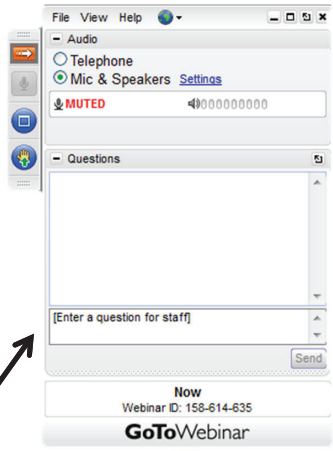
AMCP Update
FDA Preapproval Information Exchange

AMCP Webinar

October 30, 2018

2:00-3:00pm

How to Ask A Question



Type your question in the 'Questions' area

Participants

Mary Jo Carden, RPh, JD,
Vice President, Government & Pharmacy Affairs, AMCP

Amy Duhig, PhD
Vice President, Consulting Services, Xcenda

Iris Tam, PharmD
Director and Head, Outcomes Research & Quality of Care, Medical Affairs, Achaogen

Steven G. Avey, MS, RPh
Vice President, Specialty Pharmacy, MedImpact Healthcare Systems, Inc.

Webinar Overview

- Update on AMCP activity related to preapproval information exchange (PIE)
- Payer and manufacturers' perspective on importance and usefulness of PIE
- Relationship to AMCP *Format for Formulary Submissions* (Format)
- Manufacturer perspective on PIE
- Payer perspective on PIE

AMCP PIE ACTIVITY

Mary Jo Carden, RPh, JD
Vice President, Government and Pharmacy Affairs
AMCP

Final FDA Guidance-Payer Manufacturer Communications

- Released by FDA on June 11, 2018
- Clarifies how biopharmaceutical manufacturers can communicate truthful and non-misleading information with payers across a product's lifecycle
- Provides assurance that manufacturers can share with payers certain health care economic information (HCEI) on **unapproved products and unapproved uses of cleared drugs**, as long as certain conditions met
  - Studies
  - Disclosures that indicates indication has not been FDA approved

Final Guidance on Drug and Device Manufacturer Communications With Payers, Formulary Committees, and Similar Entities

- On July 13, AMCP submitted comments to FDA
- AMCP supports final guidance measures that expand the scope of preapproval communications to include new indications of approved molecules, and not solely new molecular entities
- AMCP had previously developed recommendations that aligned largely with this guidance at a Partnership Forum
  - Partnership Forum Proceeding Link: https://www.jmcp.org/doi/10.18553/jmcp.2016.16366
- AMCP will continue efforts to advocate for the passage of H.R. 2026, The Pharmaceutical Information Exchange (PIE) Act of 2018 to legally codify provisions to allow for payer-manufacturer communications
- Link to AMCP comments: http://www.amcp.org/WorkArea/DownloadAsset.aspx?id=23741

PIE Bill

H.R. 2026—Pharmaceutical Information Exchange Act of 2018

- Introduced in April 2017 by Rep. Brett Guthrie (R-KY)
- Clarifies scope of permitted health care economic and scientific information communications between biopharmaceutical manufacturers and health care decision-makers
- AMCP participated in hearing held on July 12, 2017
- House Energy and Commerce Health Subcommittee approved on January 17, 2018

PAYERS' AND MANUFACTURERS' PERCEPTIONS OF THE IMPORTANCE AND USEFULNESS OF PIE

Amy Duhig, PhD
Vice President, Consulting Services
Xcenda

Payer and Manufacturer Surveys on PIE

Two surveys conducted (December 2017 and April–July 2018) to understand United States (US) payer and manufacturer experiences, attitudes, and perceptions of PIE

Payers (n=44)

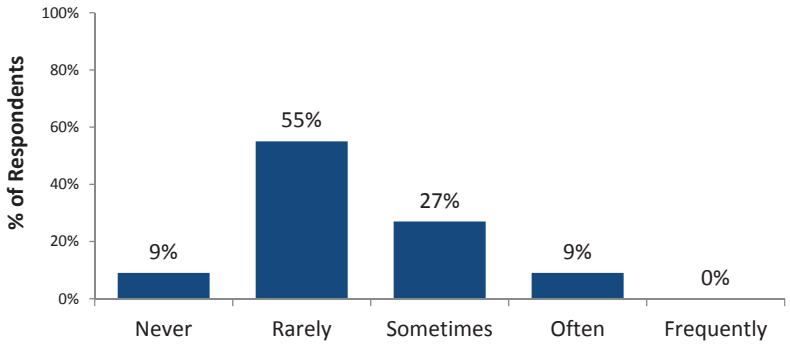
- 10 items
- 68% managed care organizations, 27% pharmacy benefit managers
- 231 million lives
- 68% regional plans
- 64% pharmacy directors

Manufacturers (n=41)

- 10 items
- Small- to large-sized companies
- 56% health economics and outcomes research, 22% market access
- 75% director or above

PAYER

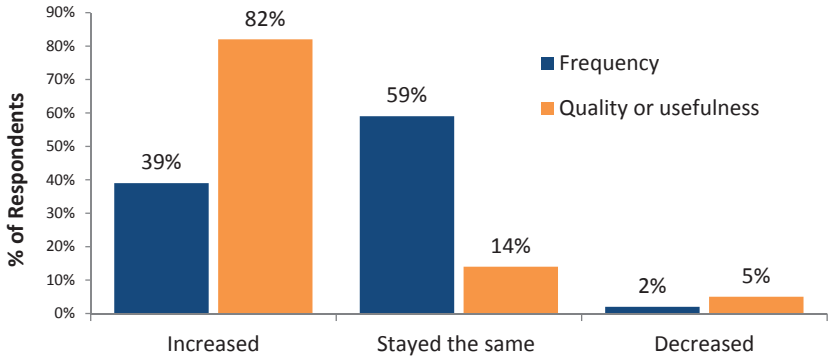
Frequency of PIE Prior to January 2017



Prior to January 2017, how frequently did you receive proactive preapproval communications from pharmaceutical manufacturers about investigational products?

PAYER

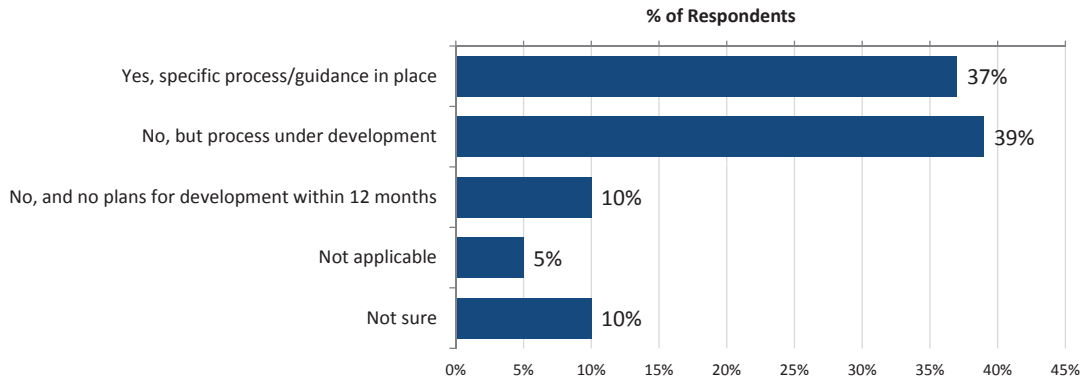
Change in PIE Frequency and Quality or Usefulness Since 2017



Over the past year (since January 2017), how would you characterize the change in frequency for proactive communication by manufacturers for investigational products?
Over the past year (since January 2017), how would you characterize the change in quality or usefulness of the type of information shared proactively about investigational products?

MANUFACTURER

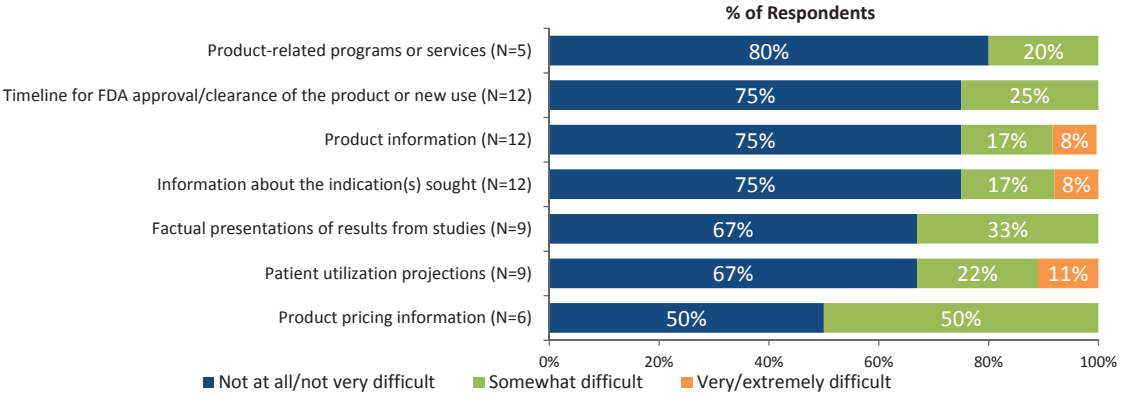
Availability of Process/Guidance Within Organization to Approve Materials Intended for PIE



Question: Is there a specific process/guidance (eg, SOP, formal committee, etc) in place within your organization to approve materials intended for PIE? (N=41)

MANUFACTURER

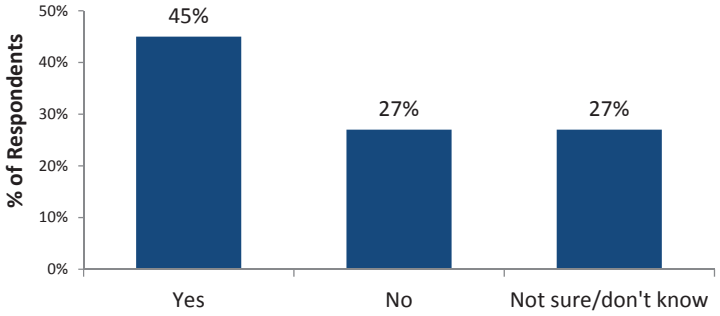
Difficulty Experienced in Gaining Approval for Each Type of PIE Within Organization



Note: Ratings based on types of PIE used within respondent organizations.
1. Question: For each type of PIE listed, please rate the level of difficulty experienced in gaining approval. (N=See chart)

PAYER

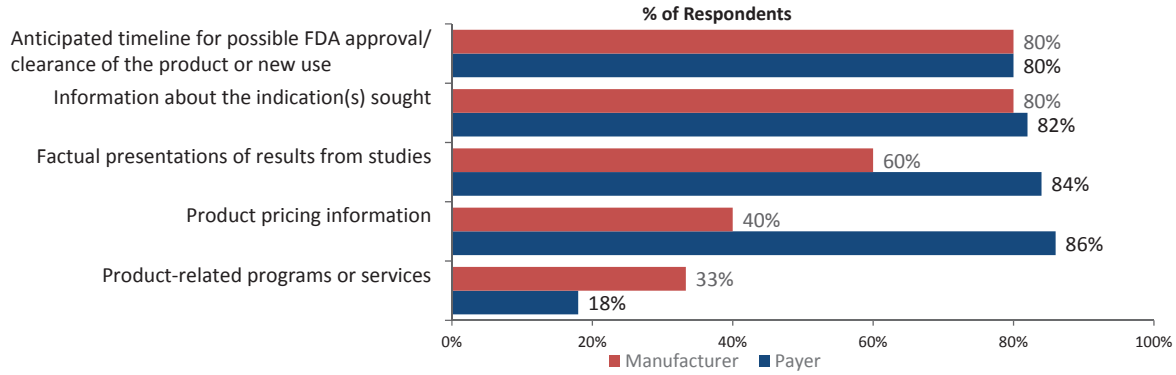
Gap Between Needed and Available PIE



Question: Is there a gap between the type of PIE about investigational products your organization needs for the formulary decision-making process and what is available in the literature and/or supplied by the manufacturer?

PAYER AND MANUFACTURER

Types of Information About Investigational Products Desired vs Approved

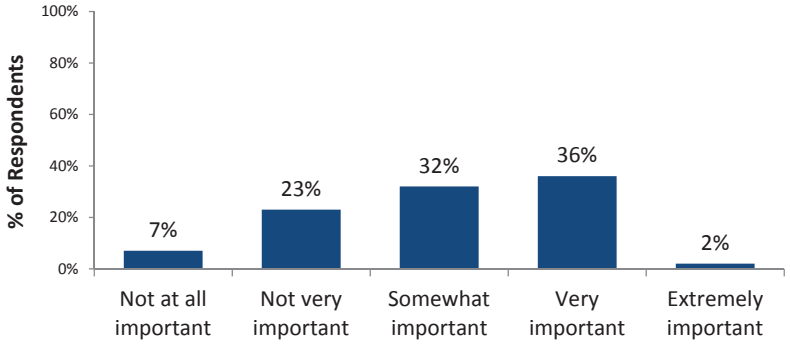


Question: Which, if any, of the following types of information about investigational products are approved in your organization for PIE discussions with eligible entities? (N=15)

According to the draft guidance, the following information about investigational products may be communicated by manufacturers to a payer or similar organization. For each of the following, please rate the level of importance to you or your organization in receiving this information proactively and prior to approval from a manufacturer. Note: Percentages reflect the proportion of respondents who rated each type of information as "very" or "extremely" important.

PAYER

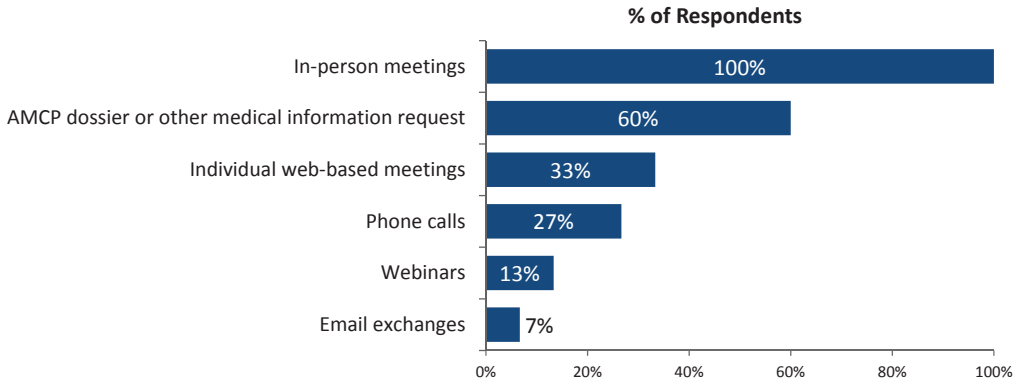
Importance of Healthcare Economic Information (HCEI) in PIE



The FDA draft guidance does not include PIE related to HCEI. How important is it for you to receive proactive HCEI communications about investigational products?

MANUFACTURER

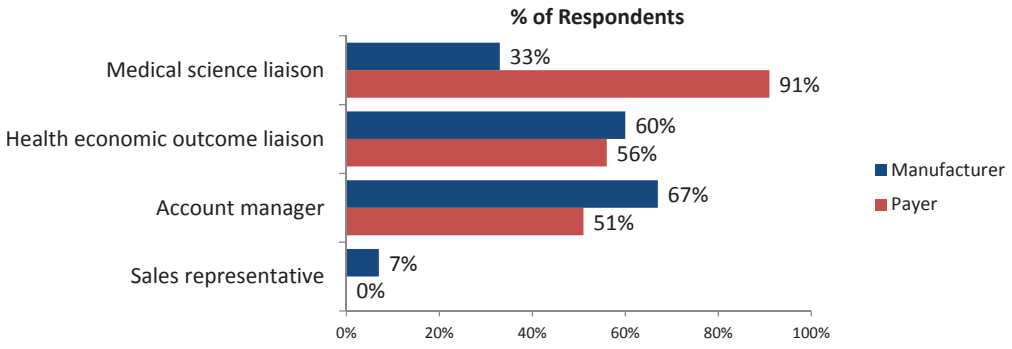
Methods Currently Used in Communicating With Eligible Entities Regarding PIE



Question: How is your organization currently communicating with eligible entities regarding HCEI? (N=41)

PAYER AND MANUFACTURER

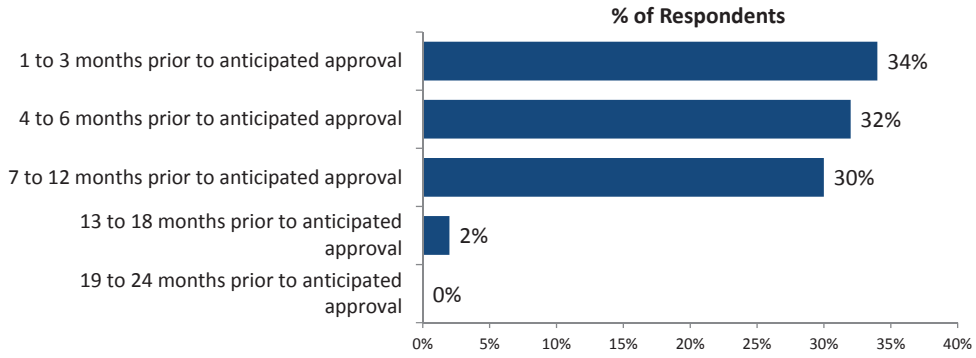
Sharing of PIE Information[a]



Payers: Which manufacturer representative do you prefer to have share PIE with you/your organization? Please rank order. [a] Ranked first or second.
Manufacturers: Who in your organization is conveying PIE with eligible entities?

PAYER

Timing of PIE Receipt



How early would you/your organization like to receive PIE from a manufacturer?

Summary

- Payers view PIE about investigational products as important, yet a gap exists regarding the information received
- In order to be useful for plan forecasting, PIE will need to be delivered prior to the timelines suggested by payers in the current study
- Value and pricing will only be emphasized more during PIE discussions as time progresses
- The FDA's most recent guidance (June 2018) on HCEI communication may further evolve this practice, along with potential legislative revisions to codify PIE activity

PIE: RELATIONSHIP TO AMCP *FORMAT* AND MANUFACTURER PERSPECTIVES

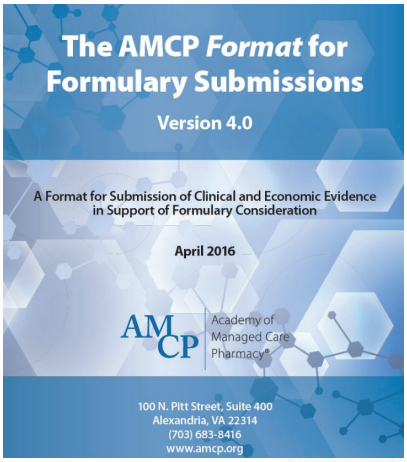
Iris Tam, PharmD
Director and Head, Outcomes Research &
Quality of Care Medical Affairs
Achaogen

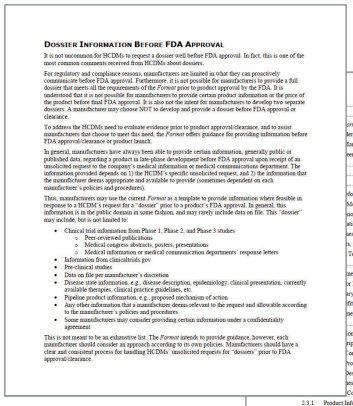
Topics

- Review AMCP Format for Formulary Submissions, Version 4.0
  - Dossier Information Before FDA Approval
- Recap FDA Guidance (Pre-approval Information Exchange, PIE)
- Compare AMCP Format and FDA Guidance
- Manufacturer considerations for implementing PIE

AMCP Format for Formulary Submissions, Version 4.0

Dossier Information Before FDA Approval





Dossier Information Before FDA Approval

It is not uncommon for HCDMs to request a dossier well before FDA approval. In fact, this is one of the most common comments received from HCDMs about dossiers.

For regulatory and compliance reasons, manufacturers are limited in what they can proactively communicate before FDA approval. Furthermore, it is not possible for manufacturers to provide a full dossier that meets all the requirements of the *Format* prior to product approval by the FDA. It is understood that it is not possible for manufacturers to provide certain product information or the price of the product before final FDA approval. It is also not the intent for manufacturers to develop two separate dossiers. A manufacturer may choose NOT to develop and provide a dossier before FDA approval or clearance.

To address the HCDMs need to evaluate evidence prior to product approval/clearance, and to assist manufacturers that choose to meet this need, the *Format* offers guidance for providing information before FDA approval/clearance or product launch.

In general, manufacturers have always been able to provide certain information, generally public or published data, regarding a product in late-phase development before FDA approval upon receipt of an unsolicited request to the company's medical information or medical communications department. The information provided depends on 1) the HCDM's specific unsolicited request, and 2) the information that the manufacturer deems appropriate and available to provide (sometimes dependent on each manufacturer's policies and procedures).

Thus, manufacturers may use the current *Format* as a template to provide information where feasible in response to a HCDM's request for a "dossier" prior to a product's FDA approval. In general, this information is in the public domain in some fashion, and may rarely include data on file. This "dossier" may include, but is not limited to:

- Clinical trial information from Phase 1, Phase 2, and Phase 3 studies
  - Peer-reviewed publications
  - Medical congress abstracts, posters, presentations
  - Medical information or medical communication departments' response letters
- Information from clinicaltrials.gov
- Pre-clinical studies
- Data on file per manufacturer's discretion
- Disease state information, e.g., disease description, epidemiology, clinical presentation, currently available therapies, clinical practice guidelines, etc.
- Pipeline product information, e.g., proposed mechanism of action
- Any other information that a manufacturer deems relevant to the request and allowable according to the manufacturer's policies and procedures
- Some manufacturers may consider providing certain information under a confidentiality agreement

This is not meant to be an exhaustive list. The *Format* intends to provide guidance, however, each manufacturer should consider an approach according to its own policies. Manufacturers should have a clear and consistent process for handling HCDMs' unsolicited requests for "dossiers" prior to FDA approval/clearance.

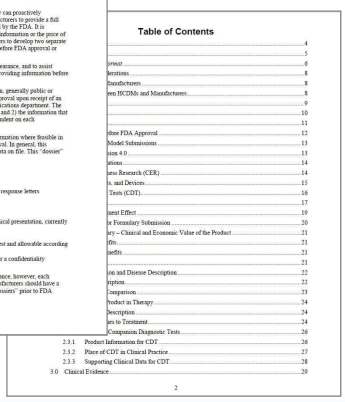


Table of Contents

AMCP *Format*: Information Before FDA Approval

- Clinical trial information from Phase 1, Phase 2, and Phase 3 studies
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  - Medical information or medical communication departments' response letters
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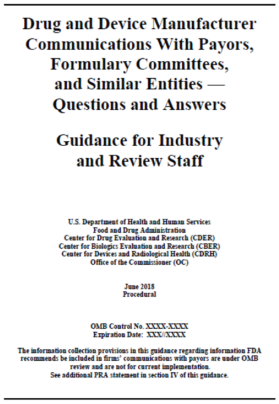
AMCP Format Executive Committee. The AMCP Format for Formulary Submissions, Version 4.0. April 2016. Available at: http://www.amcp.org/FormatV4/. Accessed 7/15/18.

FDA Guidance: Manufacturer Communications with Payers

- Final guidance released June 12, 2018
- FDA's thinking about manufacturers':
  - Communication of healthcare economic information to payors regarding approved drugs
  - Communications to payors about
    - Unapproved drugs
    - Unapproved uses of approved drugs



Drug and Device Manufacturer Communications With Payors, Formulary Committees, and Similar Entities — Questions and Answers

Guidance for Industry and Review Staff

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Center for Devices and Radiological Health (CDRH)
Office of the Commissioner (OC)

June 2018
Procedural

OMB Control No. XXXX-XXXX
Expiration Date: XXX/XXXX

The information collection provisions in this guidance regarding information FDA recommends be included in firms' communications with payors are under OMB review and are not for current implementation.
See additional PRA statement in section IV of this guidance.

FDA. Drug and Device Manufacturer Communications with Payors, Formulary Committees, and Similar Entities – Questions and Answers: Guidance for Industry and Review Staff. June 2018.

Communications About Unapproved Drugs & Unapproved Uses of Approved Drugs

Types of information:

- Product information (e.g., drug class, device description and features)
- Information about the indication(s) sought, such as information from the clinical study protocol(s) about endpoint(s) being studied and the patient population under investigation (e.g., number of subjects enrolled, subject enrollment criteria, subject demographics)
- Anticipated timeline for possible FDA approval/clearance/licensure of the product or of the new use
- Product pricing information
- Patient utilization projections (e.g., epidemiological data projection on incidence and prevalence)
- Product-related programs or services (e.g., patient support programs)
- Factual presentations of results from studies

FDA. Drug and Device Manufacturer Communications with Payors, Formulary Committees, and Similar Entities – Questions and Answers: Guidance for Industry and Review Staff. June 2018.

Communications About Unapproved Drugs & Unapproved Uses of Approved Drugs (2)

Other information that should be provided:

- A clear statement that the product or use is not approved/cleared/licensed, and that the safety or effectiveness of the product or use has not been established
- Information related to the stage of product development…in which a product/new use is being investigated and how it relates to the overall product development plan, whether a marketing application for the product or new use has been submitted to FDA or when such a submission is planned
- For factual presentations of results from studies, describe material aspects of study design, methodology, material limitations related to the study design, methodology, and results; ensure that results are not selectively presented
- A prominent statement disclosing the indication(s) for which FDA has approved, cleared, or licensed the product and a copy of the most current FDA label
- Provide follow-up information to payers if previously communicated information becomes materially outdated as a result of significant changes or as a result of new information regarding the product, e.g., development or regulatory status

FDA. Drug and Device Manufacturer Communications with Payors, Formulary Committees, and Similar Entities – Questions and Answers: Guidance for Industry and Review Staff. June 2018.

FDA Final Guidance v AMCP *Format*

FDA Final Guidance

- Product information (e.g., drug class, device description and features)
- Information about the indication(s) sought, such as information from the clinical study protocol(s) about endpoint(s) being studied and the patient population under investigation (e.g., number of subjects enrolled, subject enrollment criteria, subject demographics)
- Anticipated timeline for possible FDA approval/clearance/licensure of the product or of the new use
- Product pricing information
- Patient utilization projections (e.g., epidemiological data projection on incidence and prevalence)
- Product-related programs or services (e.g., patient support programs)
- Factual presentations of results from studies, including clinical studies….

AMCP *Format* V.4.0

- Clinical trial information from Phase 1, Phase 2, and Phase 3 studies
  - Peer-reviewed publications
  - Medical congress abstracts, posters, presentations
  - Medical information or medical communication departments' response letters
- Information from clinicaltrials.gov
- Pre-clinical studies
- Data on file per manufacturer's discretion
- Disease state information, e.g., disease description, epidemiology, clinical presentation, currently available therapies, clinical practice guidelines, etc.
- Pipeline product information, e.g., proposed mechanism of action
- Any other information that a manufacturer deems relevant to the request and allowable according to the manufacturer's policies and procedures

Considerations for Industry

CONTENT
Evidence/data, information, price/economics

DEVELOPMENT PROCESS
When
Who
How

COMMUNICATION PROCESS
When
Who
How

PIE: PAYER PERSPECTIVE

Steven G. Avey, MS, RPh, FAMCP
Vice President, Enterprise Specialty Clinical Solutions
MedImpact Healthcare Systems, Inc

Topics to be Covered

- Payer challenges with new therapies
- Current services to meet payer needs
- How PIE will help managed care better serve members and payers
- Best practices that I have experienced with manufacturers

PAYER CHALLENGES WITH SPECIALTY DRUGS

Specialty Spend and Trend

- **1.5 to 2%** of prescriptions are for a specialty medication
- Close to **50% of total prescription drug costs** are for specialty drugs
- Average cost of a specialty drug is approximately **$4,000** but the range is from **$670 to $58,000** for one month of medication
- Specialty trend continues to escalate each year between **10% and 14%**
- Specialty cost inflation has run between **11 and 15%** - 2017 was slightly lower at ~ 10%
- In 2017 trend for Hepatitis C drugs were down between **30 and 35%** due to lower utilization and costs being substantially down

Treatments for Orphan Diseases Are Challenging

Orphan Drugs: Providing Hope… Creating Concerns

Rare diseases are not so rare

The affect nearly **30 Million** Americans–compare this to the14.5 million with a history of cancer and the 1.5 million who have a stroke or heart attack.

Of the new drugs approved in 2016 **41%** where orphan drugs used to treat a rare disease or condition.

Only **5%** of rare diseases have treatments available.

When they are available they tend to very expensive – with average annual drug costs per patient of **$140,000.**

Drug cost is a primary concern to employers

55%

With the high price tags associated with many orphan drugs, it is unsurprising that over half (55%) of respondents rated drug costs as their top concern.

71% do not feel the current prices of orphan drugs are sustainable.

But other concerns abound:

- "Lack of information on efficacy."
- "How much we don't know about then and what's out there that could at some point devastate our healthcare cost budget."
- Patient/provider demand – even though a drug may not be overwhelmingly effective, if it is the ONLY treatment option for that disease, patients and providers demand it and insist that the plan must cover it (e.g., Spinraza)."
- "There is going to reach a point at which the market is not going to be able to support additional cost."

Limited Distribution Drugs (LDD) Need Attention

NUMBER OF LDDs

185

Currently on the market

AVERAGE LDD COST

$8,595

Per 30-day supply of LDD prescription

IMPACT OF LDDs

11/25

11% by claim volume and 25% by claim total cost.

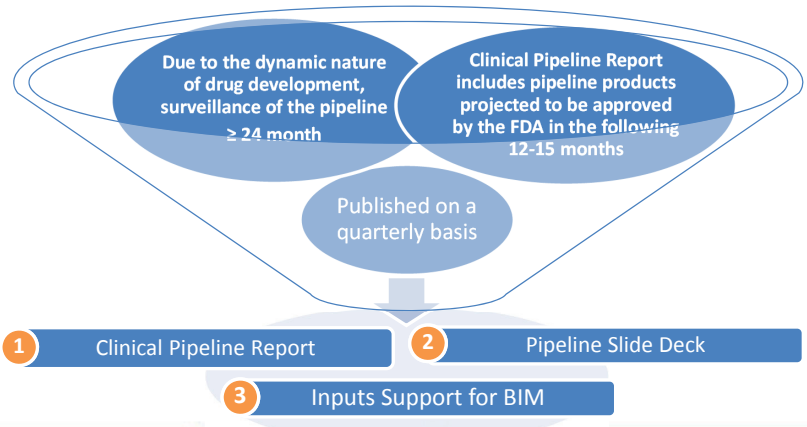
LDD TREND

51%

31 of the 61 specialty drugs launched in 2017 were LDDs

CURRENT SERVICES TO MEET PAYER NEEDS

Pipeline Process at MedImpact



Drug Information Team uses this information to develop formulary therapeutic class strategy used for P&T and Formulary committee/strategy meetings

Criteria for Modeling

- Drug Information takes the following into consideration

Consideration	Criteria
Timing	FDA filing or anticipated approval within the next 12 months • *Accounts for standard and priority/accelerated reviews*
Clinical Impact	Pipeline agent will change standard of care and has high potential for early adoption
Cost	• Must fall into one of the four cost categories: – *Displacement cost, shift in cost, additive cost or disease state breakthrough* • Considers total treatable target population and estimated cost of pipeline agent

- Pipeline agents selected for modeling by cross-functional Health Services team, led by DI
  - Team : DI, HOR, Specialty, and Clinical Account Services Team

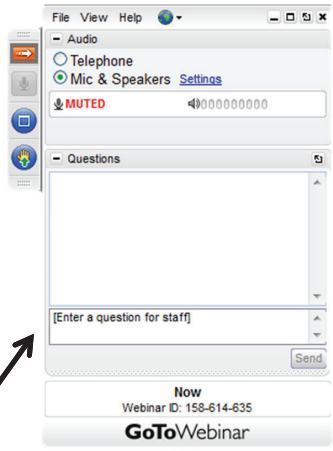
What is missing…

- Pricing information
- Target population
- Indication(s) – initial and subsequent
- Discussion about phase III trial end points
- Anticipated Limited Distribution discussion

Best Practices

- Pharmaceutical company A discussed their potential phase III trial and asked for our input on proposed end points
- Pharmaceutical company B shared their strategy on Limited Distribution pharmacies. We had a discussion about the challenges we have with certain specialty pharmacies
- Manufacturer C shared the primary indication they were seeking for the new medication as well as other possible indications they could go after subsequent to launch with the relative number of additional patients that could benefit from the medication
- Manufacturer D discussed the potential "value" statement they believed their new medication offered and asked our opinion as to our thoughts if that value statement was relevant

How to Ask A Question



Type your question in the 'Questions' area

AMCP Webinars

Available at http://www.amcp.org/webinars/

Check your email for more information on AMCP webinars on late-breaking topics!