ispe

REAL-WORLD EVIDENCE TASK FORCE

July 2021 ISSUE

WEBINAR & WORKSHOP UPDATES

- There are four webinars planned from the RWE TF in collaboration with the Education committee (three from the Manuscript subgroup, one from the Cancer subgroup). Topics include:
  - "RWE to support regulatory decisions of new or expanded indications" (Completed on June 10, 2021: 12-1pm US EDT);
  - "Pragmatic trial extensions using RWE" (September 14, 2021: 12-1pm US EDT);
  - "Methods to mitigate biases in the post-COVID19 era (Date/time to-be-confirmed)",
  - "Real World Data Sources and Advanced Methods in Oncology"; (July 21, 2021: 12-1pm US EDT)
- Feedback was very positive from the 1st webinar by Jessie Franklin with 240 participants.
- The COVID-19 subgroup is planning a webinar on COVID-19 in pediatrics, as a follow up of the ICPE workshop in August. Have received support and suggestions from EC members and will discuss the best way to move forward.

BIAS FOR AI – ISPE RWE UPDATE

- Kenneth Man and Nancy Lin took the lead in commenting on the "Bias for AI" on behalf of ISPE
  - The draft comments (available at: https://docs.google.com/document/d/1v5CWW3c3-eZUj6hN5y3wY6jbOD8FJD0wLON98VIVUPU/edit?usp=sharing) are circulating for comments from ISPE members per ISPE process.

CONTENTS

COVID-19 REAL-WORLD DATA TEXTBOOK UPDATE

We are pleased to announce a collaboration between ISPE, Reagan-Udall Foundation for the FDA (FDA Foundation), and Friends of Cancer Research (Friends), as editors and chapter leads for a new online "living" textbook on RWD/RWE with a focus on COVID-19.

- Proposed textbook sections include:
  - Introduction
  - Chapter 1: Overview of RWD sources
  - Chapter 2: Methods for RWE generation
  - Chapter 3: Example COVID-19 RWE studies
  - Chapter 4: Major RWD initiatives for COVID-19
  - Chapter 5: Parallel analysis and the COVID-19 Evidence Accelerator
  - Chapter 6: Dissemination
- As a society comprising international experts in RWD and methods for generating RWE, ISPE is uniquely positioned to join the FDA Foundation and Friends in creation of the textbook and to ensure that design and not data-driven approaches are employed, especially in situations where rapidly generated evidence is needed to guide management of a global crisis.
- The ISPE Board has approved ISPE as co-sponsor of this textbook with the following ISPE members on the Editorial Board:
  - Josh Gagne, ISPE/Johnson & Johnson
  - Christopher Rentsch, ISPE/LSHTM/Yale
  - Donna Rivera, ISPE
  - Almut Winterstein, ISPE/University of Florida
- RWE Task Force subgroups are also leading the development of two Chapters: Chapter 2 (to be led by the Methods Training subgroup) and Chapter 4 (to be led by the COVID-19 subgroup)

SUBGROUP UPDATES

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

SPOTLIGHT: ISPE RWE ONCOLOGY SUBGROUP

- Please join the webinar organized by Benjamin Bates on *Real World Data Sources and Advanced Methods in Oncology*"; 7/21/ 21, noon
  - Speaker 1: Adam Reich, IQVIA, *Introduction to the UK National Cancer Registration and Analysis Service*
  - Speaker 2: Christen Gray, AstraZeneca, *Framework for External Comparator Study Designs in Real World Oncology Data*
  - Speaker 3: Monica D'Arcy, National Cancer Institute, *Cancer Algorithms in RWD: Detection Bias, Active Comparators, and Cancer Survival*
- External Collaborations
  - The subgroup is working with the Board and EC to outline the processes for external collaboration to develop a joint workshop with ASCO and ESMO in Oncology RWD and RWE.
- One workshop on External Controls in Oncology is accepted by ICPE

SPOTLIGHT: ISPE RWE COVID-19 SUBGROUP

- Several Scientific publications ongoing or under discussions
  - Knowledge gaps related to COVID-19 infection (COVID-19 effects on healthcare systems, other health issues) from the current literature
  - Knowledge gaps related to COVID-19 infection (effects of vaccines and therapies) from the current literature
  - Potential key knowledge gaps related to COVID-19 infection (Methods to study COVID-19 and effects of therapies/vaccines) from the current literature
  - Potential key knowledge gaps related to COVID-19 infection (Data sources to study COVID-19 and effects of therapies/vaccines) from the current literature
  - Potential key knowledge gaps related to COVID-19 infection (COVID-19 characterization, short, long term outcomes) from the current literature
- ISPE conferences related activities
  - Abstracts for 2 symposia for 2021 annual ICPE meeting on "RW data sources" and "COVID-19 in children" were accepted
  - Moderation of plenary session at ISPE Mid-Year meeting on COVID-19 (Session co-led by Ann McMahon, Christopher Rentsch)

SPOTLIGHT: RWE AND REGULATORY DECISIONS SUBGROUP

- The following manuscripts are underway or planned and should transition by ICPE 2022
  - Framework for data quality and representativeness for regulatory and HTA decision-making – *2 meetings held. On hold due to Karolina (1st author) being on medical leave. Sundell, Jacquot, Mo, Soriano-Gabbarro – need new 1st author*
  - RWE endpoints for regulatory and HTA purposes; what are attributes of endpoints that are acceptable for regulatory decisions? What level of validation is needed? – *one meeting held and another being planned; Jacquot, Sundell, Garzarian, Chang, Girman, Iqbal (need 1st author)*
  - RWE to support regulatory decisions in vaccines – *Patricia Saddier leading*
  - Pragmatic randomized trials and approaches for regulatory decisions – *Girman, Ritchey, Leclerc – To get underway by ICPE All Access*
  - Recommendations for international and scientific requirements for pharmacoepi studies submitted to regulatory agencies to promote more effective use of RWD – *Need 1st author*
  - Assessing whether a data source is fit-for-purpose for specific regulatory questions (Girman, Ritchey) – Follow-up to *Ther Innov Regul Sci* 2020; DOI 10.1007/s43441-020-00139-x – *should start this summer*
  - What makes results of a study "believable"? – *Girman/Leclerc, Assimon, Brodovicz, Urushihara, Chang – symposium held at ISPOR, abstract accepted for ICPE; planning to start paper before ICPE*
  - Real-world evidence needs for HTA bodies – *two papers planned, Hongbo leading effort; Jaska, Garzarian, Girman and Rob Epstein – discussion underway for planning to apply for manuscript support*
  - Patient-focused regulatory decision-making: pathways and obstacles to realization – *Cathy Anne Pinto leading; 1st meeting held to discuss scope*

RWE AND STATISTICAL METHODS

- As a reminder, the subgroup is working towards a comprehensive document for use by the ISPE membership.
- The subgroup has prioritized 22 established and emerging statistical methods to document, and will have draft "entries" of all methods by end of September 2021. The entry topics can be found here.
- Currently, the group is ~30% towards its September goal.
- In September, the group will reconvene to assign reviewers and discuss format finalization.

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)
- Previously published manuscripts
  - Considerations in characterizing real-world data relevance and quality for regulatory purposes: A commentary
    - (Pharmacoepidemiology and Drug Safety; DOI: 10.1002/pds.4697)
  - Use of real-world evidence in regulatory decisions for rare diseases in the United States-Current status and future directions
    - (Pharmacoepidemiology and Drug Safety; DOI: 10.1002/pds.4962)
  - Real-world evidence to support regulatory decision-making for medicines: Considerations for external control arms
    - (Pharmacoepidemiology and Drug Safety; DOI: 10.1002/pds.4975)
  - The Certainty Framework for assessing real-world data in studies of medical product safety and effectiveness
    - (Clinical Pharmacology and Therapeutics; DOI: 10.1002/cpt.2045)
  - Methods for external control groups for single arm trials or long-term uncontrolled extensions to randomized clinical trials
    - (Pharmacoepidemiology and Drug Safety; DOI: 10.1002/pds.5141)
- Core and extended members are now working on several wave #3 manuscripts for submission for ISPE endorsement in 2021

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 March 2021 Newsletter