American Course on Drug Development and Regulatory Sciences

Substantial Evidence in 21st Century Regulatory Science

Borrowing Strength from Accumulating Data

April 21, 2016

University of California, San Francisco
Schools of Pharmacy and Medicine
Department of Bioengineering
and Therapeutic Sciences

Overview of DIA Bayesian Scientific Working Group (BSWG)

Presentation Developed By…

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Eli Lilly and Company

Disclosures, Affiliations, and Acknowledgements

Contributors to the ideas presented today include:

- Bayesian Scientific Working Group subteam leaders and members (all volunteers!)

Disclosures

- I am an employee of Eli Lilly and Company.

DIA BSWG: Who are we?

Group of representatives from Regulatory, Academia, and Industry, engaging in scientific discussion/collaboration

- facilitate appropriate use of the Bayesian approach
- contribute to progress of Bayesian methodology throughout medical product development

Vision

Ensure that Bayesian methods are

well-understood, accepted, and broadly utilized for design, analysis, and interpretation to **improve patient outcomes**

throughout the medical product development process and to improve decision making.

Mission

To facilitate the appropriate use of Bayesian methods and contribute to progress by:

- **Creating** a scientific forum for the discussion and development of innovative methods and tools.
- **Providing** education on, and promoting the dissemination of, methods and best practices for Bayesian methods.
- **Engaging** in dialogue with industry leaders, the scientific community, and regulators.
- **Fostering** diversity in membership and leadership.

Structure

- Housed under Drug Information Association (DIA)
- Charter exists for full working group
- Full working group teleconferences ~every quarter
- Face-to-face meeting ~annually

Structure

To make progress, we prioritized several topics

-Subteams generally meet monthly

-Chair or co-chairs for each subteam

-Membership within subteams spans academia, regulatory, and industry

-Each subteam has ‘mini-charter’ with key deliverables, goals, etc.

-Each subteam is planning publications, presentations, and so forth

Bayesian Survey

In 2012, we conducted an industry-wide Bayesian survey

-first industry-wide survey to collect information on the use of Bayesian methods amongst statisticians working in medical product development

-results and recommendations were included in the 2014 Special Issue of Pharmaceutical Statistics

Top Hurdles

Insufficient knowledge of the Bayesian approach, particularly on the practical level

Lack of clarity of the regulatory position and/or lack of guidance and experience

Lack of tools including case examples and user-friendly software

Company-internal difficulties (lack of time, lack of support/guidance, general reluctance from team members to accept the Bayesian approach)

Subteams as of April 2016 (chair/co-chair)

1. Safety (Karen Price/Amy Xia/Melvin Munsaka)
2. Use of historical data (John Zhong/Satrajit Roychoudhury)
3. Education (Fanni Natanegara/Mat Davis)
4. Non-inferiority (Mani Lakshminarayanan)
5. Reporting/Tools (Mani Lakshminarayanan)
6. Missing Data (Frank Liu/Stacy Lindborg)
7. Pediatrics (Meg Gamalo)
8. IMI/MAPPs (Robert Campbell/Zoran Antonijevic)
9. Program-wide Decision Making (Bin Yao)
10. Joint Modeling (Larry Gould)

Areas of Focus for 2016

- Developing and disseminating case examples
- Best practice for simulation of Bayesian designs
- Medical outreach
- Engaging with other external initiatives / legislation
- Driving forward work of various subteams

Examples of DIA BSWG Impact

Published numerous papers, including Special Issue Pharmaceutical Statistics

Presented at several conferences, held webinars, taught short courses, etc.

Facilitated many productive conversations across academia/industry/regulatory

Joint DIA Bayes/AD conference (Feb 2015)

Summary

- Bringing together representatives from academia, industry, and regulatory is essential
- Via open scientific dialogue, we can proactively influence on most important issues and learn from one another
- Leads to broader acceptance, enhanced decision making, improved patient outcomes