An Industry Perspective and Expectations on Benefit-Risk Assessment of Vaccines

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Disclaimer

- The views in this presentation are speaker’s personal views and do not represent views of the company
Regulatory Framework for Benefit-Risk Assessment


Regulatory View of Qualitative versus Quantitative Methods

- European regulatory agencies do not currently have a suggested qualitative or quantitative benefit-risk method.
  - PrOACT-URL framework was used in the EMA’s recent Benefit-Risk Assessment methodology project.
  - EMA encourages further exploration of MCDA to aid benefit-risk decision making and considers MCDA as a strong candidate for testing in regulatory setting.
- US FDA position the best presentation of benefit-risk considerations involves focusing on the individual benefits and risks, their frequency and weighing them appropriately.
  - FDA believes that this can be accomplished by a qualitative descriptive approach for structuring the benefit-risk assessment (BRAT).
Clinical value judgment:
"Conducting a benefit–risk assessment requires superimposing evaluative judgments on the scientific facts, such as the available efficacy and safety data." (Veatch, Drug Information Journal 1993)

**What?**
Methods for systematic evaluation of risks and benefits of new or existing medical interventions

**Why?**
These methods evaluate risk benefit trade offs to assist Regulatory and clinical decision-making in absence of directly comparable metrics

**Who?**
Regulators, clinicians, and patients who routinely make decisions that require trading safety for desired clinical benefits

Pharmaco Vigilance

Signals

Risk estimates
(Pharmaco Epidemiology)

Disease Surveillance

Alerts

Vaccine/program effectiveness

Benefit / Risk analysis (Takes years!)

V. Bauchau, GSK Biologics Mar 2014
Pharmaco Vigilance

Signals

Risk estimates (Pharmacovigilance)

Disease Surveillance

Alerts

Vaccine/program effectiveness

Benefit / Risk analysis (Takes years!)
Vaccine Safety

Benefit / Risk Monitoring

Disease Surveillance

[Academic groups, Regulators, MAH]

Alerts

[Public health institutes, ECDC]

Analysis

(Assessment)

V. Bauchau, GSK Biologics Mar 2014
Stakeholders involved in Vaccine Benefit-Risk Assessment

- Regulatory agencies (EMA, FDA, National Regulatory agencies)
- Public health institutes / Ministry of Health / Centers for Disease Control and Prevention
- WHO / PAHO
- Advisory Committees on Immunizations (e.g., ACIP in US, STIKO in Germany, NACI in Canada)
- Vaccine manufacturers
- Academic partners
- Health care providers
- Public (parents, patients etc)
Many stakeholders in BRA but are the views the same?
Industry BRA: Perception

- Perception of Industry BRA
  - Able to perform Benefit-risk assessment for their product on the individual and population levels (impact on disease, herd immunity)
  - Increased transparency and structured approached of BRA involving different internal company stakeholders.
  - Communication tools with HA
- MAH is often taking view of the regulatory agency or public health when making product BRA assessment
Industry BRA: Expectations from Regulatory Agencies

- **Expectations from the Regulators**
  - MAH should be able to perform BRA of vaccine “brand name” X (safety, effectiveness, assessment of alternatives etc)
  - The issues with regard to benefit–risk assessment are complex as well as the lack of guidance and standardization in BRA
  - Need of guidance on B-R methods qualitative vs. quantitative by disease/vaccine areas from Regulators
  - MAH needs discussion with the regulators BRA especially for new vaccine (selection of benefit and safety criteria, value judgment ...)
Challenges in individual product BRA

- While safety profile is established per “brand name”, the vaccine effectiveness data per “brand name” is not available.
- Not the same level of safety information for other MAH products (alternatives).
- Data available may vary by country (better in EU and US).
- MAH may not have access to some data owned by MoH / Public Health.
WP1.2 Landscape analysis of existing models

WP1.2.1: Survey of partners on PP interactions
PP = public-private, public-public, private-private

Survey launched in March 2014 to ADVANCE members

Analysis by June 2014
Report in July 2014
Public-Private Partnership in BRA

- Need to build up partnerships with health authorities for post-licensure
  - Varying vaccine administration settings
  - Access to immunization programs data
  - Program planning and data collected

- Developing trust and transparency between authorities and industry

- Vaccination data collection/access
  - Large population size, many vaccine doses
  - Varying vaccination coverage between regions and over time
  - Multiple vaccination schedules
  - Absence of vaccination registries in most Low and middle income countries (LMIC)
  - Vaccination strategies for age groups targeted and catch up campaigns
Global Support, Data Sharing and Collaboration

- Exchange of safety data between regulatory authorities and market authorization holders from other LMICs
- Vaccine safety / effectiveness initiatives with focus on reference or sentinel sites in the countries
- Global collaboration, plans and partnership needed
  - Country-level criteria
  - International support for standards, methods and tools, information exchange, independent expert advice, communication tools, health care workers, and decision-makers
- Vaccine Safety BluePrint, collaboration with WHO to:
  - Enhance capacity and increase level of vaccine safety activity in countries where vaccines will be introduced
  - Foster international collaboration and strategic planning that leverage infrastructure and expertise
Thank you