Saira Sultan
Policy consultant
Saira.Sultan@haystackproject.org

Haystack Project's Mission

Haystack Project is a non-profit coalition of **140+ ultra-rare patient organizations** that come together to present a coordinated, focused and unified effort to educate about the need to recognize and address **systemic access barriers.** The participants are committed to working together collaboratively to ensure patient access to novel treatments, specialists, and diagnostics for extremely rare conditions.

Haystack Project participating groups, educational initiatives, comment letters, etc. are all on our website at ***haystackproject.org***

HAYSTACK PROJECT
2023 Policy Work

- **HEART Act Implementation**
- PROTECT Rare Act H.R.6094
  Matsui, Dunn, Thompson and Kelly
- State-level Access Barriers/Delays
- Value Assessments/ICER re: RARE

PRIORITIES

NASEM Study

- *Flexibilities, authorities, or mechanisms available to regulators in the United States and the European Union applicable to rare diseases or conditions;*
- Consideration and use of supplemental data submitted during review processes in the US and EU, including data associated with open label extension studies and expanded access programs specific to rare diseases or conditions;
- Assessment of collaborative efforts between United States and European Union regulators related to: product development programs under review; policies under dev./recently issued; scientific information related to product dev./regs

Jim.Caro@haystackproject.org

NASEM Study

Flexibilities, authorities, or mechanisms available to regulators in the United States and the European Union applicable to rare diseases or conditions;

- ***ARE THEY USING THEM? AND HOW? DOCUMENTING THEM? TO WHOM? WHEN?***
- ***…COMPARISON***
- ***ADVISORY COMMITTEES***
- ***PFDDs/Listening Sessions***
- ***REVIEW THE REVIEWERS***
- ***DIVISION ASSiGNMENT***
- ***POST MARKET COMMITMENTS/AA APPROVALS***

Consideration and use of supplemental data submitted during review processes in the US and EU, including data associated with open label extension studies and expanded access programs specific to rare diseases or conditions;

- ***AND NOT SUBMITTED;***
- ***COMPOSITE ENDPOINTS;***

Assessment of collaborative efforts between United States and European Union regulators related to: product development programs under review; policies under dev./recently issued; scientific information related to product dev./regs

Saira Sultan
Policy consultant
Saira.Sultan@haystackproject.org