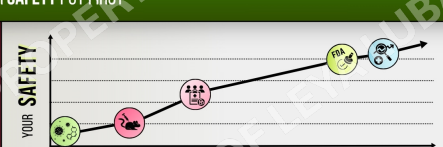
STARTING A **NEW MEDICATION?** CONSIDERING A **NEW TREATMENT?** WONDERING HOW A DRUG GOES FROM **DISCOVERY TO DELIVERY?**

KNOW THE **DRUG DISCOVERY** AND **DEVELOPMENT** PROCESS AND HAVE

CONFIDENCE IN YOUR DRUGS AND SAFETY

YOUR **SAFETY** PUT FIRST



DRUG DISCOVERY AND DEVELOPMENT **TIMELINE**

AVERAGE COST TO BRING A DRUG TO MARKET
$2.3 BILLION

THOUSANDS TO **MILLIONS** OF COMPOUNDS TESTED : ONLY **1** makes it to market

40 - 50 NEW DRUGS APPROVED annually by **FDA**

EARLY DRUG DISCOVERY

EXPLORATORY RESEARCH

on a disease or medical condition ***without*** *an effective, safe treatment*

What is **known** about the disease?
Have there been **treatments in the past**?
How does the disease work?

TARGET IDENTIFICATION and VALIDATION

Identifying a biological entity that contributes to your disease

ENZYMES and PROTEINS | GENES, DNA, RNA | CELLS and RECEPTORS

Validating that a change to it's function provides a **beneficial effect** against the disease

HIT COMPOUNDS to LEAD

high-throughput SCREENING of **1,000 - 1,000,000+** potential hit compounds

hit compound and initial **safety** assessment

select a **lead compound**

WHAT are **HITS** ?
Compounds that can cause the desired beneficial effect

a **LEAD** shows...
- *potential*
- **safety**
- *selectivity* for only the target
- **efficacy**

LEAD DESIGN, OPTIMIZATION AND PRE-CLINICAL STUDIES

DATA ANALYSIS

Analysing *in vitro*, *ex vitro* and *in vivo* test results for

1. **SAFETY AND TOXICITY**: the ***utmost priority***
2. **POTENCY AND DOSAGE**: the strength of **effect** given the **dose**
3. **PHARMACODYNAMICS**: what the ***drug*** does to the body
4. **PHARMACOKINETICS**: what ***the body*** does to the drug

ABSORPTION
DISTRIBUTION
METABOLISM
EXCRETION

PRE-CLINICAL TESTING

From data analysis, extensive drug **DESIGN** ***modifications*** are made to improve overall **safety** and **efficacy**

IN VIVO
is to test drug ***within*** the body, typically using rodents
shows drug–body interactions, toxicity and needed drug dose

IN VITRO
is to test drug using bacteria, and **cells *outside*** the body
shows drug interactions with target, metabolism and toxicity

EX VITRO
is to test drug using human and animal **tissue** and **organs *outside*** the body
mimics human-like reactions *without harming living things*

OPTIMIZATION

The leads design and properties are ***structurally modified*** to improve its safety, efficacy, selectivity and interactions in the body

Computer systems model the lead molecule

Harmful components to your health are **removed**

EFFICACY vs. **EFFECTIVENESS**
How well drug works in situations that are:
- ***controlled*** *(efficacy)*
- ***real-world*** *(effectiveness)*

PATENT

an exclusive *right to an* ***invention***

At this stage, companies often file patents to ***protect*** their drug from replication without permission

To apply, **DRUGS MUST BE**:
- **new** and **novel**
  NOT been done before
- **not known** to public yet

CLINICAL TRIALS AND CLINICAL RESEARCH

For the final test drug to move onto human clinical trials, an

INVESTIGATIONAL NEW DRUG (IND) APPLICATION must be FDA APPROVED

Why? to ensure the **safety** and **rights** of clinical trial volunteers are **protected** and the drug and clinical study plans meet **federal standards**

PHASE I
clinical trials

WHO:
20 to 100 participants
FOCUS:
safety, side affects and dose
LENGTH:
several months
~70% OF DRUGS MOVE ON

PHASE II
clinical trials

WHO:
several hundred participants with the disease/condition
FOCUS:
safety and efficacy
LENGTH:
several months - 2 years
~33% OF DRUGS MOVE ON

PHASE III
clinical trials

WHO:
300–3,000 participants with the disease/condition
FOCUS:
efficacy and side effects
LENGTH:
1 - 4 years
~28% OF DRUGS MOVE ON

PHASE IV
clinical trials *after* approval

WHO:
several thousand participants with the disease/condition
FOCUS:
safety and efficacy
LENGTH:
several years

FDA REVIEW AND POST MARKET MONITORING

1. NEW DRUG APPLICATION is submitted to FDA for market approval

APPLICATION *must report:*
- ☑ all clinical trial data
- ☑ any side effects, serious problems and changes made in clinical study

2. FDA REVIEW of application, study results, manufacturing facilities, proposed market price and drug labels, information and packaging

3. FDA **APPROVAL** if drug is **SAFE, NON-TOXIC, EFFECTIVE** and **NOVEL**

4. DRUG GOES ON MARKET and is available for use. An **advisory committee** of **inspectors** is formed to monitor adverse reactions and effectiveness for your continued **SAFETY** and **CONFIDENCE**

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YOUR **SAFETY** IS ALWAYS THE **PRIORITY**

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