

# HAYSTACK PROJECT

## The Voices of Rare & Ultra Rare

MARCH NEWSLETTER

MAR 2023

## Victoria Blatter Joins Haystack Project's Board of Directors

Haystack Project is pleased to announce the appointment of Victoria Blatter, MPH to the organization's board of directors.

Haystack Project announced on March 30, 2023, that Victoria Blatter, MPH will join Haystack Project's board of directors. Blatter brings over 20 years of leadership in industry and government, legislative expertise, and a wide breadth of knowledge around patient access to care.

"Victoria is a dynamic thought leader," said Chevese Turner, chief executive officer of Haystack Project. "Her intelligence, wisdom, and broad network will make her a valuable member of an already talented board. I am grateful for her willingness to share her experience and knowledge which will ultimately benefit patients in the rare and ultra-rare community."

Blatter is the recently retired Senior Vice President of, Global Government Affairs for Amgen where she was responsible for federal and state legislative issues and managing relationships with U.S. agencies, legislatures, and governmental administrations. She was also responsible for international lobbying and diplomacy in Washington, D.C. Before joining Amgen, she worked at Merck & Co., Inc. as a Vice President of U.S. Policy and Federal Government Relations and previously served as professional staff for the U.S. Senate Special Committee on Aging and as minority staff director for the U.S. House Select Committee on Aging's Subcommittee on Retirement Income and Employment. She received a bachelor of arts from Binghamton University and a master's degree in public administration from the Maxwell School of Citizenship and Public Affairs at Syracuse University.



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# WORK GROUPS

Get involved! **Reminder on each workgroup, meeting times, and missions. Contact [Tiara.Logan@haystackproject.org](mailto:Tiara.Logan@haystackproject.org) to request meeting invites for any/all work groups**

## Ad Hoc Heart Work Group

1st Tuesday at 3 pm ET (1 hour)

**Focus: implementation of Haystack's HeartAct (Haystack bill passed in 2022)**

## HR6160/Medical Necessity Work Group

1st Wednesday at 4:30 pm ET (1 hour)

**Advocates for legislation to make off-label treatments easier to get covered and paid for by insurance for rare disease patients (see above).**

## HP50 Work Group

3rd Tuesday 3 pm ET (1 hour)

**Focus: capacity building for patient groups interested in increasing their state-level advocacy as it relates to insurance delays and denials, access barriers, etc. (Ask about the pilot underway for patient groups nearing treatment approval.)**

## First Look Work Group

2nd Wednesday at 12 pm ET (1 hour)

**Working on conceiving a unique path to shorten the diagnostic journey for conditions with 'tell-tale' signs/symptoms. Initial positive progress; further funding needed**

## Coverage & Payment (CMS/CMMI)

2nd Tuesday at 1 pm ET (1 hour)

**Just Starting! Focus: Response to recent CMS and CMMI proposals that fail to consider rare disease circumstances and will, if implemented, significantly reduce/deny access to treatments our doctors say their rare patients need.**

## Rare Cancers

4th Tuesday at 1 pm ET (1 hour)

**TBD. Let us know if you would like to participate.**

**Consider new Congressional/Biden Administration proposals in cancer to ensure rare cancer focus is included**



## DEFINING ULTRA RARE

Discussion regarding value and nuances of recent stakeholder interest in defining rare versus ultra rare diseases. Group discussion and input on value of a definition, what special incentives and benefits might inure to those who study treatments for smaller diseases and a 2,000 vs 20,000 number. Pink Sheet article [here](#). Robust discussion!

# IRA NUANCES

Haystack participants have been discussing IRA and its implications for rare disease development and commercialization for several months. The discussion continued this month:

1. **Inflation Rebates.** The groups discussed IRA penalties for price increases over inflation. Specifically, the impact of inflation penalties in shortage or in the context of value based arrangements.
  - a. Groups raised instances of companies reducing or withdrawing inventory, which is concerning re: shortage
  - b. Implications of ‘artificial shortages’ created by companies
2. **Drugs Subject to Negotiation, Timelines**
  - a. Explain context
3. **Small Biotech Exception**
  - a. Implications for rare – patient groups highlighted that a lot of the innovation in rare come from really small companies
  - b. Discussion informed Haystack comments on small biotech exception
4. **Orphan Exemption**
  - a. Mechanics, context discussed
  - b. History of our advocacy in opposing the exemption
  - c. Implications for our bill, HR 6160
  - d. Implications for replacing our current science/clinical development calculation with a commercialization calculation – the change in paradigm would damage rare innovation
  - e. Advocating for CMS to use its discretion in favor of rare diseases
  - f. Patient groups’ discussion included helping nuance our comments to include that many rare diseases include multiple subtypes, why multiple NDCs are really complicated from a pharmacy perspective.
5. **Other “Mega Guidance” Provisions to come**
  - a. Data elements – value of the treatment, other drugs that treat the same condition
  - b. These elements could broaden CMS’ thinking
  - c. The need for options for patients



# LEGISLATION

1. Participants discussed HR6160, including efforts to reintroduce the bill introduced this year, progress on policy implications, and how to join relevant work group to engage more actively.
2. Robust discussion on recent legislation on QALYs and implications for rare; focused on reasons to ‘value’ a treatment, by whom, for whom, the implications to access and the need for patient participation, shortcomings of the QALY, but also other similar tools. Folks asked for [link](#) to the ICER work (webinar and slides) from our website as background.

# 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](mailto: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!*



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

