RCTs meeting causal inference: principal stratum strategy and beyond

BBS webinar
7th September 2020
Kaspar Rufibach, on behalf of BBS and the organizing committee
Supporting organizations

Basel Biometrics Section
http://bbs.ceb-institute.org

Industry working group “Estimands in Oncology”
www.oncoestimand.org
Organizing committee

Björn Bornkamp (Novartis)

Giusi Moffa (Uni Basel)

Kaspar Rufibach (Roche)
Randomization may not be enough to establish causality!
Goal today:
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Connect causal inference methodology and clinical biostatistics in pharma
14:00 – 14:10  Kaspar Rufibach (member of BBS board and co-lead industry working group “estimands in oncology”)
   Welcome and scene setting

14:10 – 14:35  Vanessa Didelez (Keynote speaker, Leibniz Institute for Prevention Research and Epidemiology - BIPS, Bremen)
   Time-Varying Treatments in Observational Studies: Lessons for Clinical Trial

14:35 – 14:50  Jack Bowden (University of Exeter)
   Connecting Instrumental Variable methods for causal inference to the Estimand Framework

14:50 – 15:05  Kelly van Lancker (Ghent University)
   Efficient, doubly robust estimation of the effect of dose switching for switchers in a randomised clinical trial

15:05 – 15:15  Break

15:15 – 15:30  Björn Bornkamp (Novartis)
   Principal Stratum Strategy: Potential Role in Drug Development

15:30 – 15:45  Dominik Heinzmann (Roche)
   Principal stratum strategy to investigate anti-drug antibody impact on cancer immunotherapy outcome

15:45 – 16:00  Aiesha Zia (Novartis)
   Exploring estimation approaches for principal stratum estimands in Phase III randomized trials in CAR-T anti-cancer therapy

16:00 – 16:10  Break

16:10 – 16:25  Fabrizia Mealli (University of Florence)
   The ICH E9 addendum from an academic causal inference perspective and feedback on the previous talks

16:25 – 16:40  Andrew Thomson (EMA)
   Regulatory feedback on the previous talks

16:40 – 16:55  All speakers
   Comments on talks

16:55 – 17:00  Giusi Moffa (member of BBS board)
   Next webinars and closure
Total number of registrations: 465
type of institution (multiple mentions allowed)

- Pharma: 301 (62%)
- Academia: 100 (21%)
- CRO: 28 (6%)
- Biotech: 27 (6%)
- Regulator: 18 (4%)
- Other: 10 (2%)

Total number of registrations: 465
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- Statistics: 427 (86%)
- Clinical: 35 (7%)
- Regulatory: 9 (2%)
- Epidemiology: 5 (1%)
- Other: 22 (4%)
Total number of registrations: 465

Institution (multiple mentions allowed)

- Roche: 128 (28%)
- Novartis: 57 (12%)
- Bayer: 17 (4%)
- Novo Nordisk: 13 (3%)
- AstraZeneca: 12 (3%)
- Boehringer Ingelheim: 9 (2%)
- University of Basel: 8 (2%)
- BMS: 7 (2%)
- LEO Pharma: 7 (2%)
- Takeda: 7 (2%)
- Ghent University: 6 (1%)
- University of Heidelberg: 6 (1%)
- Other: 187 (40%)
Total number of registrations: 465

country (multiple mentions allowed)

- Switzerland: 151 (32%)
- Germany: 72 (15%)
- United States of America: 64 (14%)
- United Kingdom: 52 (11%)
- Denmark: 23 (5%)
- Belgium: 14 (3%) 14 (3%)
- China: 12 (3%)
- Italy: 8 (2%)
- Canada: 8 (2%)
- Netherlands: 8 (2%)
- Sweden: 39 (8%)
- Other: 14 (3%) 12 (3%) 8 (2%)

Number of registrations: 0-150
Broad representation
Please everyone *mutes* him / herself.
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Ask questions in *chat*. We will reply in discussion or in writing.
Housekeeping

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If presenter approved: **slidedecks** will be posted on BBS webpage.
Housekeeping

Please everyone *mutes* him / herself.

Ask questions in *chat*. We will reply in discussion or in writing.

If presenter approved: *slidedecks* will be posted on BBS webpage.

If presenter approved: *Recording* will be posted on BBS webpage.
Thank you for your attention.

kaspar.rufibach@roche.com
http://www.kasparrufibach.ch

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Doing now what patients need next