration and use of supplemental data submitted during review processes in the US and EU, incl. data associated with open label extension studies and expanded access programs specific to rare diseases or conditions; (c) an assessment of collaborative efforts between US and EU related to— (i) product development programs under review; (ii) policies under development and those recently issued; and (iii) scientific information related to product development or regulation; and (d) recommendations for how Congress can support collaborative efforts described in subparagraph (C). The contract with NAS (1) shall provide for consultation with relevant stakeholders, incl. representatives from FDA, EMA, patients with rare diseases and patient groups that represent patients with rare diseases and have international patient outreach. The contract with NAS shall provide for, not later than 2 years after the date of entering into such contract, the completion of the study and the submission of a report on the results of such study to Congress, and to the public.

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!

You should 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.