ispe

REAL-WORLD EVIDENCE TASK FORCE

January 2022 ISSUE

OFFICIAL RESPONSE TO FDA GUIDANCE

On September 29, 2021, the U.S. Food and Drug Administration (FDA) released a draft of Guidance for Industry on use of Electronic Health Records (EHR) and Medical Claims to support Regulatory Decisions on Drug and Biological Products. The RWE and Regulatory Decisions working group constructed and coordinated ISPE's official response, which is posted on the ISPE website.

BIAS FOR AI – ISPE RWE UPDATE

On behalf of the RWE and Real-World Data Sources subgroup, Kenneth Man and Nancy Lin took the lead in developing ISPE's official response to a public consultation of a National Institute of Standards and Technology (NIST) publication, A Proposal for Identifying and Managing Bias in Artificial Intelligence (NIST Special Publication 1270). The comments are posted on ISPE's website.

INTERESTED IN JOINING?

Information about the RWE Taskforce, subgroups, leadership and members can be found at the RWE Task Force page on the ISPE website. Do you want to get actively involved, feel free to reach out to the specific subgroup leads.

CONTENTS

BOARD MEETING UPDATES

The RWE TF provided updates on its activities during the board meeting last November. Besides providing an update on ongoing activities and future plans, the RWE TF draft Strategic Plan Deliverable on Outreach Prioritization List was presented. The list, together with the draft outreach process, will be shared with the RWE TF members for commenting. Following that it will be open for comments by members of the Strategic Planning Committee and members from the Bylaws Policies and Procedures Committee. For those interested in receiving a copy of the ISPE RWE TF board report, please feel free to reach out to one of the co-chairs.

Action (Per Strategic Plan)	Deliverable	Status
Create ISPE-approved statement of RWE on 1-3 slides	Create ISPE approved statement of RWE	Completed
	Create ISPE approved 1-3 slides regarding ISPE and RWE, uploaded to the ISPE RWE TF members exchange community	Completed
Develop publications on RWE content w/ ISPE endorsement	Develop publications on RWE content with ISPE endorsement	Ongoing
Develop process for strategic collaborations with groups	Develop and propose process to Board to guide strategic collaboration with external groups (collaborate with SPC)	Draft completed Next steps: RWE TF, SPC & BPPC review
	Develop and propose list of potential collaborators/partner organizations and propose to Board (collaborate with SPC)	Draft completed Next steps: SPC & BPPC review
Establish ISPE presence (lead role) at "big data science" conferences	Develop and propose process for identification of "big data science" conferences to target for ISPE presence	Ongoing
Explore ways to raise awareness of ISPE methods in regulatory agencies	Strengthen relationships of ISPE and regulatory authorities, particularly on initiatives of mutual interest	Draft recommendations completed Next steps: RWE TF, SPC & BPPC review

SUBGROUP UPDATES

FOR A LIST OF SUBGROUP MEMBERS VISIT THE RWE TASK FORCE WEBPAGE

SPOTLIGHT: RWE AND STATISTICAL METHODS

- The ISPE RWE Task Force Statistical Methods Subgroup focused on completed short entries (2-10 pages in length) describing a total of 11 statistical methods, which we categorized as established, emerging, or cutting-edge. The idea was to create documents with enough information at hand to help practitioners make an appropriate choice of method, understand key strengths and weaknesses, but not to reinvent the wheel by writing a paper or textbook chapter on the approach. We hope that this can be a good reference to ISPE members looking to find the right method for an RWE problem they're looking to solve.
  Each Entry Covers:
  - What problem the method is designed to solve
  - A short description as background: Speaker 3 - Monica D'Arcy, National Cancer Institute, *Cancer* Notes where the method is most appropriately or commonly used
  - Canonical citations should the method be employed
  - Some key examples of the usage
  - Issues the design and/or method can have
  - Key criticisms
  - Key implementations

SPOTLIGHT: RWE AND REGULATORY DECISIONS SUBGROUP

- The RWE and regulatory decisions workgroup compiled and submitted ISPE-endorsed comments on the draft FDA Guidance for Industry (issued end Sept 2021) on use of EHR and claims for regulatory decisions. The workgroup is evaluating whether other draft guidances issued by FDA warrant official ISPE comments. Smaller subgroups continue to meet on an ongoing basis to develop four manuscripts; other manuscripts are on hold due to lack of a lead author.

ISPE RWE MANUSCRIPT DEVELOPMENT SUBGROUP

- Newly published manuscripts
  - Real-world evidence for assessing treatment effectiveness and safety in pediatric populations
    - (Journal of Pediatrics; DOI:10.1016/j.jpeds.2021.06.062)
  - Real-world evidence to support regulatory decision making: New or expanded medical product indications
    - (Pharmacoepidemiology and Drug Safety; DOI: 10.1002/pds.5222)
  - A framework for extension studies using real-world data to examine long-term safety and effectiveness
    - (Therapeutic Innovation & Regulatory Science; DOI: 10.1007/s43441-021-00322-8)

RWE AND ISPE-ISPOR SUBGROUP

- RWE Transparency and collaboration with ISPOR:
  - We are currently working on use cases/examples to pressure-test a draft harmonized protocol template to facilitate transparency and reproducibility
  - An ISPOR-ISPE-Duke Margolis-NPC collaboration is working on an alternative study registration site designed specifically for RWE (especially hypothesis evaluating treatment effect studies). It would be wonderful if ISPE members could explore the registration site, test it out and provide feedback. The site is currently in a "soft launch" phase. https://osf.io/registries/rwe/discover.

To view subgroup member lists, visit the RWE Task Force page on the ISPE website.

View our past July 2021 Newsletter