Stanford MEDICINE

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INTRO TO HEALTHCARE STUDY GUIDE

MODULE 5 - HEALTH CARE PRODUCTS AND PRESCRIPTION DRUGS, AND QUALITY MEASUREMENT AND IMPROVEMENT

LEARNING OBJECTIVES

- Describe the regulatory structure governing approval of prescription drugs
- Describe the role of patents and the role of generic drugs
- Describe the main institutions involved in negotiating drug prices and paying for pharmaceutical products
- Identify key organizing concepts for efforts to improve quality
- Identify structure, process, and outcome measures of quality
- Describe some different types of approaches to improving quality

HEALTH CARE PRODUCT REGULATION: OVERVIEW

Types of products and equipment

- Medical Devices
- Imaging Equipment
- Biologic Products
- Drugs

Most products or equipment used in medical care are regulated, in case of the US, FDA (Food and Drug Agency).

Drugs: Substances intended for use in the diagnosis, treatment, mitigation, cure, or prevention of disease.

Main groups of drugs:

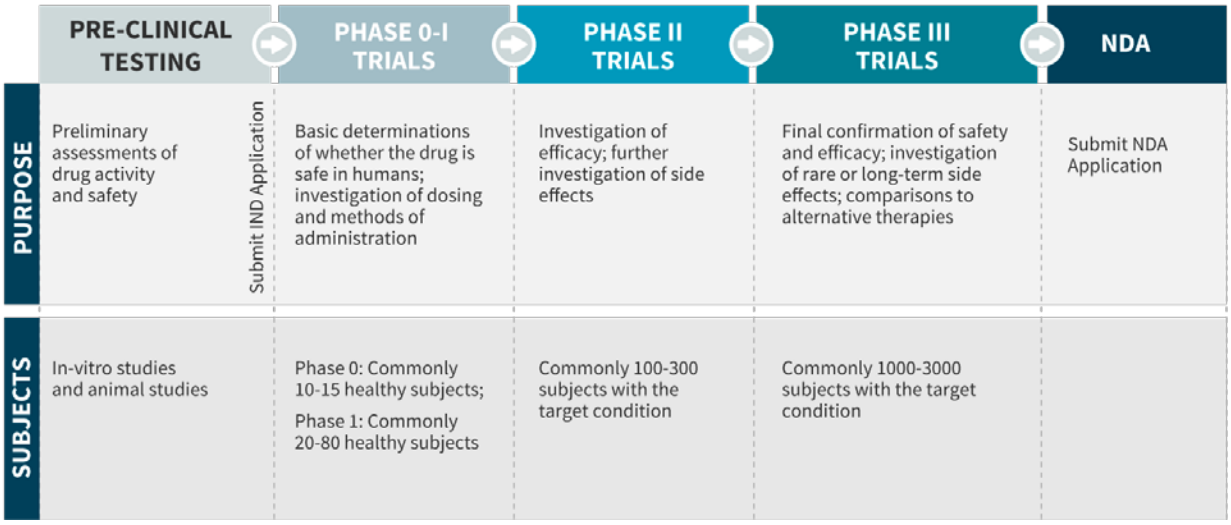
1. Prescription drug
    - Drugs that requires a prescription to obtain
2. Over-the-counter or OTC drugs
    - Drugs that do not require a prescription to obtain

From an approval and regulatory process, both prescription drugs and OTC drugs are regulated, but the processes are different. The process for prescription drugs is more involved, and prescription drugs have gotten a lot of attention lately.

PRESCRIPTION DRUG REGULATION AND PRICING

Prescription drug is a pretty complex area, with a lot of regulations. In the U.S., the Food and Drug Administration, or FDA, is tasked with overseeing prescription drugs - their specific mandate is to see that prescription drugs are safe and efficacious – they work. To sell a prescription drug in the U.S., one needs FDA approval.

Getting FDA approval involves a number of steps:



- Drug companies can start the normal approval process with the filing of something called an investigational new drug application, an IND for short.
- The FDA looks this over, and if approved, the company is allowed to begin the process of testing the new drug in humans. This involves a series of 3 different types of trials, called Phase I, Phase II, and Phase III trials.
    - Phase I trials are smaller, first steps, designed to test basic things about the drug including its safety in humans and dosages.
    - If Phase I trials are successful, then Phase II trials can be conducted on dozens or a few hundred people with the disease or condition of interest to test for effectiveness and side effects.

- If Phase II trials are successful, then the company can move on to Phase III trials, with thousands of people to gather more data on effectiveness and outcome for longer terms.

If all of this goes well, then the company can file a new drug application or NDA. The FDA reviews this, and if approved, the company is finally able to begin marketing the drug – doctors can prescribe it and patients can buy it.

It takes a long time for drug approvals; 9 years is a general estimate number. It can be pretty expensive for the companies to do the work, many millions or even billions of dollars.

The FDA may continue to monitor drugs once they're on the market to assess their performance – sometimes people refer to these as Phase IV trials. FDA can regulate the labeling, manufacturing processes, and other aspects of the process of making and selling drugs.

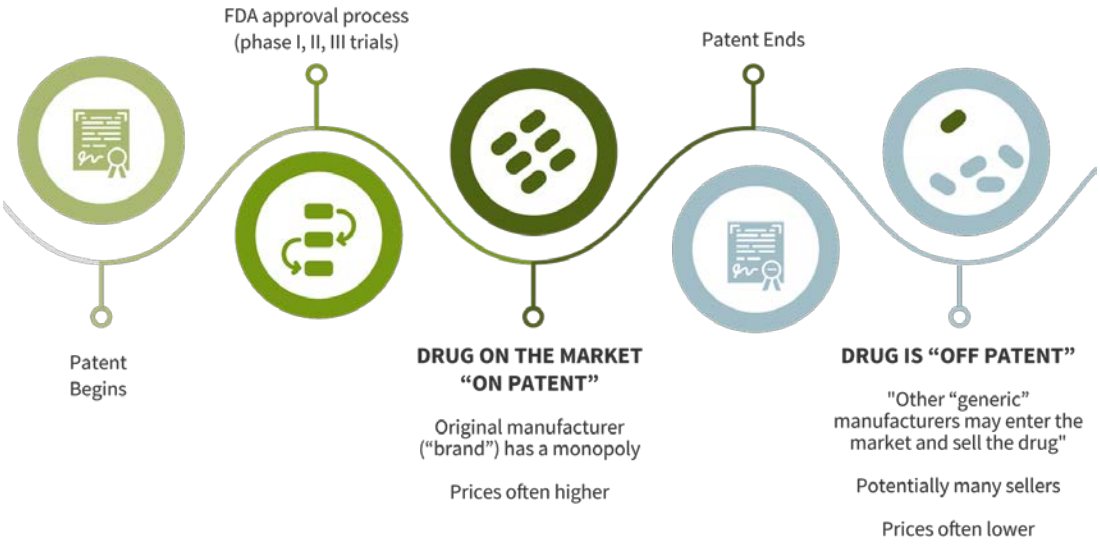
In other countries, the regulatory agencies are similar but it is a separate process.

PATENTS, BRANDED DRUGS, AND GENERIC DRUGS

For prescription drugs, an important distinction is between "branded" and "generic" drugs.

- **"Branded" drugs:** Drugs sold by the manufacturer that first brings the drug to market with patent protection
- **"Generic" drugs:** Drugs sold by additional manufactures after the primary manufacturer's patent expires.

A key part of this discussion has to do with patents.



Alongside the FDA approval process, a new idea for a drug can be seen as an invention and can be patented. When an inventor comes up with an invention and patents it, the inventor gets the right to be the only one selling the invention for a period of time, which can be called "market exclusivity."

The drug is brought to market with patent protection as a "branded" drug. They are sold as brand name products, which tend to be more expensive than generics.

After a while, patents expire, and other manufacturers may be able to figure out how to make the same drug. They could then start making it and selling it as well, which is called "generic drug" or just a "generic".

One important thing to note is the effect on price associated with the entry of generic drugs. While a drug is covered by a patent, and there is only the one seller allowed, prices tend to be higher. Once generics enter the market, the arrival of more competition often leads to reductions in the prices charged for the drug, and these price changes are often notable.

INSURANCE AND FORMULARIES

When a patient is going to use a prescription drug, they need a prescription from a physician or other suitably authorized provider –The general rule is that you can't get a prescription drug without a prescription.

To get the drug, or sometimes we say to get the prescription "filled," the patient needs to take the prescription to a pharmacy. Pharmacies can be wholesale or retail, online or brick and mortar. Pharmacies are staffed by pharmacists, people with advanced training who are experts in different drugs and their uses, and how to handle them.

Most patients have insurance coverage to help them cover the cost of buying prescription drugs. It's common for insurance plans to have some provisions, that affect the drugs patients can use and the ways the insurer will cover them.

A key concept is the **formulary**; this is most generally the list of drugs that the insurance company will allow the patient to use and get reimbursed for.

Formularies can vary from one insurer to another, and formularies can be tiered.

- Tiered formulary: A formulary arrangement in which drugs are placed in different "tiers" with different levels of patient cost sharing

The insurer can create incentives for patients to use drugs in preferred tiers.

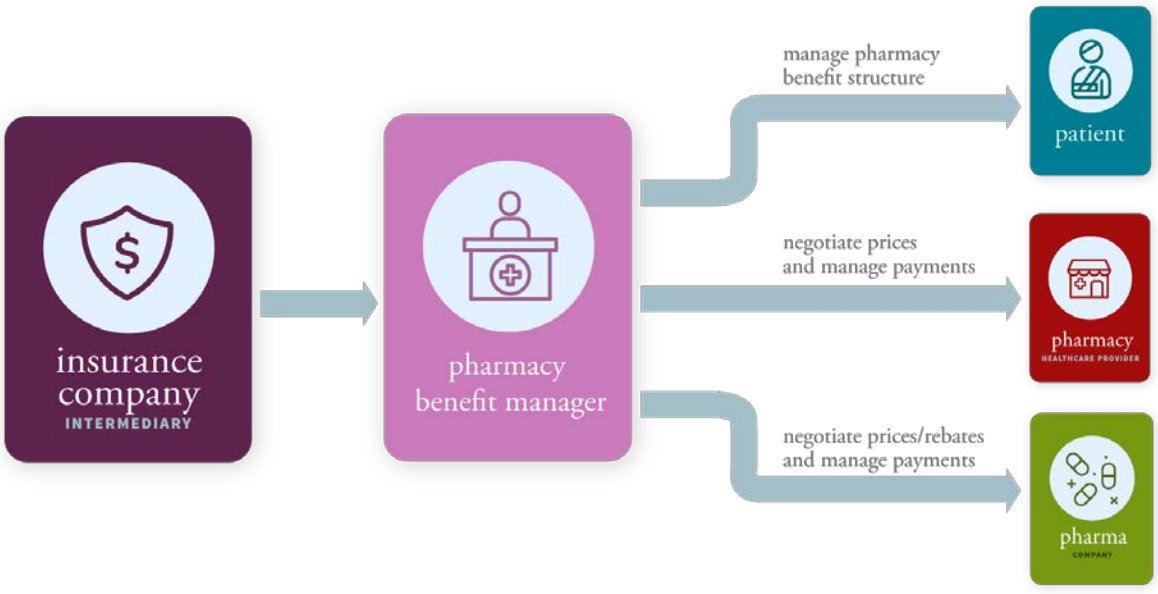
Plans may use utilization review, like pre-authorization, where a patient needs to seek pre-approval from the plan before getting the medicine. Plans may sometimes require "step therapy," where the plan might impose a requirement that a patient tries a more preferred drug first before they will cover a less preferred one.

INTERMEDIARIES, PHARMACY BENEFIT MANAGERS, DRUG PRICES, AND REBATES

An interesting and important set of interactions in the health care system revolves around negotiating prices and payments between insurers and drug manufacturers for prescription drugs and managing the prescription drug portion of insurance plans.

Two things intermediaries do with respect to prescription drug benefits:

1. Work out prices to be paid to manufactures when covered patients use prescription drugs
2. Set up the structure of insurance benefits for prescription drugs

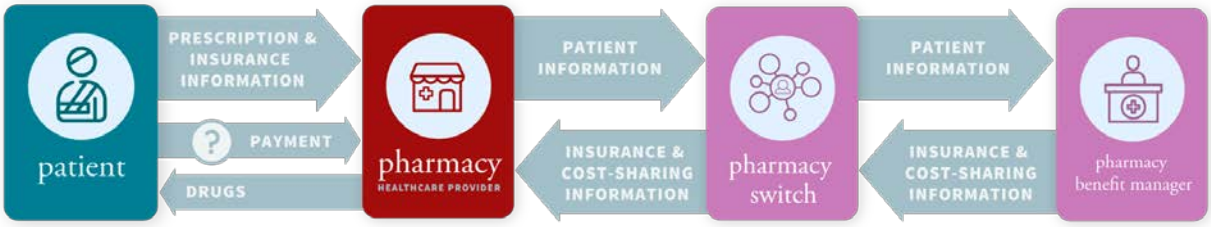


Most intermediaries commonly contract with a third party, called a **pharmacy benefit manager**, or **PBM**, to handle these things. PBMs can manage prescription drug coverage.

Three things PBMs can do:

1. Manage the design of prescription drug benefits (formularies or networks)
2. Manage payments to pharmacies
3. Negotiate prices with pharmacies and negotiate rebates with manufacturers

PBMs are an important player in this world. When trying to understand prices that insurers pay for prescription drugs, you have to always keep in mind the rebates. If you see, for example, the retail prices paid by PBMs to pharmacies, this may or may not be a reflection of the actual amount an insurer paid at the end of the day, net of rebates.



While we're talking about the interesting world of prescription drug benefits and the entities involved in them, there is one more interesting entity to note here, a company commonly called a **pharmacy switch**.

- **Pharmacy Switch:** A third-party vendor used by pharmacists to transmit claims from the pharmacy to the PBMs

They help the pharmacy determine all the rules about who pays what for what--as in co-pay. They can take the insurance information, figure out which PBM is handling the coverage for the patient, electronically get the info about cost-sharing or other rules from the PBM, and then turn around and send it back to the pharmacy.

The pharmaceutical space has lots of interesting things going on in it relevant to innovation and data. Drug discovery and FDA approvals are very interesting areas, with lots of opportunity for innovations in figuring out new targets and new areas for research, and in working on the data and studies that are part of the approval processes. On the insurance and payment side, prescription drugs can be quite expensive, so there is a lot of room for innovation that can help manage the use of drugs, figure out processes to make the best use of drugs when there are different choices, figure out which patients will be the best candidates for drugs and so on.

Insurers can have a lot of data, and pharmacies may have some. But PBMs and switches may as well, and may actually offer different angles of insight into what is happening.

QUALITY MEASUREMENT AND IMPROVEMENT

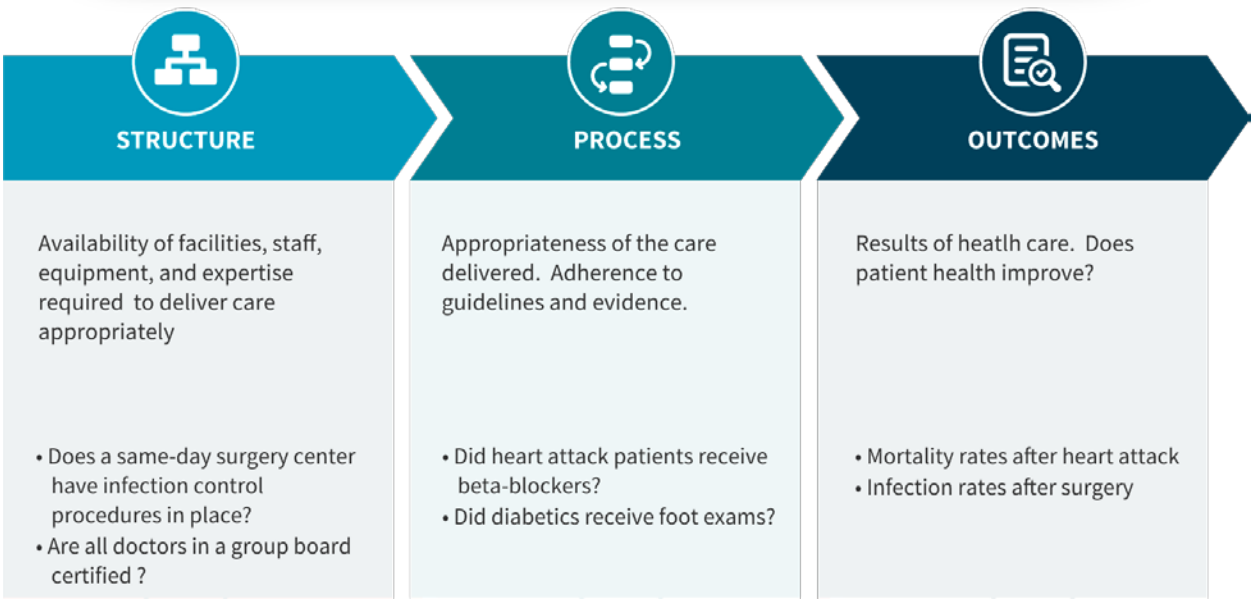
KEY ORGANIZING CONCEPTS OF QUALITY

Six domains of quality: widely known in a report on quality from the U.S. Institute of Medicine since renamed the National Academy of Medicine, published in the early 1990s. The acronym, sometimes people use STEEEP – safe, timely, effective, efficient, equitable, person-centered.

The six domains are:

1. Safe
2. Effective
3. Patient-centered
4. Timely
5. Efficient
6. Equitable

Overuse-underuse-misuse framework: When the healthcare system either uses too much health care, or not enough health care, or uses the tools at their disposal in the wrong way or at the wrong time.



Structure-Process-Outcomes: Also called as the Donabedian model. It was three process, which is structure, process and outcome.

Individual and Team: Health care is often delivered in teams, with multiple people and organizational structures. It is important for individuals to be well trained and focused on quality, of course. But it can also be very important to getting teams and organizations to work well.

QUALITY MEASUREMENT - STRUCTURE

Building and using quality measures has become a central component of efforts to improve quality, and we should look into some of the basic aspects of quality measurement.

When we talk about measuring quality, one thing we always need to keep in mind is whose quality are we measuring?

Different measurement levels:

- Measure quality for a provider or group of providers
- Measure quality for a health plan or intermediary
- Measure quality of care provided to an individual or a group of people

A very commonly encountered framework for thinking about quality measurement is **Structure-Process-Outcome**:

- How is care delivered?
- Does the health care system have a structure conducive to delivering quality?
- Are there enough providers, in the right places, with the right training and skills?
- In the equipment and infrastructure organized and financed in ways that enable them to work effectively?

Structural quality measures for providers:

1. Is a good EMR or EHR system in place?
2. Can an organization document that they have emergency protocols in place, can they document that they have protocols to prevent the spread of infections?
3. Are the physicians or other providers appropriately credentialed?
4. Are the facilities accredited, licensed, and certified appropriately?

Structural quality measures for health plans or populations:

1. Are there enough physicians and other professionals for the population being served?

2. Are there enough hospital beds?
3. Where are they located with respect to the population?

Data used for structural quality measurement can come from a variety of sources. Structural measurement is pretty commonly done. Often when you look at different measures, the more structural ones look like the easier ones to construct. At the same time, they may be the furthest from actual health outcomes, which is what we most care about, so quality measurement these days often goes beyond just structural quality.

QUALITY MEASUREMENT - PROCESS

One aspect of quality measurement in health care that has gotten a lot of attention focuses on processes of care are "**process measures**", or "**process measurement**".

The core idea is that if you have an outcome in mind that you want to achieve, then you can work to identify some processes that would lead to that outcome Then you design measurement around those processes, to track things like whether they are occurring and how often.

One very common way of approaching process measurement focuses on measuring whether generally accepted recommendations for clinical practice are followed. This would commonly involve specifying a population of people or patients of interest, specifying a particular treatment or procedure for which there is evidence of benefit, based on research or possibly on other things like professional opinion, and then creating a method to measure and track the share of the population that gets the recommended service.

One can define those populations, and then query databases or use some other method to see if people in those groups get recommended care or not. One can go gather data and assess whether the symptom/disease is happening. A variant is measuring care that should not be delivered, situations where evidence says not to do a particular type of procedure.

Process measures are probably the most common form of quality measures. Process measures can be created for different types of organizations.

There are a variety of sources of information for process measures. Often EMR or claims databases can be used to identify patients and track whether they got recommended care or not. There are registries that are sometimes used; registries are databases that try to collect and compile relevant information about all or at least many patients with a given condition or patients receiving a particular treatment, often for the purposes of having a resource for monitoring or improving quality.

QUALITY MEASUREMENT - OUTCOME

One very salient measure of quality is the **outcomes** of care.

The core idea here is that we define something we want to happen, or not happen, and see if it does or not. How is their health status, quality of life, or life expectancy impacted? This could also include things like whether their behavior changed, or their knowledge is improved.

Outcome measure examples:

1. Mortality Rates
2. Readmission Rate
3. Complication Rate
4. "Potentially Preventable" Admissions
    - Sometimes these are called "ambulatory care sensitive admissions" or "potentially preventable admissions."
5. Patient-reported outcome measures
    - Called by their acronym P-R-O-M, or PROMs

Outcome measures have many attractive features, but can also face challenges. One key challenge is the possibility that, while variations in outcomes from one provider or health plan, to another may be the result of differences in actual underlying quality, they could also be the result of other things, some beyond the control of providers or plans. Depending on the situation, this can be an important factor – there can be major differences in the patients cared for across providers, though there are not always.

As a result, doing a good job of measuring outcomes usually involves adjusting for risk.

- **Risk adjustment**: Approaches designed to correct for differing characteristics of patients

QUALITIY IMPROVEMENT

Quality measurement. Doing a good job of this can take some real effort and involve multiple aspects. One aspect is finding the right set of measures to use. Structure, process, and outcome measures can all be useful in different circumstances, and often they are useful in combinations.

Measurement activities are being developed and implemented by a variety of different entities. Some measurement activities are undertaken by organizations working to improve quality. The National Center for Quality Assurance has developed a system of quality measurement for health plans called the Healthcare Effectiveness Data and Information Set, or HEDIS, that includes a range of measures (structure, process, and outcome) that can be used to compare health plans.

Government organizations may be involved in quality measurement such as the Medicare program that has developed tools for measuring the quality of health care providers.

Providers themselves are often measuring quality and assessing their own performance. Sometimes individual hospitals or doctor practices will implement quality measures.

What then to do with the quality data being collected? There are several different things being done.

Uses of quality data:

1. Monitoring and improving provider quality
2. Public reporting
3. Designing payment incentives

Measuring quality and using measures to try and drive improvement is an important area in the health care system, with many different aspects to keep eye on, lots of activity, and lots of opportunities for further investigation and innovation.

Quality has gotten a lot of attention and generated a lot of work. There remain a number of important opportunities for people with interests in data and measurement, and interests in AI. Some of the opportunities for innovation are in measurement. There are multiple domains of quality – as the STEEEP framework, like effectiveness or safety, and there are in others like equity or patient-centeredness and risk adjustment.

There may still be room for innovative ways to figure out how to use them to guide treatment choices, figure out the best choice of provider for patients, figure out the best network for a payer, figure out the best incentives to attach to quality measures, and other implementation steps that can further our pursuit of improved health care quality.