**Minutes ISPE RWE taskforce: RWE and Regulatory Decisions Core team**

February 17, 2022

Attendees:

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
|  | **First name** | **Lastname** |  | **Firstname** | **Lastname** |
|  | Onyinye | Akunne |  | Emmanuelle | Jacquot |
|  | Karolina | Andersson Sundell | X | Jacinthe | Leclerc |
|  | Magdalene | Assimon | X | Jingping | Mo |
|  | Stella | Blackburn |  | Daniel | Morales |
| X | Kimberly | Brodovicz |  | Kenneth | Quinto |
|  | Shirley | Wang |  | Montse | Soriano Gabarro |
|  | Marie Louise (Marieke) | De Bruin |  | Hisashi | Urushihara |
|  | Helga | Gardarsdottir | X | Cathy Anne | Pinto |
|  | Madlen | Gazarian | X | Cynthia (Cindy) | Girman |
|  | Steven | Chang |  | Esther | Zhou |
|  | Ann | McMahon | X | Amy | Winter |
|  | Hongbo | Yuan |  | Meredith | Smith |
|  | O’Mareen | Spence |  | John | Concato |
| X | Sigal | Kaplan |  |  |  |

**AGENDA:**

1. Review FDA guidance documents released Nov and Dec with annotated remarks to decide whether to submit formal comments
2. Update on transitioning workgroup back into ISPE infrastructure
3. Manuscript updates

This meeting was called to order at 8:37 am ET by Cynthia Girman

* Meeting opened discussing the FDA Guidance documents and any additional comments
  + Most of comments were repetitive with those submitted in Nov on Use of EHR and Claims for Regulatory Decisions
  + Team agreed that the best path forward was to submit comments to the Duke-Margolis liaison team at Montse’s suggestion, and the PhRMA group at Kim’s suggestion, allowing them to incorporate into their own comments
  + Cindy to send the annotated guidance documents to these groups
* Kim Brodovicz and Cindy gave update on transitioning workgroup back into ISPE infrastructure
  + Most of remaining work of group is on manuscripts and responding to FDA or other regulatory guidances
  + Publications & Policy committee is a natural transition
  + Meeting next week with RWE Task Force co-leads and Chair of P&P Committee

*Manuscript Updates*

* What Makes RWE Believable – in progress; meeting next week to discuss; Cindy, Kim, Jacinthe, Magdalene
* Patient Experience Data for Regulatory Purposes – Lead Cathy Ann Pinto
  + Survey to be deployed in 2nd Quarter
  + Moving along with a lot of progress
* Many papers in limbo
  + If interested in leading one, please reach out to Cindy (please see table below)

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| Framework for data quality and representativeness for regulatory and HTA decision-making | Emmanuelle Jacquot; Karolina Andersson Sundell; Jingping Mo; Montse Soriano-Gabbarro  ***Need new lead*** |
| RWE endpoints for regulatory and HTA purposes; what are attributes of endpoints that are acceptable for regulatory decisions? What level of validation is needed? | Emmanuelle, Karolina, Madlen, Stephen Chang, Cindy, Shahed Iqbal  ***Need lead*** |
| RWE to support regulatory decisions in vaccines | Patricia Saddier |
| Pragmatic randomized trials and approaches for regulatory decisions | Cindy Girman, Mary Beth Ritchey,Jacinthe Leclerc |
| Robustness and evaluation of novel designs for regulatory fit-for-purpose and application of these designs in specific product development or submissions | ***Need lead and interested authors*** |
| Recommendations for international and scientific requirements for pharmacoepi studies submitted to regulatory agencies to promote more effective use of RWD | Jacinthe Leclerc; Madlen Gazarian (?); Hisashi Urushihara; Jingping Mo, C Girman, Peter Arlett (reviewer), Kim Brodovicz, Eliott Bosco, John Concato (review), Linda Kalilani-Phiri, Sigal Kaplan ***Need lead*** |
| 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 | M Ritchey; C Girman; M Assimon; K Andersson Sundell; H Gardarsdottir; M Gazarian, Stephen Chang |
| What makes results of a study “believable”? | Cindy Girman; Magdalene Assimon; Jacinthe Leclerc; Kim Brodovicz  *in progress* |
| Increasing the efficiency of RCTs for regulatory decisions post-approval | David Lilienfeld, LT Bloem, Benjamin Bates, Cindy |
| Real world evidence needs for HTA bodies | Kate Bykov, Elisabetta Palerno leading with Hongbo, Cindy, Magdalene, Ashley and others *in progress* *under ISPE manuscript funding* |
| Patient-focused regulatory decision-making using patient experience data: pathways and obstacles to realization | Cathy Anne Pinto, Cindy Girman, Montse Soriano-Gabbarro, Josephine Norquist, Bennett Levitan, Tommi Tervonen, Brett Hauber  *Survey being conducted for paper – in progress* |

**Assignments**

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| No | Action point | Due by | Responsible |
| 1. 1 | Check Papers without a Lead. If interested in leading a paper, reach out to Cindy | 3/15/2022 | Cindy |
| 2 | Cindy to send annotated guidance documents to ISPE liaison to Duke-Margolis and to PhRMA | Complete | Cindy |
| 3 | Meet with RWE Task Force co-leads and chair of Publications & Policy Committee to transition this work group | Meet 2/24 | Kim B |
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The meeting was called to a close by Cindy Girman at 8:55 am ET