PhRMA/BIO Joint Principles

Responsible Information Sharing with Health Care Professionals and Payers

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Bio Biotechnology Innovation Organization

Featured Presenters

WEBINAR FACULTY

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Agenda

1:00 pm **Welcome and Introductions**

Kelly Freeman

1:05 pm **Maximizing the Potential of Real World Evidence to Support Health Care Innovation**

Tom Hubbard

1:25 pm **Points to Consider: Truthful and Non-Misleading Product Communication**

Allen Waxman

1:45 pm **Principles on Responsible Information Sharing with Health Care Professionals and Payers**

Jeff Francer and Deborah Shelton

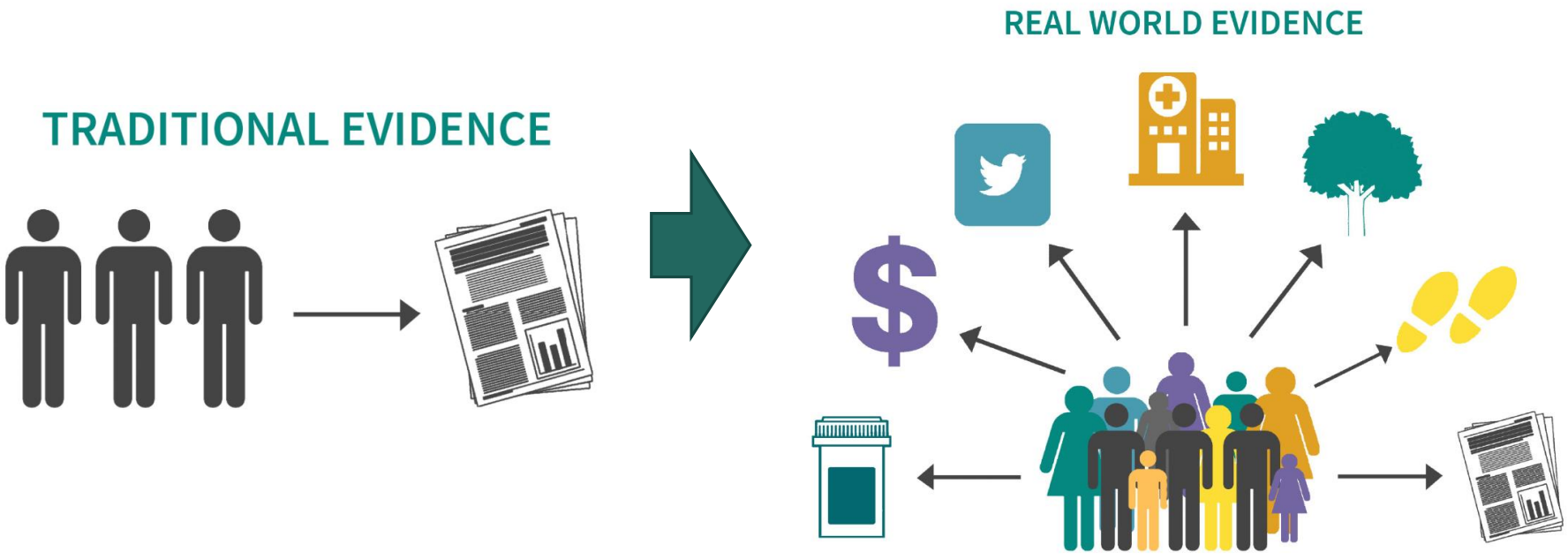
2:20 pm **Q&A**

2:30 pm **Webinar Adjournment**

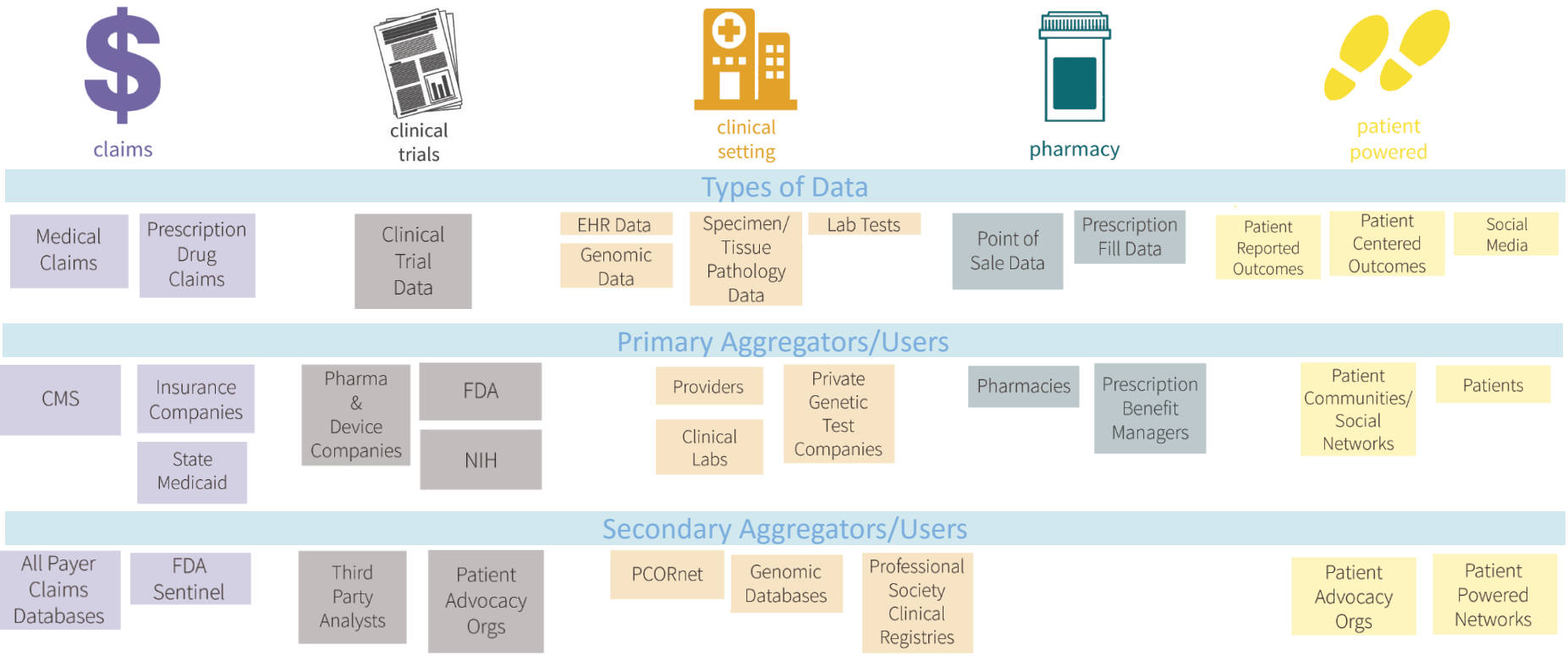
Maximizing the Potential of Real World Evidence to Support Health Care Innovation

Tom Hubbard

Communicate what?

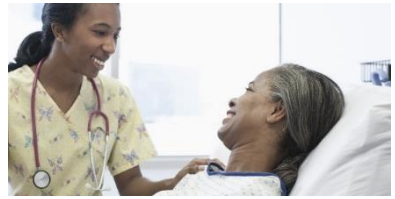


Promise of Real World Evidence (RWE)



RWE and Better Patient Outcomes

1. Safety
2. Effectiveness
3. Effectiveness for previously excluded, under-served or under-identified populations
4. Demonstrate value



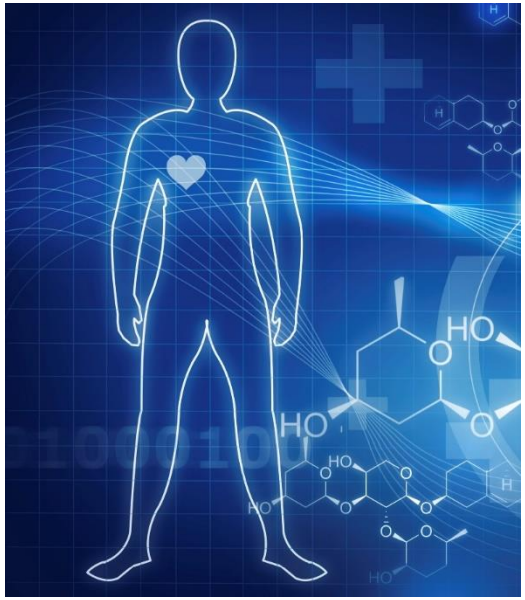
The Role of RWE

A complement, not a substitute for –

Randomized controlled trials (RCTs), or

Randomization, prospective study

Goal – Evidence that is **“fit for purpose”**



RWE and Alternative Payment Models

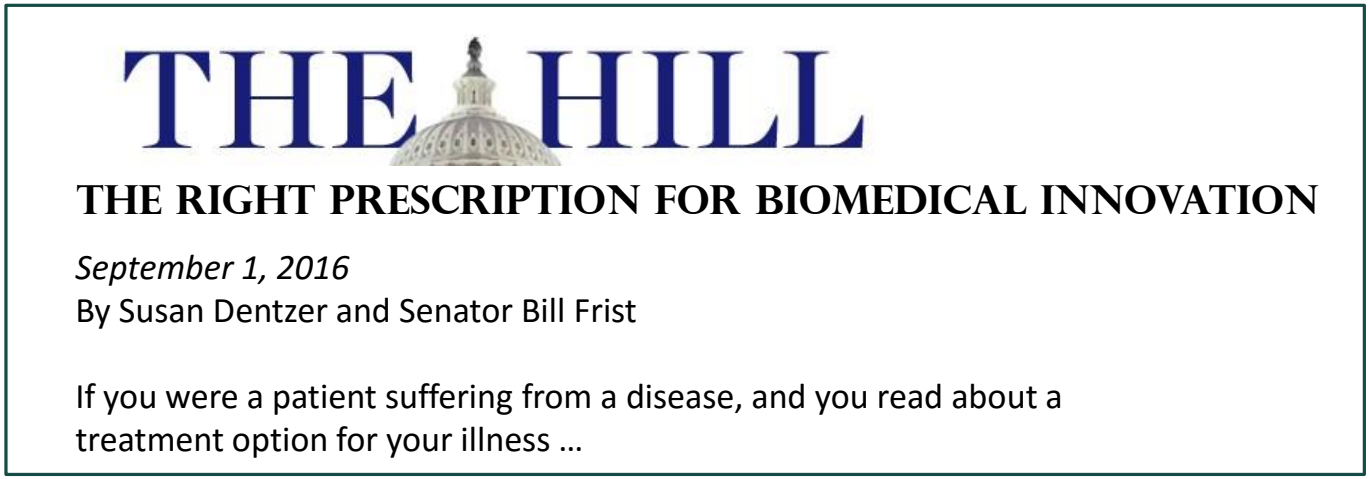
Real world data and fit-for-purpose RWE supports the shift away from fee for service to value-based payment models

- Robust, fit for purpose RWE sharpens the estimation of the value of interventions

Looking Ahead

Stakeholders need to further clarify the rules-of-the-road on fit for purpose, real world evidence

This includes regulatory guidance from the FDA


THE HILL

THE RIGHT PRESCRIPTION FOR BIOMEDICAL INNOVATION

September 1, 2016
By Susan Dentzer and Senator Bill Frist

If you were a patient suffering from a disease, and you read about a treatment option for your illness …

Policy Back Drop

Legislative action on cures

Precision Medicine Initiative

PCORI reauthorization

Open data and clinical trial initiatives

Potential action on FDAMA 114, off-label promotion

PDUFA
GDUFA

User fee negotiations

Amarin vs. FDA

VALUE

Shift to value based payments

Emerging value frameworks

Points to Consider: Truthful and Non-Misleading Product Communication

Allen Waxman

Disclaimer: Mr. Waxman's remarks are his personal opinion, and should not be considered to represent the position of his employer.

BIO's Board Standing Committee on Bioethics

For the last year, the Bioethics Committee has studied the issue of biopharmaceutical companies' truthful and non-misleading communications about their products with healthcare professionals and payers from a bioethical perspective.

This work has included engagement with external experts representing the legal and regulatory perspective on the existing Food and Drug Administration (FDA) regime, representatives of the provider and patient communities, and experts in bioethics.

BIO Bioethics Committee Points to Consider, August 2016

www.bio.org/sites/default/files/Bioethical_PTC_for_Communication_Aug_2016.pdf

Identifying Why Change is Needed

In the past, BIO has primarily focused our advocacy with regard to the issue of truthful and non-misleading product communication around what aspects of FDA's existing regulatory regime need to change, how those changes should be implemented, and to align the regime with First Amendment principles.

However, to ensure that our advocacy is informed by a thoughtful, comprehensive perspective on the issue, the Bioethics Committee's study sought to identify the bioethical underpinnings of why change is needed in the first place and how such considerations should influence the overall framework governing such communications.

BIO's Statement of Ethical Principles

1. Support science-based regulatory standards to govern the use of biopharmaceuticals
2. Help educate the public about biotechnology to enable an informed public discourse about its benefits and implications
3. Facilitate productive relationships among stakeholders to achieve the goal of optimizing patient care for individual patients
4. Support universal access to high-quality health care for all

Four Bioethical Considerations Emanate from the Principles

Through BIO's broader advocacy and through the Bioethics Committee's recent study, we have identified four primary Considerations that can foster an ecosystem that supports those Bioethical Principles:

1. Timely Access
2. Quality Information
3. Meaningful Information
4. Clarity with Regard to Conflicts of Interest

Timely Access

As a crucial aspect of an efficient, effective healthcare system, all relevant parties should ensure that truthful and non-misleading product information— including, but not limited to, the clinical and other information contained in a product's FDA-approved labeling—is available at the point of:

- Patient/provider decision-making
- Payer determinations with regard to coverage and reimbursement

This should include relevant, meaningful, and quality information communicated by biopharmaceutical companies.

Quality Information

No matter the source, it is critical that information communicated about medicines is of high quality.

Thus, advocacy to expand biopharmaceutical companies' ability to communicate truthful and non-misleading information about their therapies should consider:

- Standards for evaluating the quality of the information
- How and by whom the quality will be assessed
- How that assessment will be incorporated into the information communicated

Timely, efficient mechanisms must be in place to ensure the quality of the truthful and non-misleading information that is communicated.

Meaningful Information

Advocacy should consider the scope of the responsibility to provide healthcare providers and payers with information concerning a therapy or medicine.

That scope might be defined by the information that is considered meaningful to the responsible use of therapies or medicines. While providing information necessary to evaluate risks and benefits is important, providing too much information or information that is not meaningful can be detrimental.

Advocacy efforts should consider:

- Standards for evaluating the meaningfulness of the information to be shared
- How and by whom that meaningfulness will be assessed
- How that assessment will be incorporated into the information communicated

Any regime governing truthful and non-misleading communication should take into account whether such information is meaningful in the context of patient care.

Clarity with Regard to Conflicts of Interest

Potential conflicts of interest (COI), with respect to truthful and non-misleading product communication, are not confined to a single stakeholder group, but may exist across the spectrum of healthcare sector stakeholders.

A systematic mechanism for providing adequate context for information communicated by any stakeholder is a crucial part of advocacy to ensure appropriate communications from all stakeholders, with the goal of promoting the best outcome for patients.

COI should be assessed through a common mechanism, not through stakeholder-specific means, and mechanisms to address COI should be built from the existing work on this subject.

Conclusion

These four Considerations should guide a regime that will enable stakeholders to make well-informed decisions based on the individual clinical circumstances of each patient.

In doing so, such a regime will be better able to promote patient access to the most appropriate technologies for them and contribute to increasing the efficiency and effectiveness of the healthcare system.

Robust scientific and medical dialogue serves the widely supported healthcare Triple Aim:

- Improving the patient experience of care (including quality and satisfaction)
- Improving the health of populations
- Achieving a reasonable cost of health care without compromising quality, outcomes, or access

Principles on Responsible Information Sharing with Health Care Professionals and Payers

Jeff Francer

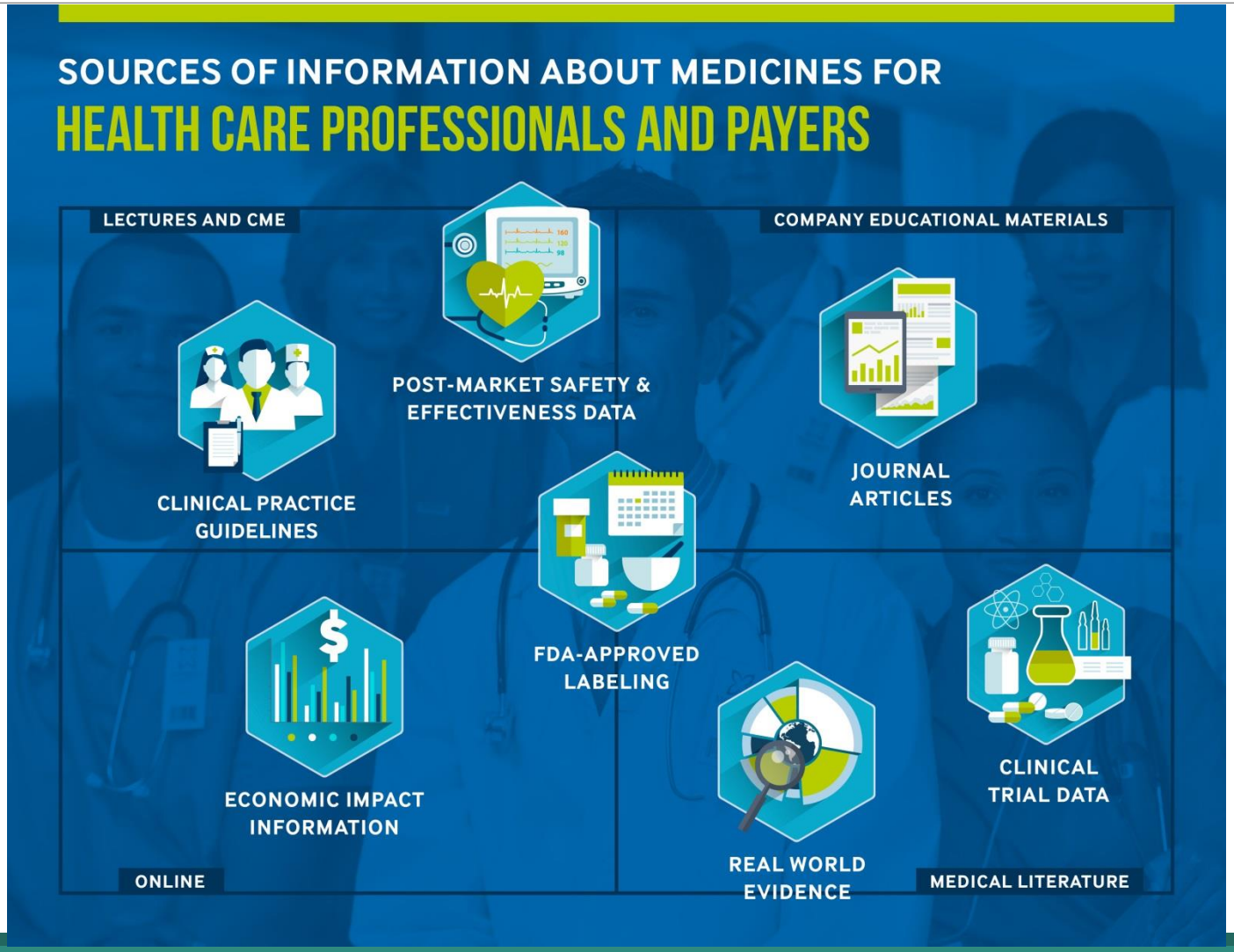
Deborah Shelton

Challenge in Medicine's Information Age

In the era of data-driven medicine, health care professionals and payers seek more, not less, information about the safety, effectiveness, and value of treatments

- ✓Today, the wealth of information about medicines is more comprehensive and complex than ever before
  - Scientific knowledge and new findings go far beyond data sets produced from clinical trials, often are outside the scope of the parameters established by Food and Drug Administration (FDA) regulations, and often outdate the FDA-approved labeling
- ✓In addition to information in the approved labeling for medicines, biopharmaceutical companies continually generate and collect important data and analyses that can **benefit patient care and enhance the efficiency** of our health care system

Patients Expect Physicians to Receive Accurate, Data-Driven Information About Medicines



Opportunity for Regulatory Modernization

Recent court rulings have complicated FDA's regulatory framework:

- Sorrell v. IMS Health Inc. (S. Ct. 2011)
- U.S. v. Caronia (2nd Circuit 2012)
- Amarin Pharma v. FDA (S.D.N.Y. 2016)

FDA may not prohibit communications about medicine if the information is "**truthful and non-misleading**"

A new regulatory framework is needed to ensure that doctors have access to the most timely, accurate data on the medicines they prescribe to patients

Value-Based Health Care: Communications with Payers

Value-based and other innovative approaches to healthcare offer potential to maximize patient benefit and drive smarter spending within healthcare system

Enhanced, responsible information-sharing with payers is important to robust implementation of value-based health care

Enhanced, responsible information-sharing with payers helps to facilitate patient access to the right medicine at the right time

Payers need and want more information from companies

- Pipeline information
- Information consistent with approved indication but not in labeling

Payers are highly sophisticated consumers of clinical and pharmacoeconomic data, and have capabilities to analyze large and complex datasets to inform decision-making

A Responsible Path Forward

FDA should define clear standards governing *responsible, truthful, and non-misleading communications* to inform health care professionals and payers about the safe and effective use of medicines.

Key principles should include:

- **Science-based communication**
- **Provide appropriate context about data**
- **Tailoring communications to the intended audience**

The PhRMA-BIO Principles pertain primarily to data and information outside of FDA-approved labeling, such as additional clinical trials or analysis of real-world patient outcomes.

Overview of Principles for Responsible Information Sharing

Commitment to Accurate, Science-based Communication

- ✓ Communications should be based on analyses using scientifically- and statistically-sound methodologies
- ✓ Information can include pharmacoeconomic analyses, analyses of real world evidence and post-hoc analyses that focus on specific sub-populations

Overview of Principles for Responsible Information Sharing

Commitment to Transparency About Claims and Substantiation

- ✓ FDA-approved labeling is a primary source in sharing information about medicines
- ✓ Companies should provide scientific substantiation if shared Information is not contained in FDA-approved labeling
- ✓ Substantiation should include information about limitations of the data and the analyses conducted to prevent healthcare professionals from reaching inaccurate conclusions or forming misimpressions about the efficacy or safety of a medicine

Overview of Principles for Responsible Information Sharing

Commitment to Tailoring Communications to the Intended Audience

- ✓ Communications should take into account the sophistication of the intended audience so that the intended audience can accurately incorporate the new information into existing body of knowledge and expertise
- ✓ Distinction between formulary committees / payers / practitioners
- ✓ Not intended to limit scientific communication (e.g., medical meetings, peer-reviewed publications)

Management of Regulatory Reform Categories of Communication

Communications with Payers / Population Health Decision Makers

- Pharmacoeconomic information
- Pipeline information (pre-approval)
- Broad clinical information to payers

Communications with HCPs (*Consistent w/ Approved Indication*)

- Real World Evidence
- Subpopulation information
- Other information from clinical trials

Communications with HCPs (*Medically Accepted Alternative Uses*)

- Real World Evidence
- Subpopulation information
- Other information from clinical trials

Nine Principles on Responsible Sharing

1. Commitment to Accurate, Science-Based Communications
2. FDA-Approved Labeling is a Primary Source in Sharing Information with Health Care Professionals About Medicines
3. Companies Should Provide Scientific Substantiation if Shared Information is Not Contained in FDA-Approved Labeling
4. Additional Science-based Information from Sources Other Than FDA-Approved Labeling Helps Health Care Professionals and Payers Make Informed Decisions for Patients
5. Communications Should Be Tailored to the Sophistication of the Intended Audience

Nine Principles on Responsible Sharing

6. Science-based Information About Alternative Uses of Medicines Can Improve Health Care Decision-Making
7. Communicating with Payers About New Medicines and New Uses of Approved Medicines Facilitates Patient Access Upon Approval
8. Real-World Evidence Based on Patient Experience and Pharmacoeconomic Information Can Improve Understanding of Health Outcomes and Costs
9. Commitment to Share Information Published in Scientific or Medical Journals

Scenario 9

In collaboration with a large health insurer, a biopharmaceutical company has evaluated the rate of hospitalizations for patients who use the company's cardiovascular drug for its indicated use, compared with the rate of hospitalizations for patients who use a competitor's drug, based on real-world evidence from the insurer's electronic medical records for over 200,000 adult patients nationwide.

The data demonstrate that both the company's drug and the competitor's drug significantly reduced the rate of hospitalizations in patients ages 50-65. However, the competitor's drug demonstrated a higher rate of hospitalizations in this population.

Scenario 9

After communicating accurate and balanced information about use of the company's product in accordance with the approved labeling, to communicate this real-world data to additional payers in a truthful and non-misleading manner, the company should disclose, among other things:

(a) the observational nature of this study, based on a review of the insurer's member data;

(b) the study methodology and method(s) of statistical analysis;

(c) any significant limitations of the data or the databases used;

(d) the results of the study for both the manufacturer's drug and the competitor drug;

(e) any pertinent safety results of this observational study; and

(f) any risk of bias not otherwise described above.

The company should summarize these disclosures in the oral or written communications, and can refer payers to a website for more comprehensive information about the observational study.

This scenario implicates Principles 7 and 8.

Scenario 10

A biopharmaceutical company contacts a major health plan and requests an opportunity to present information regarding its oncology product pipeline.

The company's slide presentation includes a timeline showing agents that are in Phase 3, Phase 2, and Phase 1 of development, with a one-page description of each study, including the study design and primary and secondary end points.

The presentation is for the pharmacy and therapeutics committee of the health plan ("P&T Committee"), whose members include physicians and doctors of pharmacy. This is a highly sophisticated audience.

Scenario 10

The respective descriptions of the studies include results of primary and secondary endpoints and statistical significance but do not make statements that any of the drugs has been determined to be safe or effective.

To communicate top-level pipeline information to the this audience in a truthful and non-misleading manner, the company should disclose, among other things:

(a) the lack of FDA approval;
(b) the possibility that FDA will not approve some agents in the pipeline; and
(c) any material safety risks identified in the clinical studies conducted to date.

This scenario implicates Principles 5 and 7.

Scenario 11

A biopharmaceutical company has submitted to FDA its NDA for an investigational oncology drug and expects approval within nine months.

The company has scheduled meetings with the P&T committees of several pharmacy benefit managers and health plans to inform them that the product likely will be available within the year and to request that they consider placing it on their formularies promptly upon approval.

Scenario 11

To communicate information about the anticipated product indication, any limitations of use, and the safety and efficacy data submitted to FDA as part of the application for approval in a truthful and non-misleading manner, the company should disclose, among other things:

(a) the current status of the NDA;

(b) the type of research that supports the safety and efficacy for the use of the product under consideration by FDA (with appropriate, context-specific disclosures regarding the specific research);

(c) any FDA opinion on the sufficiency of the evidence; and

(d) other relevant evidence that is necessary to an informed medical judgment, including any peer-reviewed contrary evidence.

The company should make these disclosures as part of the oral or written communication.

This scenario implicates Principle 7.

Q & A

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