



# GLOSSARY OF KEY TERMS

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## RESEARCH STAGES

**Basic Research:** Focuses on learning how living things work, but not necessarily with any application to a specific disease or condition.

**Translational Research:** Described as “bench to bedside” research, or applying findings from basic research toward developing drugs for specific diseases or disorders. The step from basic research to preclinical research requires numerous resources, many of which are expensive, and the risk of not finding a drug safe and effective enough for moving to clinical research is high. This results in a “translational research gap” because of these challenges of moving a finding from basic research to a drug to be tested in preclinical research.

**Preclinical Research:** Identifies a particular therapeutic and tests its safety and efficacy before beginning clinical studies in humans.

**Clinical Research:** Tests ways to treat, diagnose, prevent, or provide supportive care for diseases or conditions to be sure they are safe and effective.

**Post marketing research:** Examines therapeutics for long-term safety issues and additional information about the benefits and clinical use.

## APPROVAL APPLICATIONS

(These are reviewed in the U.S. by the FDA. International applications may have different names and are reviewed by country-specific agencies.)

**Investigational New Drug (IND):** Application to allow clinical trials to begin for a drug based on preclinical test results.

**New Drug Application (NDA):** Application to allow sales of a therapeutic made by a chemical process based on clinical trial results.

**Biologic License Applications (BLA):** Application to allow sales of a therapeutic made by a biological process (e.g., vaccines, gene therapy, antibodies) based on clinical trial results.

## RESEARCH TOOLS

**Biobanks or Biospecimen repositories:** Collection of human biological samples (e.g., tissues, blood) that can be used for research.

**Animal Models:** Non-human animals that are studied to gain understanding of how a disease or disorder may develop or progress in humans.

**Cell lines:** A population of cells that can grow and divide indefinitely that can be used to study the structures and biochemistry of the cells.

**Biomarkers:** Short for biological marker, is something that can be measured to determine the presence or progression of a disease/disorder or the response to a treatment.

**Genomics/Proteomics/Metabolomics:** Analysis of the structure and function of the genome (an individual's DNA, both sequences that code for proteins and sequences that do not code for proteins), the proteome (the proteins), or metabolome (the small molecules that are produced during metabolism).

**Assays:** A rigorous test that can measure the presence, amount, or activity of a molecule. For example, these tests can be used to study the effect of a drug on killing tumor cells or on blocking a protein that damages nerve cells or to study the expression of genes and how that changes with a disease or drug.

**Patient/Disease Registries:** A collection of data from individuals with a particular disease or disorder. Registries vary widely but often include clinical history, genomic data, and laboratory test results.

## TYPES OF CLINICAL TRIALS

**Phase 1:** Small clinical trials usually involving 10-50 people designed to find out if a therapeutic is safe.

**Phase 2:** Clinical trials of a few hundred people designed to continue to confirm safety and find out how effective a therapeutic is for a particular disease or condition.

**Phase 3:** Clinical trials of several hundred to thousands of people designed to continue to confirm safety and to clearly show that a therapeutic is effective.

## SPECIAL STATUS THAT SPEEDS REGULATORY PROCESS (IN U.S.)

Special status is possible if certain criteria are met regarding advantages of the therapeutic.

**Accelerated Approval:** Allows marketing approval based on intermediate effect or biomarker rather than the final effect.

**Priority Review:** Provides review of the NDA by the FDA within 6 months.

**Fast Track:** Provides the company testing the therapeutic with more frequent meetings at the FDA for feedback on clinical trial design and may have rolling review of the final approval application. Therapeutics with Fast Track designation may also receive Accelerated Approval and Priority Review if qualified.

**Breakthrough Therapy:** Receives Fast Track designation and recognition that the therapeutic may be a large improvement over currently available treatments.

**Pediatric Priority Review Voucher Program:** Upon approval of an NDA for a rare pediatric disease therapeutic, the sponsor receives a voucher that can be used to designate Priority Review for another therapeutic or may be transferred or sold to another sponsor to be used for an unrelated therapeutic. This program is not currently permanent.

**Orphan Drug Status:** Specifically for therapeutics to treat rare diseases and provides tax credits and other financial benefits related to the development of the therapeutic.

## PATENT ISSUES

Patents provide incentives for therapeutic development. A patent holder has exclusive rights to sell a therapeutic or use a particular technology used to develop or manufacture a therapeutic for 20 years from the filing of the patent application. Patents can be licensed (sometimes for a very high fee) to others to use the patented technology.

If a company does not agree to license a patent, other companies cannot pursue development that would involve the use of the patented therapeutic or technology. If the therapeutic is not eligible for a patent, companies might not pursue development since the costs may be greater than the eventual revenue.

Companies can pursue patent lawsuits for improper use of their patents or to challenge other patents that are very similar. These lawsuits can be filed at any time during the patent protection period and may affect a pharmaceutical company at any time in the research and development process.

## PRICING AND REIMBURSEMENT

Prices for therapeutics are rapidly rising. There have been cases internationally where government agencies have determined that the benefits of a therapeutic are not significant enough for the company's proposed cost and declined to provide reimbursement at that price. In the U.S., there is now increasing concern that therapeutic prices will continue to increase and some insurance programs may decide to limit coverage for some therapeutics.

## FUNDING

**Grants from Federal and State Government Agencies:** In the U.S., the principal federal agency that funds basic and translational research in biological and biomedical sciences is the National Institutes of Health. The National Science Foundation also funds grants for biological basic research, but it does not fund research that explicitly tests a therapeutic or seeks to understand the development of a disease. State governments also provide grants for biomedical research. Government agencies provide the majority of funding for basic and translational research.

**Industry Funding:** Companies provide limited funding of grants to basic and translational researchers. However, most of industry funding is targeted at preclinical and clinical research.

**Nonprofit grants:** Nonprofit organizations provide funding through grants for research throughout the R&D process.

**Venture philanthropy:** This is a new approach used by nonprofit groups that involves adopting tools and strategies of venture investment firms to philanthropy to support biomedical research. Rather than grants where the money is provided to a researcher with little follow-up, nonprofit groups view their support as a contract, including monitoring the progress and evaluating the results against specified goals. These efforts are being credited with changing the R&D process to significantly accelerate therapeutic development.

# DRUG DEVELOPMENT ROADMAP

## BASIC RESEARCH/DISCOVERY

### RESEARCH TOOLS

Biobanks, Animal models, Cell lines, Biomarkers, Genomics/ Proteomics/Metabolomics, Assays, Patient/Disease registries

### "TRANSLATIONAL RESEARCH GAP"

Building bridges between basic research and industry with new funding models and reducing risks of commercial development



### HOW PATIENTS CAN SHARE THEIR VOICES

- Providing clinical data, genomic data, and biospecimens
- Forming patient communities
- Encouraging research focus through personal connections
- Encouraging sharing of data
- Providing funding

## PRECLINICAL RESEARCH

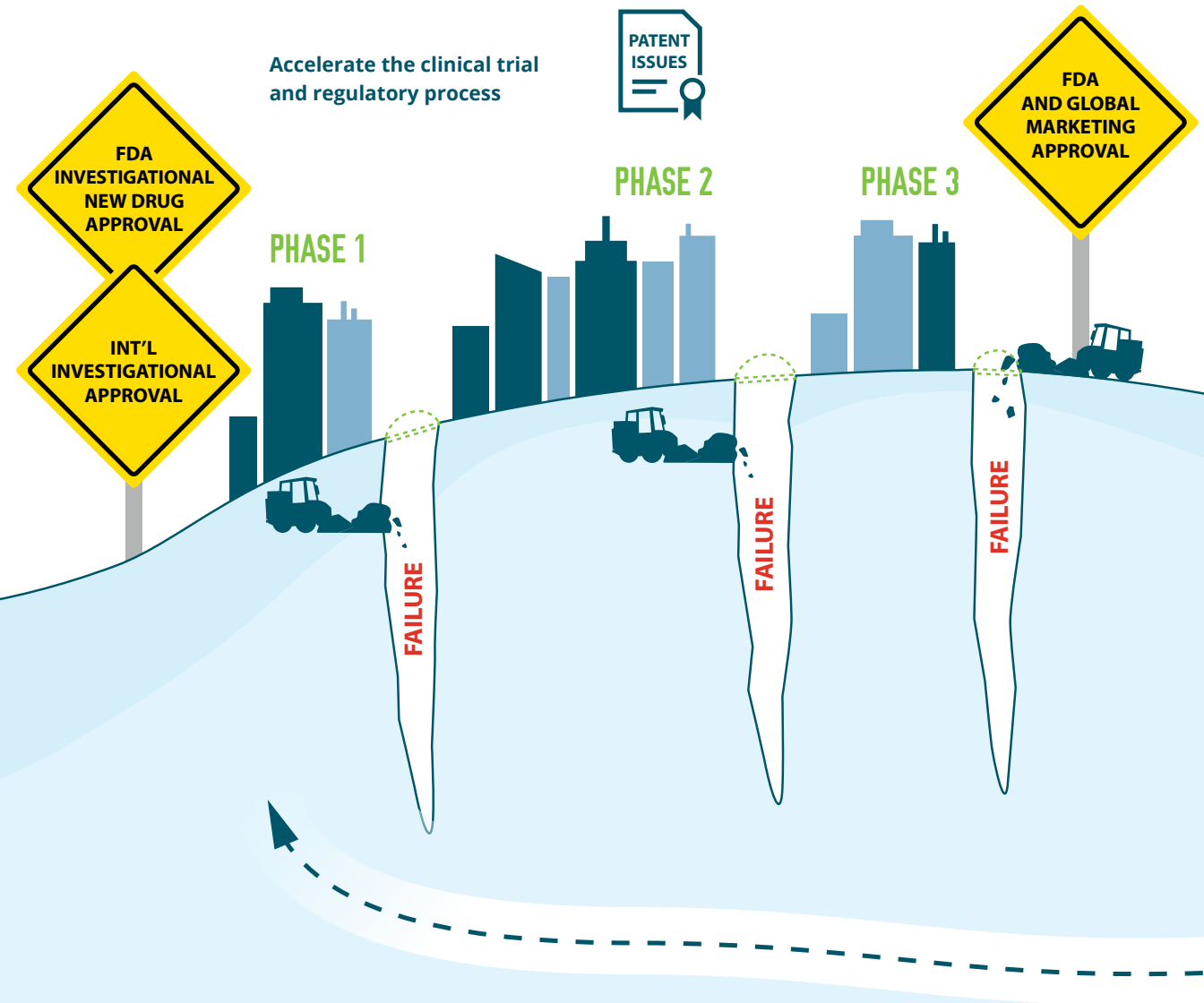
### FUNDING

Venture philanthropy,  
Federal/state grants,  
Industry support, Nonprofit  
grants

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## CLINICAL RESEARCH



### HOW PATIENTS CAN SHARE THEIR VOICES

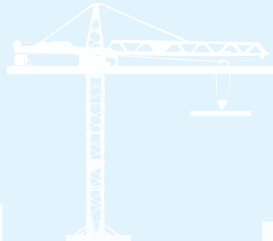
- Participating in clinical and natural history trials
- Serving on government or industry advisory committees
- Advocating for regulatory changes that will facilitate rare disease clinical research
- Providing clinical data, genomic data, and biospecimens
- Providing funding for clinical trials

## DISTRIBUTION AND ACCESS



### HOW PATIENTS CAN SHARE THEIR VOICES

- Advocating for and funding repurposing of approved drugs
- Working with patient advocacy and outreach at companies to ensure access to new drugs



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