Science & Innovation Theater WEBINARS

Developed for Managed Care Pharmacy by AMCP Corporate Members

Pre- and Post-Approval Dossiers: Making All Evidence Count

September 12, 2018

Presented By

Moderator: Laurie Fazio
Senior Vice President, Market Access Technologies, Dymaxium

Elizabeth Sampsel, PharmD, MBA, BCPS
Vice President, Payer Strategy and Relations, Dymaxium

Sina Carlson, PharmD
Senior Director, Drug Intelligence, OptumRx

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

Objectives for the Webinar

2018 US payer responses - AMCP eDossier System @FormularyDecisions.com® community

Payer perspective - dossier use including strengths and opportunities for improvement

Manufacturer perspective - dossier development and maintenance, request response and adherence to AMCP Format for Formulary Submissions.

AMCP format for formulary submissions - v4.0 future considerations (pre- and post FDA-approval)

Perspectives of pre-approval dossiers

Science & Innovation Theater WEBINARS

Environmental Scan – Payer Perspective

Elizabeth Sampsel
Vice President, Payer Strategy and Relations
Dymaxium

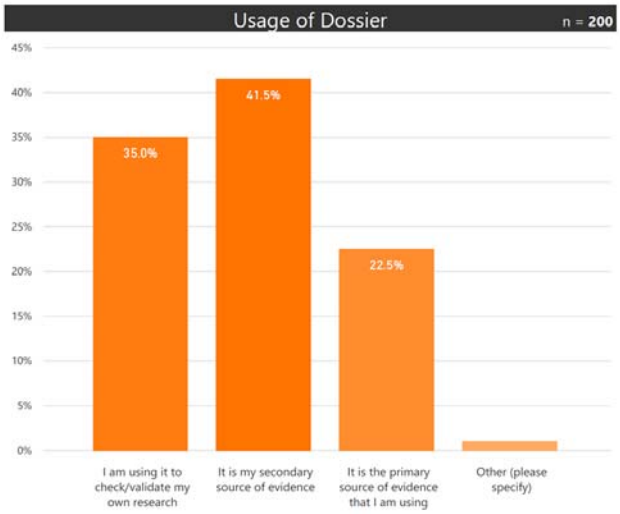
Environmental Scan – Payers

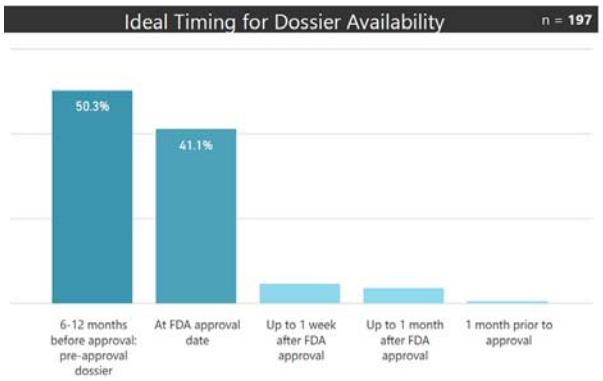
AMCP eDossier system FormularyDecisions.com dymaxium

- Purpose: To better understand the payer perspective on new product reviews and the quality of evidence that was used to inform formulary placement.
- N = 243
- Survey timeline: 11/28/2017 to 8/15/2018
- Respondents:
  - VP/Directors/Managers/Supervisors (31%), Clinical Pharmacists (65%), Other (4%)
  - MCOs (52%), also represents PBM, Providers, Government, Other
  - Organizations represented cover nearly all of the US covered lives.

Dossier Usage and Timing

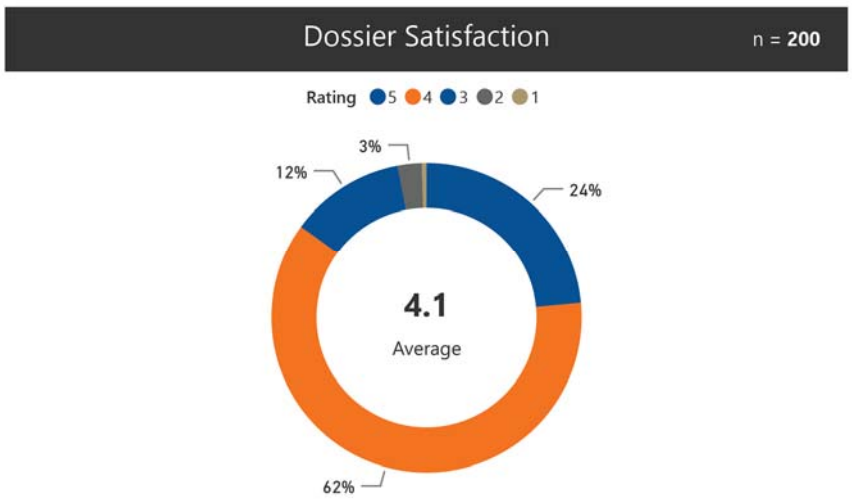
88.6% payers using dossiers





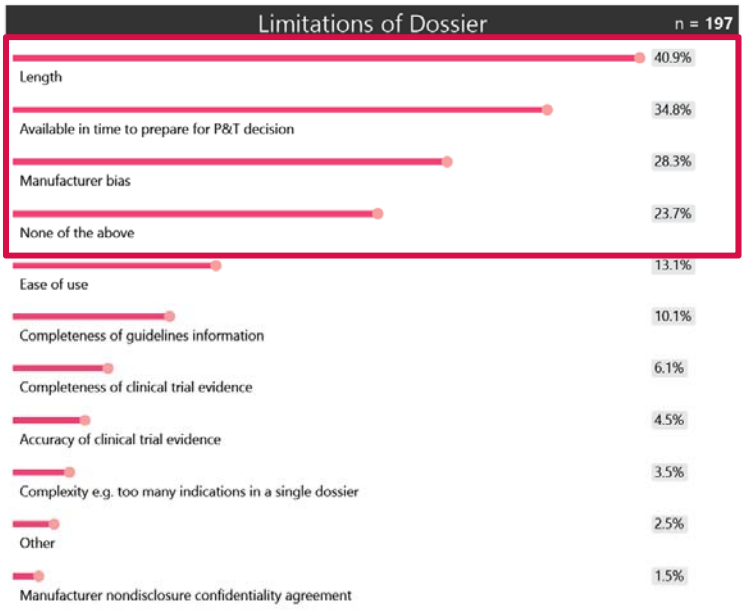
Payers/HCDM are using dossiers for their reviews and prefer availability 6-12 months prior to FDA approval

Dossier Satisfaction



Overall, payers are satisfied with manufacturer dossiers.

Dossier Limitations



N=197

Feedback/Suggestions for Manufacturer

N=88

- Be more direct about the statements you are trying to make
- Make more clear and concise statements
- Charts and tables make information easy to read so that is always appreciated

- Need cost-effectiveness analysis compared to similar products in class
- Include real world data
- Include guidelines for ALL indications, not just new ones
- Summary of place of therapy, efficacy and safety with cost info by indication in one table/page

- Pre-launch version could be helpful
- Make available sooner
- None, it is in the standard dossier format
- It needs to be updated faster!
- Please have this available sooner through the AMCP eDossier System

Environmental Scan – Payer Respondents

- Key takeaways:
  - Payers are using dossiers for product reviews and overall are satisfied with the dossiers.
  - Payers appreciate when dossiers are provided in the standard format.
  - Payers identified these important factors for manufacturers to consider when preparing dossiers: be concise, make dossiers available sooner and update faster.

Pre-and Post-Approval Dossiers: Making All Evidence Count

The Payer Perspective

Sina Carlson
Senior Director, Drug Intelligence
OptumRx

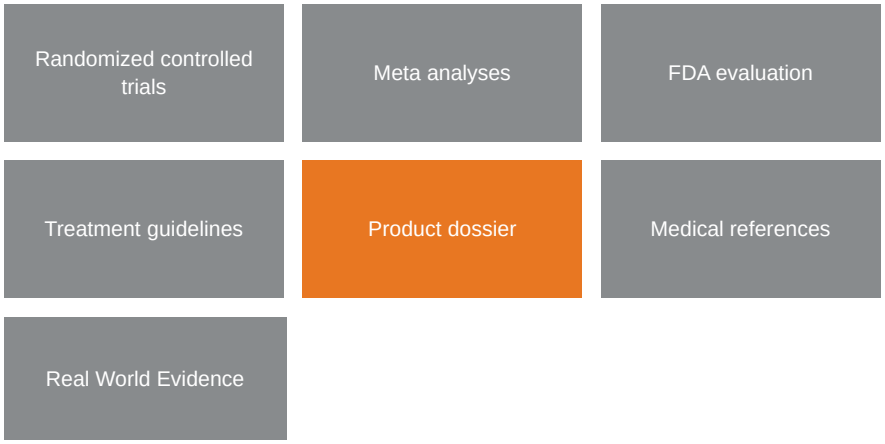
Drug Evaluation Philosophy

Looking at drug selection from all perspectives

Total health care value

Clinical Efficacy	Cost Savings	Choice
• FDA-Approved Indications and Dosing. • Potential Side Effects. • Drug Interactions. • Drug-Disease Interactions. • Comparative Clinical Trials.	• Average Wholesale Price. • Rebates. • Ingredient Cost. • Cost of Care. • Copayments. • Coinsurance. • Generic Pipeline.	• Market Factors. • Customer Impact. • Regulatory Restraints. • Overall book of business. • Number of Equivalent Alternatives in Class. • Number of Indications Treated.

Dossiers In Drug Evaluation Process



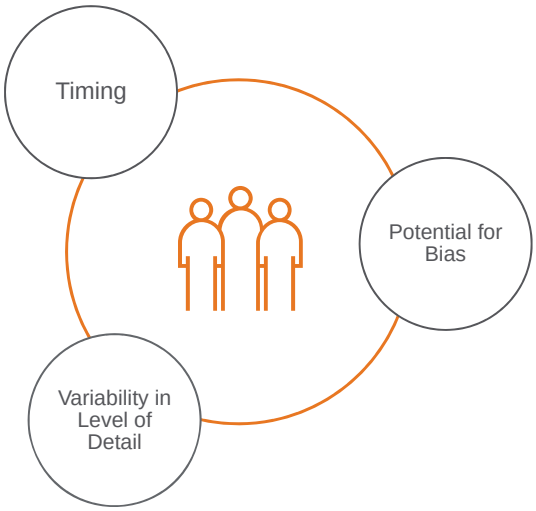
Dossiers In Drug Evaluation Process

Dossier: Benefits

1. Clinical trial information, especially where unpublished
2. Disease state, epidemiology
3. Drug comparison tables
4. Pipeline/ potential future indications
5. Clinical Guidelines

OPTUM Rx

Dossier: Limitations



OPTUM Rx

Dossier: Future Considerations

Pre-approval Package

More timely release upon approval

Clinically meaningful outcomes
Including comparisons to other drugs

Link references to PubMed locations

OPTUM Rx®

OPTUM Rx®

Sina Carlson

Senior Director, Drug Intelligence

Tel: 516-232-1830

OPTUM Rx

Pre- and Post-Approval Dossiers: Making All Evidence Count

Industry Perspective

Iris Tam, PharmD, FAMCP

Disclosure and Disclaimer

- I am an employee and stockholder of Achaogen.
- I am the current Chair of the AMCP Format Executive Committee, and have been an official member of this committee since 2010.
- The views and opinions expressed in this presentation are my own.

Outline

Industry Perspective

- Dossier development and maintenance
- Responding to requests for dossiers
- Adhering to AMCP *Format for Formulary Submissions,* V4.0
  - Including the concept of “Pre-approval dossiers”

Industry Perspective: Dossier Development & Maintenance

- Created by appropriate medical and technical personnel
- Vendors and consultants contracted to develop dossiers
  - Experience, expertise, costs
- Review and approval processes vary per company policies
  - Medical, Legal, Regulatory, other
- Update regularly and/or when significant information available

Industry Perspective: Responding to Requests for Dossiers

- Dossiers are provided upon an unsolicited request
- Defining and screening eligibility of requesters/recipients
- Requiring confidentiality agreement
- Providing PDF vs Word document versions
- Providing peer-reviewed articles
- Adopting AMCP eDossier System platform

Industry Perspective: Adhering to AMCP *Format*

- Creating pre-approval dossiers

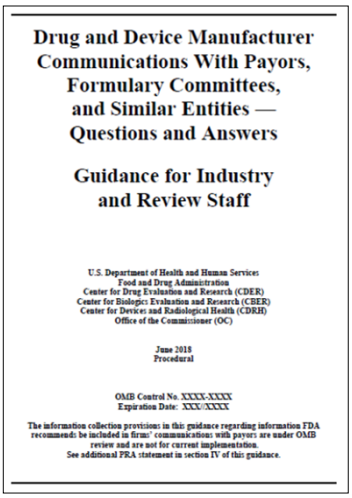
AMCP *Format*: Dossier Information Before FDA Approval

- 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

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 Payors

- 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 payors 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.

Unapproved Product Information

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

Industry Perspective: Adhering to AMCP *Format*

- Creating pre-approval dossiers
- Adhering to number of pages
- Inclusion of large number of studies
- Handling on-label vs off-label content
- Deciding between study summaries vs evidence tables
- Developing and providing economic models
  - Cost-effectiveness analysis (CEA) model
  - Budget impact model (BIM)

Educational Opportunity

- Loretta Peters, MBA
- (520) 626-5883
- training@pharmacy.arizona.edu

AGENDA

Thursday, November 1, 2018
NOON - 5:30 pm
Lunch Provided

AMCP Format for Formulary Submissions: Contents, Availability, Process
Iris Tam, PharmD, FAMCP

The Good, The Bad And The Ugly
Dan Malone, PhD, RPh, FAMCP

Clinical Evidence Summaries
Iris Tam, PharmD, FAMCP

Friday, November 2, 2018
8:00 am - 5:00 pm
Breakfast & Lunch Provided

Economic Models and Humanistic Studies
Dan Malone, PhD, RPh, FAMCP

AMCP eDossier System Demonstration
Allen Lising

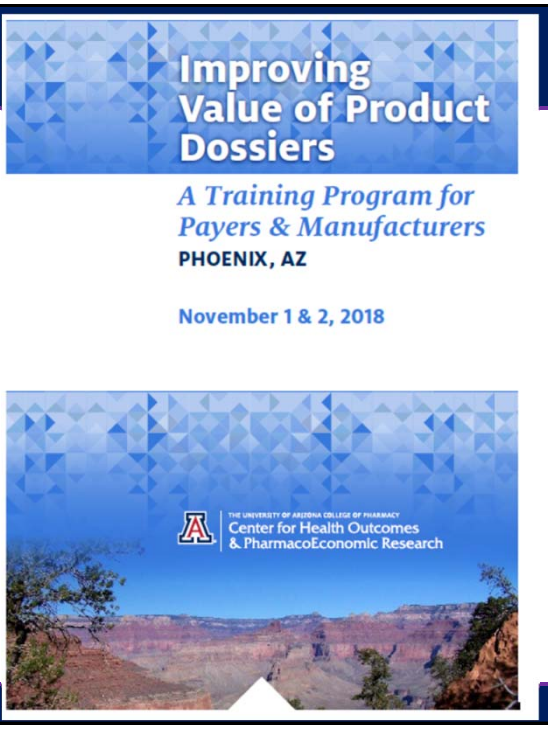
Managed Care Perspectives on Use of Dossiers
Steve Avey, MS, BS, FAMCP

Manufacturer Considerations Pertaining to Dossiers and Pre-Approval Information
Iris Tam, PharmD, FAMCP

Dossier Critique
Dan Malone, PhD, RPh, FAMCP

Dossier Roundtable

Optional Confidential Dossier Review



Questions?

Thank you!

Iris Tam
itam@achaogen.com
(415) 370-0829

Summary

- Payers are using dossiers for product reviews and overall are satisfied with the dossiers, but they prefer dossiers earlier.
- Dossiers are primarily being used as a secondary resource and/or to validate their own research for decision making.
- From the industry perspective, dossiers allow a way to share objective and thorough evidence with payers.
- AMCP *Format for Formulary Submissions* v4.0 is the one unifying guidance for communication between formulary decision-makers and industry.

Discussion & Questions

Thank you for participating!

Payers:
For further information on the AMCP eDossier System contact esampsel@dymaxium.com

Manufacturers:
For further information on AMCP eDossier System or FormularyDecisions.com subscriptions contact Lfazio@dymaxium.com

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