



HAYSTACK PROJECT

The Voices of Rare & Ultra Rare

APRIL NEWSLETTER

APR 2023

IRA CONTINUED

Discussion continued this month about CMS' proposals and Haystack Project's comments to CMS re:

Orphan Exclusion and implications for Access to Rare Indications Act. We outlined the distinction between 'indications' versus 'designations.' We also considered CMS' proposal to count 'active ingredient' as one "qualifying single source drug" instead of each NDA/BLA. The conversation and questions helped clear up (i) confusion about the implications for negotiations leading to lower prices, (ii) the impact on rare disease patients vis-à-vis the cap and smoothing in Part D 'inoculating' patients, and (iii) continued innovation in rare diseases. Questions came up about indication pricing, different routes of administration for different indications, the counter-intuitive need to protect pricing, etc. **Of note: We brainstormed ways Haystack Project could help our patient groups prepare and navigate the treatment-specific discussions with CMS, "staff" the groups in outlining alternative treatments, the value of treatments, etc.**

Implications for how CMS proposes to define 'qualifying single source drug' proved a complex area as groups understood the impact on initial and future indications or applications. Groups had good examples of their ultra-rare disease being 'skipped over' for a registration trial in favor of larger rare indications. Groups discussed how best to message one's community about protecting innovation in parallel to off-label use, changes in clinical development plans, and more.

The groups were eloquent in their thoughts about the politics around IRA and the simplistic opposition to industry, and how that lands with patient groups counting on for the next innovation. Several groups discussed the dichotomy of sounding like they are defending the industry versus being vested in the next innovation.

Haystack will work on meeting with CMS as our next step.

APRIL NEWSLETTER

APR 2023

LEGISLATION



We reported great news on our progress on the Access to Rare Indications Act! This legislation puts rare disease patients on a level playing field with patients living with more common conditions by broadening the definition of ‘medical necessity’ for rare patients so that insurance will consider covering the treatments our patients need.

We announced a new Republican E&C Health Subcommittee member who has signed up to complete the foursome to reintroduce H.R. 6160!!

We announced two Republican Senators have agreed to introduce a Senate version of HR 6160. The “Fly-Ins” worked!

We now need at least one Democratic Senator to co-lead the bill – and groups asked for more aggressive Calls to Action, a webinar, etc.



WORK GROUPS

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

First Look Project – phenotypes/“tell-tale signs” of rare diseases

HP50 – and the pilot open for patient groups likely to see an approval of a new treatment in the coming months

Access to Rare Indications – the need for new ‘calls to action’ to find a Democratic Senator to co-lead the bill. Our “FLY-INS” worked! We have secured two Republican Senators! We are also Patients asked for a webinar on this topic.

NEW WORKGROUP pending on groups expressing interest – **Rare Cancer**

NEW WORKGROUP pending on groups expressing interest – Recent CMS proposal negatively impacting rare diseases – **NCDs/CEDs, and CMMI**

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

HOUSEKEEPING

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

Pay your 2023 dues [here](#). Please help us keep up all the good work you've come to count on us for!

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

Any questions, contact 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!

The central graphic displays a grid of logos for various sponsors. At the top, it reads "THANK YOU TO THE SPONSORS" and "Please check the website for the growing list of Haystack sponsors, whose support allows us to do what we do!". The logos include: AKCEA (An Ionis Company | Therapeutics), ALEXION, AMGEN, APPLIED THERAPEUTICS, AstraZeneca, Aurinia, Biogen, BIOMARIN, bluebirdbio, GRAIL, illumina, IONIS, IPSEN (Innovation for patient care), Mitsubishi Tanabe Pharma, SANOFI, ucb, ultragenyx, ZOGENIX, All Copays Count Coalition, AMERICAN BRAIN COALITION, ata (Health. Virtually. Everywhere.), BLACK WOMEN'S HEALTH IMPERATIVE, Global Genes (RARE Foundation Alliance), GPI (Global Policy Institute), HAB (Habilitation Benefits Coalition), PARTNERSHIP FOR INNOVATION AND EMPOWERMENT, RARE X, REN (Rare Epilepsy Network), and ALLIANCE for CONNECTED CARE.



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.