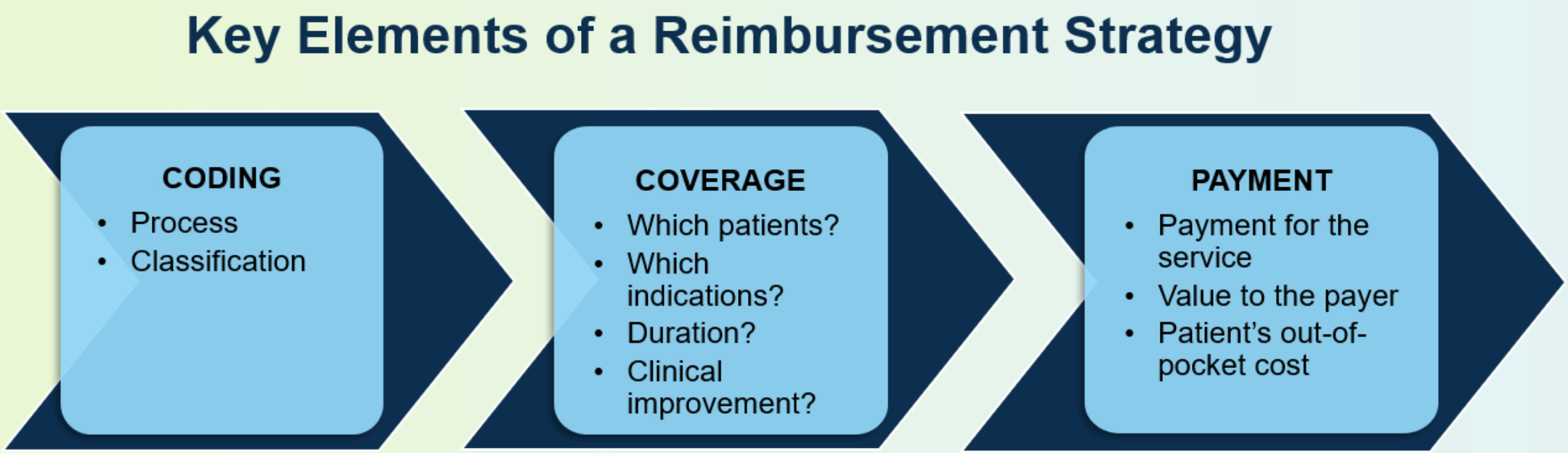
Small Molecule Drug Reimbursement Case Study

PHARMACON Company

Reimbursement Overview

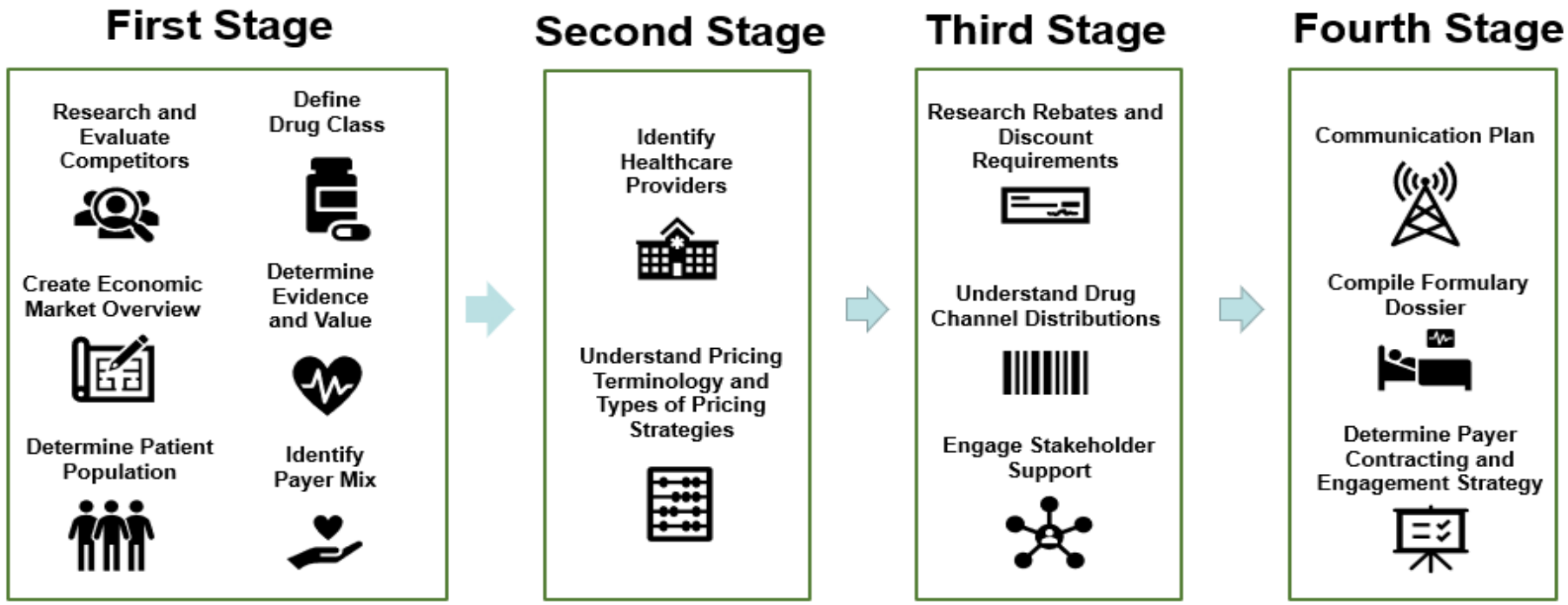
Bringing a drug product to market requires many skills sets. An innovator needs to understand the entire commercialization process and manage multiple tasks related to early-stage research and development, clinical trials, regulations, reimbursement and post-market surveillance. The goal of receiving U.S. Food and Drug Administration (FDA) approval is often considered the primary endpoint that leads a new drug to commercial success. However, if a new drug does not obtain the desired amount of reimbursement or, even worse, is not covered by payers, then physicians are unlikely to recommend and prescribe the new drug. Therefore, ensuring reimbursement for a new drug is as important as obtaining regulatory approval.



Reimbursement Strategy Activities Roadmap

This case study breaks down the process described in our Reimbursement Knowledge Guide for Small Molecule Drugs. It will take you step-by-step through a process innovators may follow to develop a reimbursement strategy for a drug. We'll walk through each phase from the innovator's point of view. Aspects of the process may be conducted together, roughly in tandem. The icons below are shown on the top right of each slide to help you follow the process.

Initiates 24 - 36 months prior to FDA approval



Introduction to PHARMACON and Hebdomadal

Ginger Smythe is the CEO of PHARMACON. She is working with Sophia Garcia, the principal investigator, on developing a small molecule drug called Hebdomadal that may be easier to take than other cholesterol lowering drugs, thereby improving patient adherence and producing better health outcomes. Ginger and Sophia are optimistic that Hebdomadal will improve health outcomes, and they know reimbursement will be critical for successfully taking the drug to market.

What does Sophia need to do during product development to ensure optimal reimbursement?

Product Description:

- PHARMACON developed Hebdomadal, a new small molecule drug that may be easier for patients to take compared to other cholesterol lowering drugs. It is intended for adults with heterozygous familial hypercholesterolemia (HeFH) or clinical atherosclerotic cardiovascular disease (ASCVD), who require additional lowering of low-density lipoprotein-cholesterol (LDL-C).
- Hebdomadal is a once-weekly, controlled-release, long-acting oral drug to lower cholesterol for those patients with HeFH or ASCVD and high levels of LDL-C
  - 190 mg/dL or higher without other risk factors
  - Higher than 160 mg/dL with another major risk factor
  - Above 130 mg/dL with two risk factors

Why Hebdomadal?

- High LDL-C contributes to plaque build-up which can lead to arterial clots forming and cause heart disease and strokes.
- Since it treats a chronic condition, convenience and efficacy will be of value to patients, so a once-weekly drug may be a big hit in the market.

First Stage

- Define Drug Class
- Identify Competitors
- Create Economic Market Overview
- Determine Evidence and Value
- Determine Patient Population
- Identify Payer Mix

Second Stage

- Identify Healthcare Providers
- Understand Pricing and Types of Pricing Strategies

Third Stage

- Research Rebates and Discount Requirements
- Understand Drug Distribution Channels
- Engage Stakeholder Support

Fourth Stage

- Create Communication Plan
- Compile Formulary Dossier
- Determine Payer Contracting and Engagement Strategy

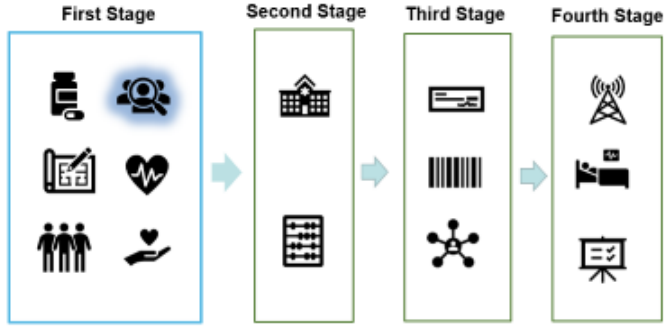
First Stage: Identify Competitors

Sophia believes Hebdomadal, a new chemical entity, as an oral, once-weekly, controlled-release, long-acting cholesterol drug, will be a first-in-class drug. She is eager to assess the company's potential competitors, their market share and product positioning.

Sophia starts by researching FDA databases for similar cholesterol lowering drugs for any potential comparators, noting that a comparator for reimbursement analysis does not have to be a small molecule drug. She confirms that Praluent and Repatha are good comparators for her reimbursement analysis. Because they are injectable drugs with a high-risk for allergic reactions under the skin (i.e., subcutaneous) at the injection site, Hebdomadal may be more convenient and tolerable in comparison.

She realizes that for regulatory purposes, comparing a small molecule to a biologic may not be a good idea. However, her drug will likely have a lower list price, so it makes sense from a reimbursement perspective to look at comparators that offer the similar cholesterol lowering benefits, especially if they do so at a much higher cost.

In addition, an oral drug could be a differentiating factor as many patients may prefer to take an oral drug rather than an injection. Her small molecule drug may disrupt the status quo and even take market share away from the biologics mentioned earlier.



Key questions:

- What are the current cholesterol-lowering products on the market that could be considered comparators?
- What are the key attributes of the potential comparators as it relates to administration, e.g., oral versus injectable?

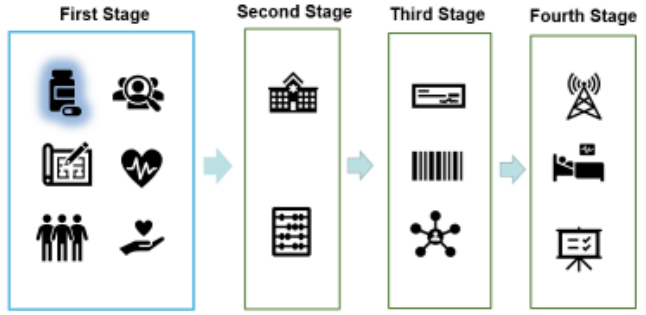
First Stage: Define Payer Drug Class

Sophia needs to better understand the drug classification system and how Hebdomadal's competitors are classified.

The United States Pharmacopeia (USP) Organization creates drug formularies. A drug formulary is a list of brand-name and generic prescription drugs approved to be covered by a particular health insurance plan or in a specific health system or hospital. They are developed based on the efficacy, safety, and cost of the drugs. Formularies consist of therapeutic categories and drug classes used by many different types of payers to define what drugs are covered by their specific plans.

Sophia starts by researching the competitors and how these drugs are classified. She learns that two drugs (Praluent and Repatha) are in a unique drug class (PCSK9 inhibitors) created by the USP in 2017. Even though both Praulent and Repatha are biologics, they are used to treat high LDL-C cholesterol in adults with HeFH or ASCVD so they would be an appropriate comparator drug to Hebdomadal.

Sophia realizes that because Hebdomadal is a small molecule drug it may be considered a first-in-class, unique drug. This designation may require additional evidence to support its inclusion in a formulary. She also learns that the USP convenes stakeholder forums to discuss new products and how they might be classified after the drug is launched.



Key questions:

- How are the current cholesterol-lowering products on the market classified?
- How would this drug be classified?
- What organization determines the drug class?
- Can we meet with the FDA and/or the organization to discuss our product?

First Stage: Create Economic Market Overview

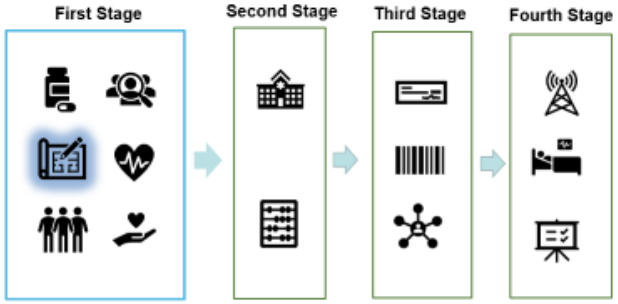
Sophia needs to gather evidence to support a reimbursement dossier for Hebdomadal, so Ginger recommends Sophia work with PHARMACON's reimbursement consultant Deepa. Deepa's expertise will help them set a launch price and frame discussions with payers and other stakeholders to get Hebdomadal added to formularies.

Sophia and Deepa start by researching journals, competitor annual reports, and data analyst reports/websites.

- They find that revenue projections and pricing vary greatly depending upon the brand/generic, administration method, and dosing interval of products.

Deepa explains to Sophia that coverage and reimbursement of drugs are determined by each payer's coverage policies.

- Payers may require different types of information to determine payment for a new drug. This information is generally compiled and presented to a payer in a dossier (see "Fourth Stage: Compiling a Formulary Dossier). A dossier contains comprehensive and concise information about clinical and economic evidence, the approved indication(s) and data sources. The reimbursement dossier serves as a continuously evolving source of objective, credible, and relevant information during the development and commercial life cycle of a drug product.



Key questions:

- Has a market share and economic assessment been created?
- Is a necessary to hire a reimbursement consultant? What information would a reimbursement consultant or pricing expert need to know about the product?
- What evidence is required to develop a value story?

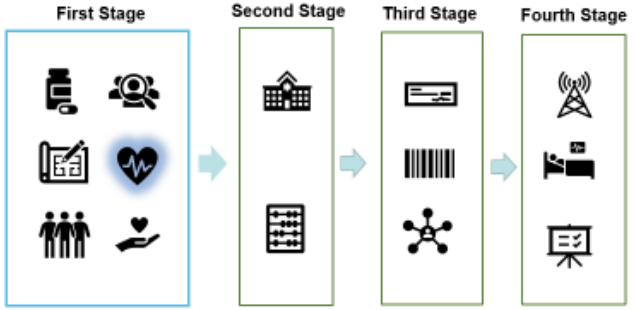
First Stage: Determine Evidence and Value

Sophia and Deepa gather evidence to support the dossier and illustrate the value of prescribing in favor of Hebdomadal over competitors.

Using research journals, competitor annual reports, FDA drug reports, and clinical trial websites they collect clinical data about their competitors' performance to support the added value of Hebdomadal compared to existing drugs.

- Their research indicates the results of the Phase III clinical trials are critical not only for FDA approval, but also to position Hebdomadal well for payer coverage and reimbursement. Critical information may include how Hebdomadal addresses treatment failures in the context of the other competitors and how the drug works in sub-populations that are of interest to specific payers. This information will help get the drug on formularies, which will in turn support the case for reimbursement coverage and payment.
- Sophia realizes that PHARMACON will need to plan to collect additional specific clinical and economic evidence during their Phase 3 clinical trials (beyond the FDA required safety and efficacy outcomes) to support the claim that Hebdomadal's once-weekly oral dosing has value for the payer and the patient.

Deepa explains that the market uptake, payer, and health providers' acceptance of a new drug can be slow, especially if the drug is considered expensive.



Key questions:

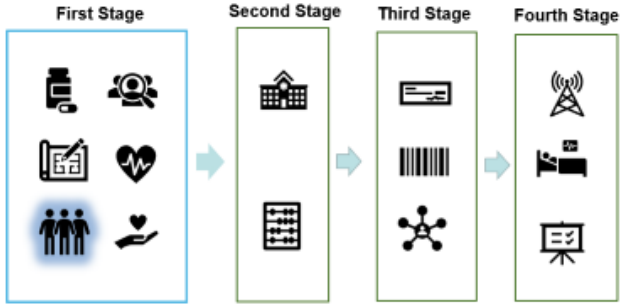
- What research would uncover potential evidence necessary for approval?
- Is there evidence in peer-reviewed literature that the drug has value for a specific population?
- What other reports or websites could yield information?

First Stage: Determine Patient Population

Sophia knows that defining the target population is one of the key factors in identifying the primary payers. Information about the patient population also helps the team define which payers to approach first.

Sophia again uses peer-reviewed literature to estimate the size of the target market.

- Hebdomadal is being developed for adults with HeFH or ASCVD, who require additional lowering of LDL-C.
- Several studies show greater than 40% of patients prescribed a daily cholesterol-lowering drug are not adherent (non-compliant). According to the studies, patients forget to take it every day and/or the out-of-pocket cost is too high. Having a once weekly oral drug at a reasonable price may help improve compliance. Post-market studies will be key in determining the real value of an oral, once weekly cholesterol lowering drug.
- She confirms that patients with HeFH and ASCVD can reduce their risk for coronary heart disease and reduce the risk of heart attack and stroke by lowering their LDL-C. This information will help Sophia's team define which payers to approach first. She believes that further demographic analysis (e.g., age, gender) of this population will help the team more narrowly define the target population.



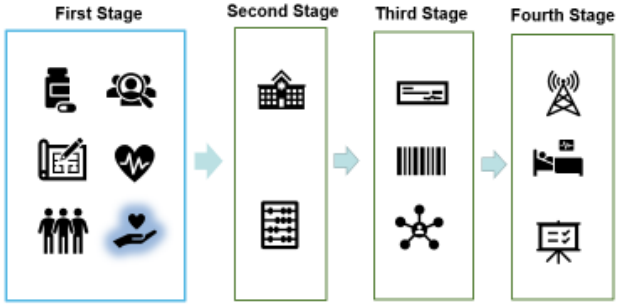
Key questions:

- What patients would benefit most from this drug?
- What is the target population?
- What analysis or study could assist in further defining the target population?
- Would post-market studies be helpful?

First Stage: Identify Payer Mix

Sophia needs to understand what each type of payer will be looking for regarding value and pricing.

- Because Hebdomadal is for adults, it will be considered by multiple payers, including Medicare Part D plans, Medicaid state programs, and commercial insurers).
- Sophia asks Deepa how the different payers impact the reimbursement strategy. Deepa explains there are multiple pricing terminologies used by each payer type and that PHARMACON will need to provide different business plans for each payer, including information on pricing, rebates, contracts, discounts, etc.
- Payers use a Pharmacy & Therapeutics (P&T) Committee to review new drugs for coverage and inclusion/exclusion on their formularies. The P&T Committees also develop or recommend criteria for utilization tools and formulary controls (e.g., prior authorization, step therapy). Sophia determines additional research of the two competitors will be necessary to better understand potential restrictions that might be placed on the product.
- Sophia meets with Ginger and PHARMACON's leadership team to review their staffing plan and discuss hiring an external team of account managers and clinical experts to meet with P&T Committees and develop materials (including pricing proposals) for the reviews.



Key questions:

- What is a P&T Committee's role by a payer?
- Who engages with a P&T Committee?
- What are utilization tools/formulary controls used by payers?

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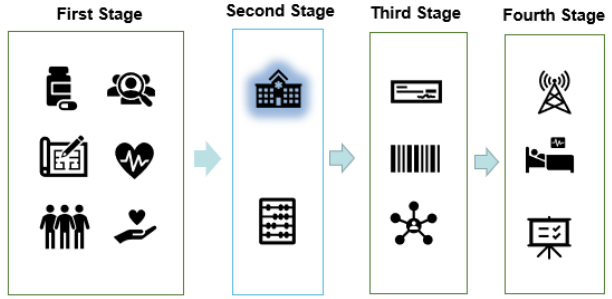
Second Stage: Identify Healthcare Providers

Sophia needs to identify the types of healthcare providers who would prescribe Hebdomadal for adults with HeFH or ASCVD and high levels of LDL-C with the goal of reducing LDL-C to a normal level.



Because Hebdomadal is for adults, Sophia needs to know what kinds of providers will prescribe the drug and what type of clinical evidence each might require. She meets with clinical professionals, research scientists, medical directors, pharmacists, and nurses to determine which providers to target. She learns the most likely prescribers will be:

1. Cardiologists
2. General practitioners (usually in conjunction with a specialist)
3. Specialists in cholesterol (lipid) clinics

Research of physician-specific data and analysis, from sources such as IQVIA – Physician Insights 360™ or Definitive Healthcare, can identify a list of high-prescribers of cholesterol drugs from the target patient demographic analysis performed earlier.

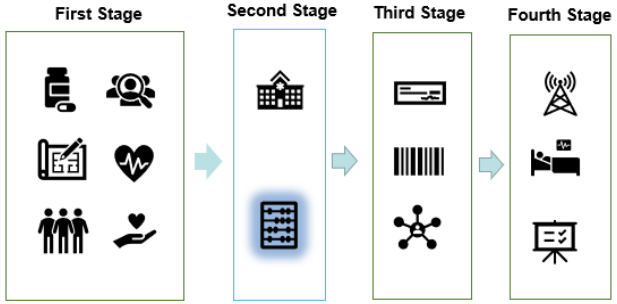
PHARMACON's team determines providers may be interested in reviewing additional published information prior to adopting Hebdomadal, such as peer-reviewed study results (e.g., randomized-controlled trials) and various clinical journals (e.g., Journal of the American College of Cardiology).

Key questions:

- What types of healthcare providers would be interested in knowing more about this drug product?
- What evidence might be required for different types of providers?

Second Stage: Overview of Drug Pricing

Sophia knows selecting the "right" launch price will influence uptake by prescribers, sales, and, ultimately, PHARMACONs' return on investment. The launch drug price should reflect both uniqueness of the drug and its efficacy, especially if clinical data shows a marked improvement over existing comparator drugs.



Like other goods bought and sold in the U.S., the list price of prescription drugs depends on a combination of:

- Supply and demand
- Marketing considerations
- R&D costs
- Manufacturing and production costs

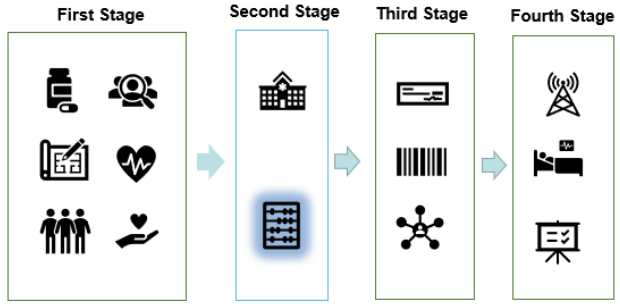
When researching pricing strategies, Sophia learns she should prioritize Hebdomadal's uniqueness and effectiveness as compared to similar products from other companies. Setting an appropriate launch drug price is essential. Too high a price can impede market adoption. Too low and PHARMACON may not recoup its R&D investment, cost of goods, and production costs. Although price adjustments will happen, there is only one chance to make a favorable first impression.

Key questions:

- How do pharmaceutical companies price a drug?
- What are the types of information used to determine a list price for launch?

Second Stage: Drug Pricing Strategies to Consider

Sophia explores pricing strategies for setting Hebdomadal's launch price. Ideally, Hebdomadal is included on payer's formularies. She knows that inclusion on a lower tier of a formulary usually translates to lower out-of-pocket costs to patients.



Value-Based Pricing – uses a blend of product attributes, costs, profit targets, and pricing, to meet the needs and desires of a payer customer.

Two common approaches are:

1. Understand the perceived value of the product by key stakeholders, e.g., prescribers. Use this data to set a premium price.
2. Use pharmacoeconomic data to calculate the potential overall cost-savings for a health plan/payer.

Competitor-Based Pricing – is a component of value-based pricing that benchmarks pricing against current competitors to assess whether a new product has perceived relative value.

Commercial Payer Considerations for Evaluating Potential Pricing of Hebdomadal

- The P&T Committee evaluates Hebdomadal for possible inclusion on their formulary.
- They review all the published clinical studies, competitor pricing, and the utilization tools used to control access. If it is clear Hebdomadal may provide added benefits for the patient, it may result in preferred status for the drug.
- If little to no benefit is seen, then achieving preferred status is often costly, taking the form of rebates, discounts, or utilization controls. Non-preferred status generally costs the patient more, e.g., higher out-of-pocket costs.

Key question:

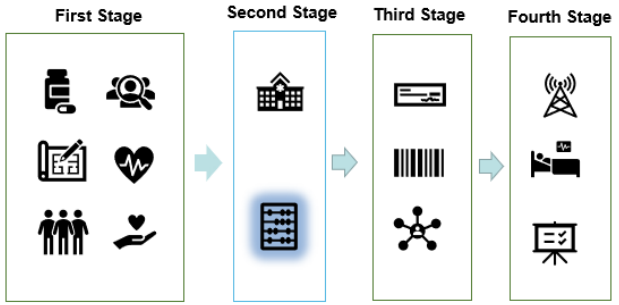
- What does PHARMACON need to consider when setting the launch price for Hebdomadal?

Second Stage: Setting a Launch Price for the Drug

Now that Sophia understands competitor-based pricing is a suitable strategy for Hebdomadal, she works with Deepa to identify an initial list price/launch price. Deepa emphasizes Hebdomadal's price should consider both competitor pricing and the added value to patients provided by the new product.

They start by researching the price of Hebdomadal's competition, Praluent and Repatha (see First Stage: Identifying Competitors). They find the drugs' wholesale acquisitions cost "WAC" (the manufacturer's list price without discounts or rebates) on the companies' websites and adjust the list price to align the dosing frequency with Hebdomadal (once a week): Praluent costs $132 per week and Repatha costs $127 per week.

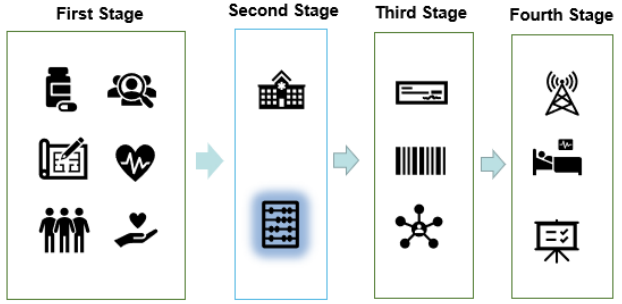
However, Deepa cautions Sophia not to make a direct cost comparison between these three drugs because Praluent and Repatha (PCSK9 inhibitors) are valued differently. Current reports from the Institute for Clinical and Economic Review found that the cost of these biologics exceeds the typical U.S. threshold for cost-effectiveness. The breakeven point is estimated at $11.54 per week. Further, a Johns Hopkins study suggests that PCSK9 inhibitors do not add value to the U.S. health system, and their provision is not profitable for private payers. Overall, the PCSK9 inhibitors are more expensive and inconvenient (i.e., injectable) while Hebdomadal – a small molecule drug – is notably less expensive to produce and more convenient (i.e., oral) to take.



Key question:

- How might PHARMACON go about estimating an initial price for Hebdomadal?

Second Stage: Setting a Drug Launch Price (continued)



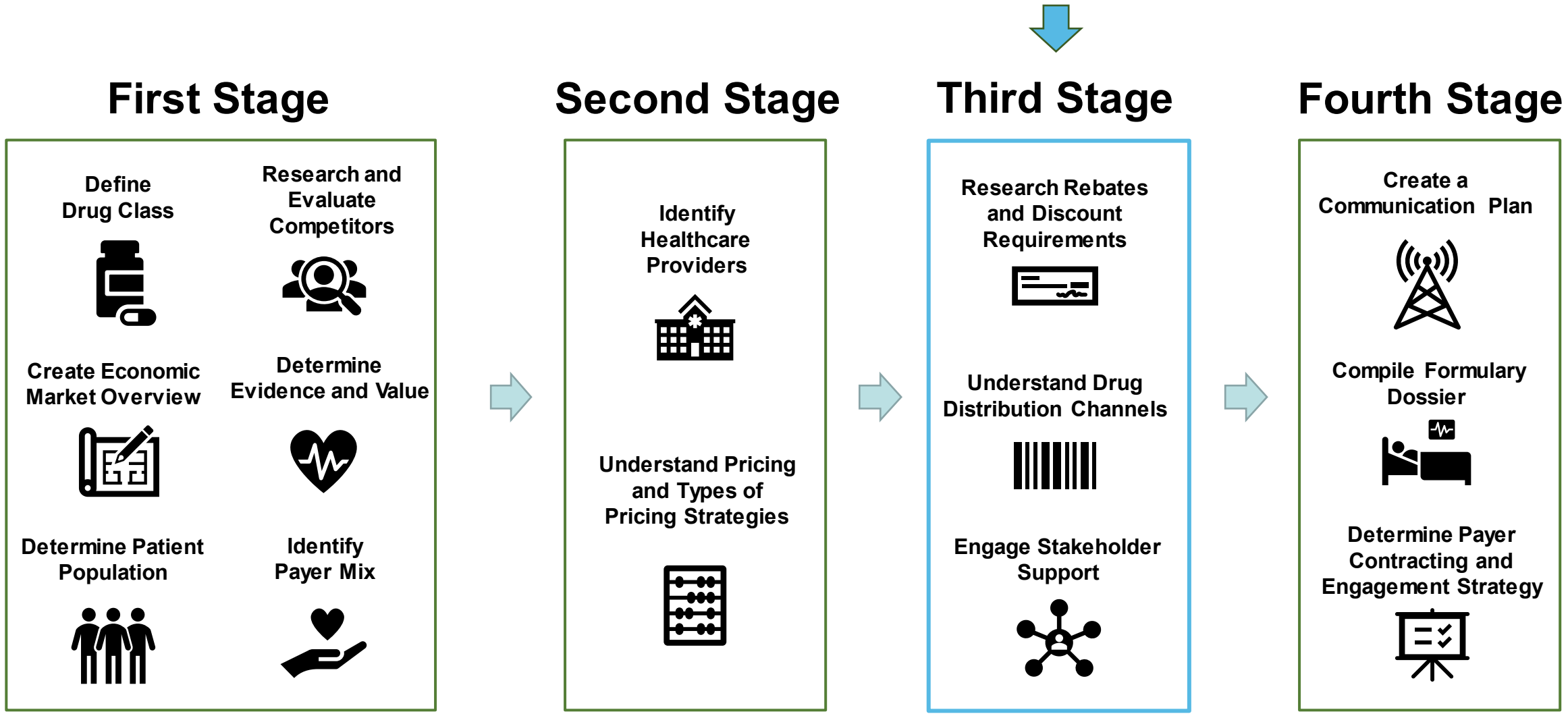
Sophia and Deepa continue to assess market conditions for a new oral LDL-C drug for adults with HeFH or ASCVD who require additional lowering of LDL-C.

They know that the data from their Phase III trial shows Hebdomadal to have similar impact of lowering LDL-C levels as the PCSK9 inhibitors. Their drug provides higher value because it is less expensive to develop and more convenient for patients than its biologic competitors. Since they already established that the PCSK9 inhibitor's minimum cost is $127 per week and that the $127 is not considered cost-effective for payers, Deepa advises Sophia that the launch price of Hebdomadal should be the breakeven point of the biologic competitors. This would position it well to gain market share. Overall, the launch price should reflect the R&D, manufacturing, production, and marketing costs as well an appropriate profit margin.

Since Hebdomadal offers added value to patients with a clinical improvement in outcomes, fewer adverse events, less dosing frequency (once a week), and easy administration (oral), Deepa suggests that Hebdomadal's benefits and added value to patients be reflected in the launch price. Based on this assessment, they make the strategic decision to use the breakeven point (of the biologic competitors) as the initial list price of $11.54 per week.

Key questions:

- Would patients have better outcomes on Hebdomadal over the comparator biologics? How do you determine the value of the better outcome?
- What would be a good launch price for Hebdomadal?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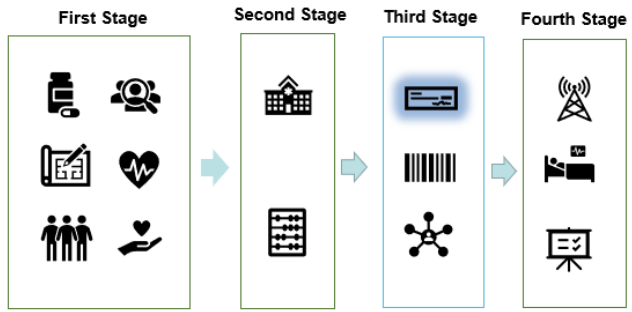
Third Stage: Research Drug Rebates and Discounts

Deepa explains how rebates and discounts are used to add new drugs to payers' formularies. Most rebates are either negotiated through Pharmacy Benefit Managers (PBMs) or required in government programs (e.g., Medicaid Drug Rebate Program).

Sophia learns that prescription drug rebates have been used for many years. Drug rebates often depend on whether the payer is a government (e.g., Medicare) or commercial entity. Not all drugs have rebates – instead of a rebate a manufacturer may lower the drugs list price to encourage sales.

Her research shows that the number of drugs available to treat cholesterol has grown. She realizes that since the drug class for cholesterol lowering drugs has grown, higher rebates or discounts may be necessary to support her product's value to get it added to the payers' formularies in a favorable position.

She also learns that PBMs negotiate rebate agreements with drug manufacturers on behalf of payers. The desired result of these negotiations is drug placement on the preferred tier of payers' formularies, which can translate into more drug sales and benefits the manufacturer's bottom line.



Key questions:

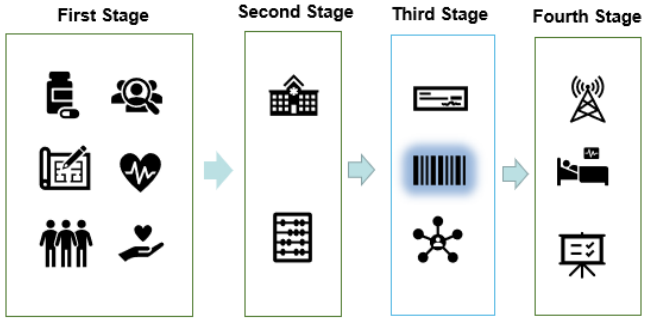
- What are rebates? What type of drugs have rebates?
- Will the payers require rebates and/or discounts to be paid for adding Hebdomadal to the formulary?
- Are rebates optional? Or do some payers require rebates to be paid? If so, what payers?

Third Stage: Type of Brand Drug

Deepa explains to Sophia that new drugs can be defined as either a specialty brand drug (high cost, high complexity, high touch, e.g., injectable), or as a brand/generic drug (treats general health and chronic conditions).

To find out whether the health plans will consider Hebdomadal to be defined as a specialty brand drug, or as a brand/generic drug, Sophia may need to start meeting with health plans/payers and possibly host some payer advisory boards to gain some real-life perspectives. Because Hebdomadal is a small molecule drug that treats a common condition (high LDL-C), it is likely that it would be defined as a non-specialty brand drug. This means:

- For patients under payer plans, PHARMACON would negotiate with PBMs and placement on their preferred formulary tier would be key for improving utilization and access for patients. The patient's out-of-pocket costs (e.g., copay, coinsurance) would be determined by the health plans.
- Hebdomadal's inclusion on Medicare Part D plan formulary lists would be critical to ensure Medicare enrollees have access to Hebdomadal. For patients to have access to a new drug covered under Medicare Part D, CMS requires evidence in the Medicare population of safety, effectiveness, appropriateness, and comparative benefit.

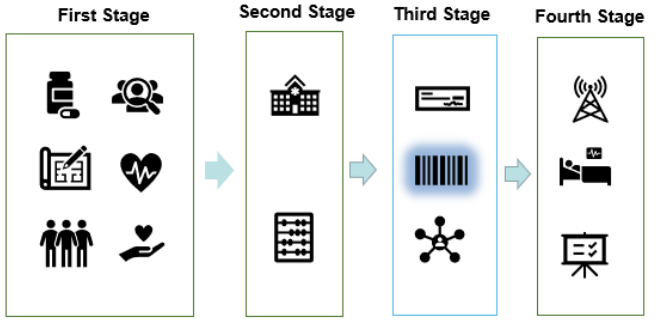


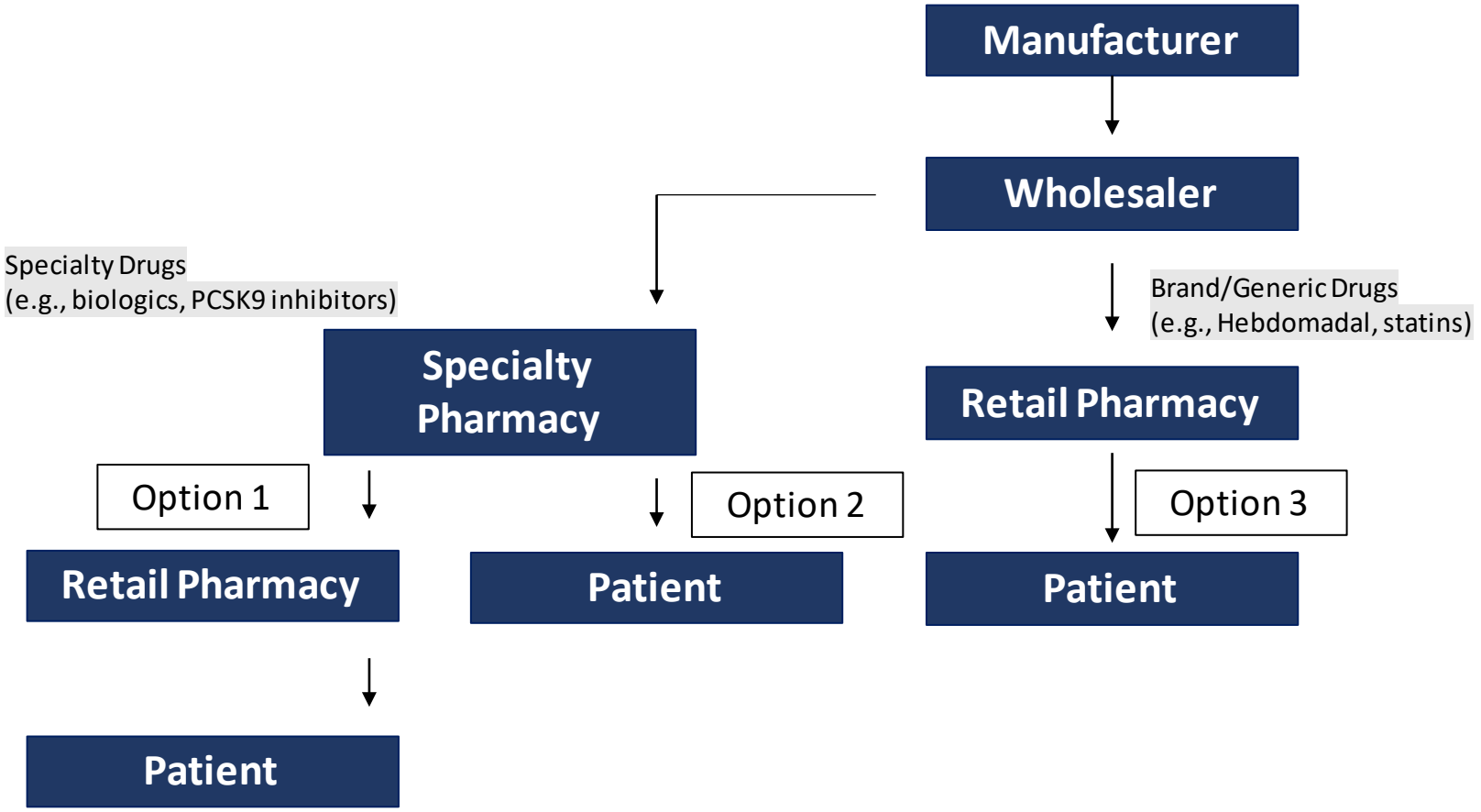
Key questions:

- Will Hebdomadal be categorized as a specialty brand drug?
- Does a favorable formulary position provide better access for patients? What factors influence formulary placement?

Third Stage: Drug Distribution Channels

Sophia needs to understand how a drug flows through the distribution channels, starting with the manufacturer and ultimately reaching the patient. In this scenario, Hebdomadal as a brand drug would follow option 3.



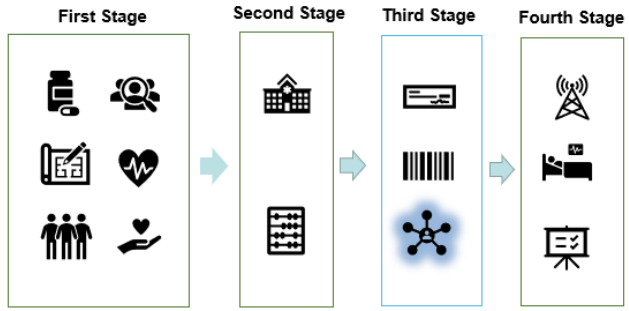


Key questions:

- How does the drug ultimately get to the right patient from the manufacturer?
- What entities are involved in this process?

Third Stage: Engage Stakeholder Support

Sophia knows engaging stakeholders throughout the research and development of a new drug is of utmost importance. She wants to better understand how to engage stakeholders regarding reimbursement and pricing.



Sophia begins developing a stakeholder engagement plan. This type of plan will require coordination across the organization involving multiple internal functions, as well as identifying key external stakeholders.

Sophia starts the process by choosing a project management team to coordinate and oversee the stakeholder engagement plan. It will be important to have direct interaction with the stakeholders and consistency of messaging to build the foundation for a lasting relationship between her company and external stakeholders.

Some of the internal stakeholders will include clinical, regulatory, commercial, marketing and senior leadership. One of the key members will be PHARMACON's Medical Affairs (MA) director, Tomaz, who will partner with both internal and external relevant stakeholders, to promote collaboration and effective messaging. MA teams often engage with payers and formulary committee members to deliver the value story about the new drug.

Sophia should consider hosting patient/provider focus forums and Advisory Board meetings with payers and key opinion leaders to help validate conclusions and plans going forward.

Key questions:

- What type of plan should be developed to ensure consistency and collaboration?
- Who should lead the stakeholder engagement process?
- What teams and/or internal stakeholders should participate?
- Who will engage external stakeholders, i.e., payers?

First Stage

- Define Drug Class
- Research and Evaluate Competitors
- Create Economic Market Overview
- Determine Evidence and Value
- Determine Patient Population
- Identify Payer Mix

Second Stage

- Identify Healthcare Providers
- Understand Pricing and Types of Pricing Strategies

Third Stage

- Research Rebates and Discount Requirements
- Understand Drug Distribution Channels
- Engage Stakeholder Support

Fourth Stage

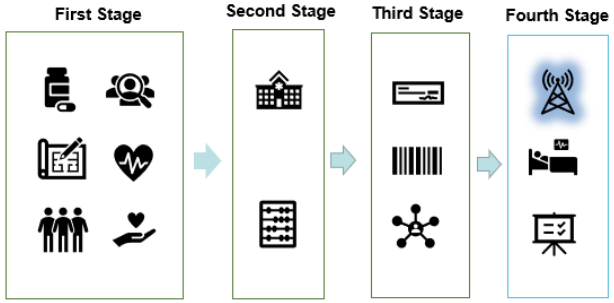
- Create a Communication Plan
- Compile Formulary Dossier
- Determine Payer Contracting and Engagement Strategy

Fourth Stage: Create a Communications Plan

Sophia develops a communications plan to inform stakeholders of Hebdomadal's attributes to garner support, drive market adoption, and receive optimal coverage.

Sophia needs a **c**ommunications plan to differentiate Hebdomadal and incentivize stakeholders to switch from other approved drugs. She develops targeted messaging for:

- **Cardiologists, Endocrinologists, Internists:** Sharing cost-benefits information on using Hebdomadal over current market leaders will be crucial. Talking points about its value (once a week oral drug) will be essential in identifying physician champions. Ginger plans to utilize her board members' existing relationships for warm introductions. Her goal is for Tomaz, PHARMACON's medical affairs director, to talk peer-to-peer about Hebdomadal's benefits over existing treatment options.
- **Professional Medical Societies:** Working with medical societies (e.g., American College of Cardiology, American Association of Clinical Endocrinology) to create provider educational materials. Convene with payers to drive adoption.
- **(Commercial) Payers:** Discuss favorable CMS coverage determinations in connection to evidence gaps (as described in commercial payers' policies) to understand specific coverage requirements. Highlight quality of life benefit (oral drug vs. an injection) as a differentiator.
- **Patient Advocacy Groups:** Integrating the voice of the patient will also be crucial in conversations with physicians and advocacy groups.



Key questions:

- What steps need to be taken to ensure a successful communications plan?
- Should publications and conferences be part of the communications plan?
- How and when do you distribute marketing materials?
- How often should the plan be updated?

Sample Communication Plan for Launch

Who is the target audience for Hebdomadal?

- Adults with HeFH or ASCVD and high LDL-C defined as
  - >190 mg/dL without other risk factors
  - >160 mg/dL with one other major risk factor
  - >130 mg/dL with two other risk factors
- Cardiologists, endocrinologists, internists, and other health practitioners

Why is Hebdomadal important? What is its market differentiator?

- Lowers LDL-C in patients with HeFH or ASCVD
- Significantly lower cost than biologic comparators (e.g., PCKS9 inhibitors)
- Improved quality-of-life: once a week oral drug in comparison to its two closest competitors (Praluent and Repatha) which are injectable biologic drugs with a high-risk for allergic reactions at the injection site

How will information about Hebdomadal be disseminated?

- Social media, print media, and television campaigns
- Peer reviewed journal article(s) summarizing clinical trial results
- Presentations at medical society meetings and conferences

Note: Develop budget for marketing activities and track the return on investment for each

https://www.ncbi.nlm.nih.gov/books/NBK459188/

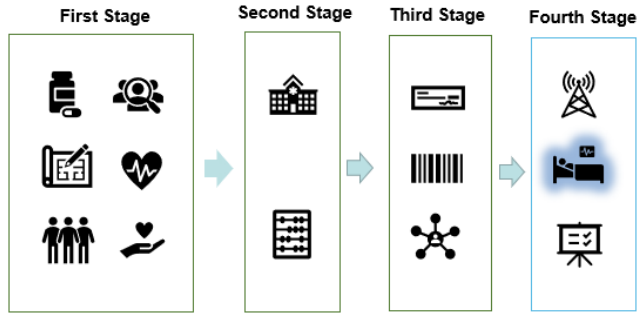
Fourth Stage: Compile a Formulary Dossier

Sophia understands that payers and formulary review committees want to receive information on new drugs in specific formats. She reaches out to Tomaz to coordinate the development of the formulary dossier.

Tomaz explains to Sophia that the AMCP (Academy of Managed Care Pharmacy) formulary dossier is the framework used by virtually all U.S. healthcare payers to evaluate adding drugs to their formularies.

It is critical that the Hebdomadal formulary dossier has the "right information" and the "right sources." This includes:

- Real-world data, comparative effectiveness data, and concise summaries of the targeted disease and populations
- Clinical studies (on efficacy and safety) as well as any peer-reviewed publications that support the unmet need for this drug
- Compliance and adherence studies are important to support the dosing schedule and price for the product



Key questions:

- What is a formulary dossier? What information is contained in it?
- Who is interested in receiving and reviewing this information?
- What team will communicate the dossier information and address questions?

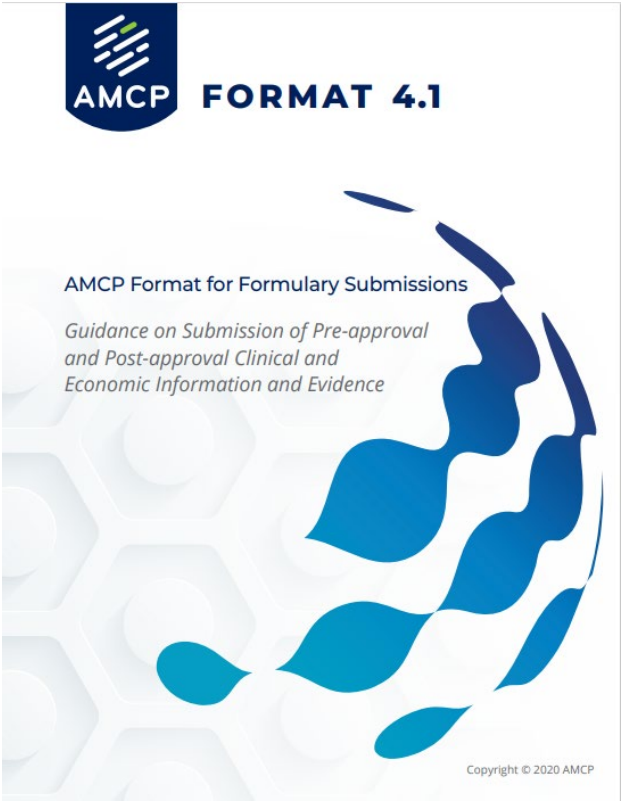
AMCP Evidence Recommendations for Approved Product Dossiers

A dossier is a key mechanism to guide manufacturers in presenting evidence for new pharmaceuticals, biologics, and vaccines to gain reimbursement and/or formulary placement in the U.S. healthcare system. Dossiers are considered a "living" document and should be updated to reflect the evolution of a drug's evidence and value.

The AMCP format provides an easy-to-use outline of the evidence innovators should provide to payers, P&T committees, etc. for evaluating new drugs for formulary and coverage decisions. The AMCP is the most widely accepted drug evidence format used in the U.S.

The primary components of the framework include:

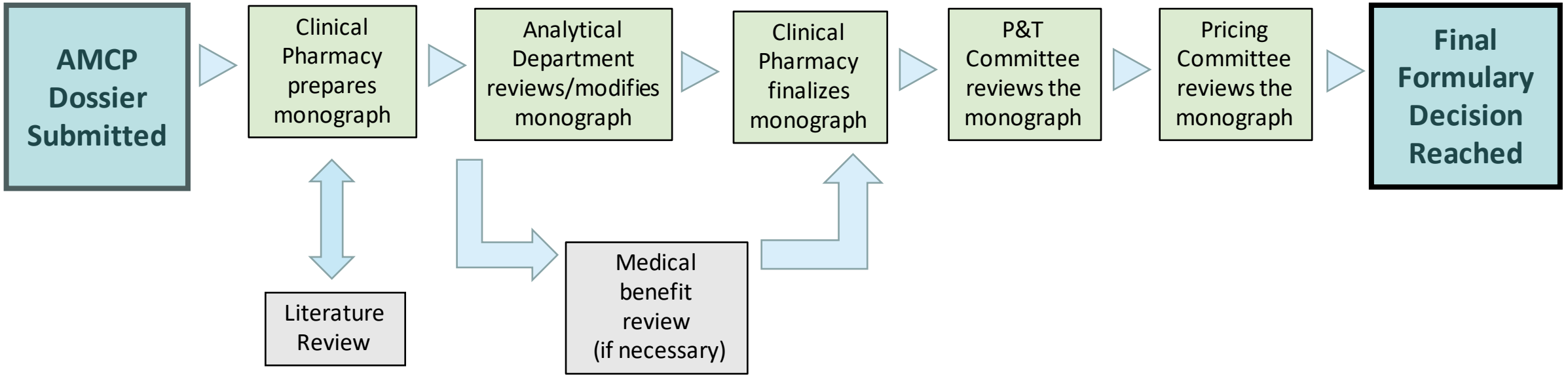
1. Executive Summary (concise summary of clinical and economic value)
2. Product Information and Disease Description
3. Clinical Evidence
4. Economic Value and Modeling Report
5. Additional Supporting Evidence
6. Appendices



Process Overview of an AMCP Formulary Dossier

Example of how information from an AMCP dossier submission is typically reviewed by Payers P&T Committees.

A monograph details a drug's identity (strength, purity, performance) and the tests used to validate it. It is important to note that payers will continue to review evidence and may adjust reimbursement after market authorization. Developers should update the dossier throughout the drug lifecycle (add any new clinical evidence of benefits, new indications, etc.).



Since the target population for Hebdomadal will include Medicare patients (and private payers often follow Medicare's coverage decisions), getting Medicare coverage of Hebdomadal is critical.

Fourth Stage: Determine Payer Contracting and Engagement Strategy

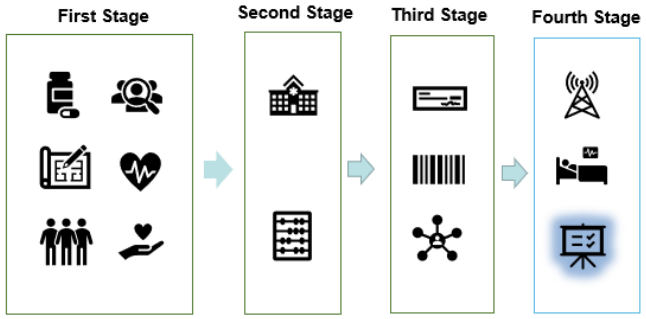
Sophia understands that payer contracting is essential to ensure Hebdomadal has the desired market access and patients can get the drug at an affordable price.

As part of her final efforts prior to gaining FDA approval and launch, Sophia works with PHARMACON 's market access consulting firm to assess the current payer contracting environment as it relates to high cholesterol treatment drugs. She realizes they must be proactive in their approach to payers because contracting models are changing from traditional volume-based contracting to value-based (outcome) contracting. It will take the right mix of pricing and discounting to gain market share while maintaining a profitable margin.

Some key questions to address before the contracting process might be:

- What is the competitive threat for this drug?
- Is the deal driven by profit goals or market share?
- Has the payer's formulary structure changed?
- What type of access should they contract for, e.g., preferred or non-preferred positioning?
- Are the deal terms profitable?
- How much discount should be offered, and to whom?

To address these questions and others, Sophia will need to assess the internal analytic capabilities, team subject matter expertise, and whether additional technology platforms to inform decision-making are necessary.

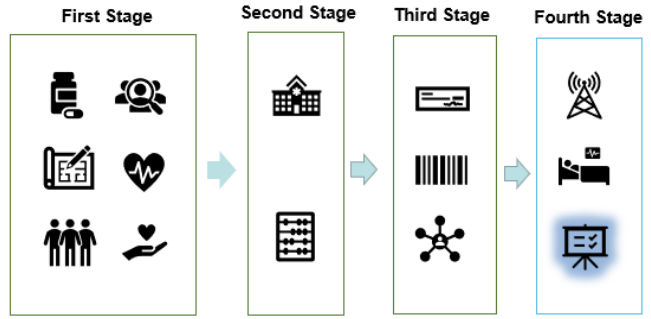


Key questions:

- What is the payer contracting process?
- What questions should be asked?
- What type of analyses can be done to support the decision-making?

Fourth Stage: Determine Payer Contracting and Engagement Strategy

Sophia understands payer contracting is typically an iterative process over the life span of a drug.



Payer Contracting Life Cycle

Key activities to support overall decision-making throughout the payer contracting lifecycle include:

- Identifying patient populations, providers, and payer mix
- Analyzing potential market share
- Tracking sales after market launch
- Calculating rebates and discounts
- Comparing forecast data and actual data

Key questions:

- What is the payer contracting life cycle?
- What type of analyses should be done to support the decision-making?

SUMMARY

Hebdomadal Press Release

The following is a fictional portrayal of what success for Hebdomadal may look like

For Immediate Release

PHARMACON's Hebdomadal™ Available for Adults with High Cholesterol

Washington, D.C., Jan. 18, 2023 –

Centers for Medicare & Medicaid Services (CMS) announced today that Medicare enrollees can get Hebdomadal – a once-weekly, controlled-release, long-acting oral drug developed by PHARMACON for treating adults with heterozygous familial hypercholesterolemia (HeFH) or clinical atherosclerotic cardiovascular disease (ASCVD), who require additional lowering of low-density lipoprotein-cholesterol (LDL-C) – as part of their Medicare Part D benefits.

The U.S. Food and Drug Administration (FDA) granted Market Authorization for Hebdomadal on Jan. 6. Because Hebdomadal demonstrated an improvement in lowering LDL-C levels in two clinical trials involving more than 3,000 adults with HeFH or ASCVD and high LDL cholesterol, FDA's Cardiovascular and Renal Drugs Advisory Committee voted unanimously to confirm Hebdomadal's clinical benefit to patients at their most recent meeting.

In a statement released this morning the CMS Administrator said, "CMS has always been committed to helping people obtain timely access to innovative treatments that meaningfully improve care and outcomes. As an FDA approved drug, Hebdomadal will be available to Medicare enrollees who meet the required criteria…..

Summary Findings: By Stage

First Stage

- Research and Evaluate Competitors: weekly oral drug may disrupt the status quo/take markets hare away from competitors
- Create Economic Overview: Gather market data of similar products
- Determine Patient Population: Adults with HeFH and ASCDV and high LDL-C levels
- Determine Evidence/Value: Research peer-reviewed studies for clinical value
- Identify Payers: Since Hebdomadal is for adults, there are multiple types of payers

Second Stage

- Identify Healthcare Providers: Identify high-prescribers of cholesterol drugs (e.g., cardiologists, endocrinologists, internists)
- Drug Pricing Strategies: Consider value-based pricing and competitor-based pricing strategies. Factor in commercial payer considerations.
- Set a Launch Price: The launch drug price of $ 11.54 per week for Hebdomadal should reflect both the uniqueness of the drug and its efficacy – especially since clinical data shows an improvement in lowering high LDL-C levels

Third Stage

- Research Rebates and Discount Requirements: New drugs are added to health plan formularies through a combination of rebates, discounts, reimbursements
- Understand Drug Channel Distributions: Brand drugs typically move from the manufacturer, wholesaler, retail pharmacy to the patient. Specialty brand drugs have a more complex path to the patient.
- Engage Stakeholder Support: Develop a strategy for engaging internal and external stakeholders to deliver the value story about the new drug

Fourth Stage

- Communications Plan: Personalize messaging for patients, healthcare providers, professional medical societies, and payers
- Compile Formulary Dossier: Create a dossier (a summary of clinical and economic value) for payers and P&T Committees to use when they are making formulary and coverage decisions
- Determine Payer Contracting and Engagement Strategy: Establish a payer contracting strategy that includes the right mix of pricing and discounting to gain market share while maintaining a profitable margin

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Appendix