STM JOURNALS

Research & Reviews: A Journal of
Drug Design & Discovery

ISSN: 2349-9036
Volume 13, Issue 1, 2026
January–April
DOI (Journal): 10.37591/RRJoDDD

https://journals.stmjournals.com/rrjoddd

Review RRJoDDD

Pathways in Drug Discovery and Development: From Molecular Targets to Market Approval

Chandani B. Patil[1,*], Jagruti G. Khairnar[2], Sunila A. Patil[2], Sunil P. Pawar[3]

Abstract

A complicated, multidisciplinary, and resource-intensive process, the discovery and development of new pharmacological drugs is essential to the advancement of contemporary medicine. Finding and optimising lead chemicals comes after a disease-relevant biological target has been identified and validated. Through preclinical research in animal models, these leads are thoroughly assessed for toxicity, pharmacokinetics, safety, and efficacy. Clinical trials, which are carried out in several stages to evaluate safety, efficacy, ideal dosage, and long-term effects in human populations, are the next step for successful candidates after regulatory assessment. The entire process, from basic research to market approval, typically takes 12 to 15 years and costs between $900 million and $2 billion for each successful treatment since so many compounds fail at different stages. This demonstrates the high level of risk and expense. Allosteric modulation, drug repurposing, multi-target agents, and the combination of biologics and natural products are some of the new drug development techniques that are expanding therapeutic alternatives and efficacy. Improvements in network pharmacology and collaborative research are also helping to identify new drug candidates. Ultimately, developing and discovering new drugs requires scientific ingenuity, effective project management, and perseverance backed by a commitment to moral and legal values.

Keywords: Target Identification, drug development, and drug discovery, approaches, pharmaceutics

INTRODUCTION

One phase in the intricate process of drug development is identifying a pharmacological molecule that is therapeutically beneficial in controlling and treating a medical condition. Researchers typically discover novel medications by gaining a better understanding of the disease process in order to create a medication that either prevents or reverses the symptoms of the disease [1]. After clinical testing, if a chemical produces positive results, the medication development procedure will begin. Drug discovery and development is an expensive process due to the high costs of R&D and clinical trials. It takes 12 to 15 years for a single new drug molecule to be created and put on the market to treat patients [2]. An estimated $900 million to $2 billion is spent on research and development for every successful medication. The price of the thousands of failures is included in this sum: Only one of the five to ten thousand compounds that go through R&D is eventually approved. Even though these figures are astounding, a basic understanding of the research and development process can assist to explain why so many compounds fail and why it takes so long and so much work to get a single medicine to patients [3]. A cutting-edge lab and equipment, meticulous project management, and the brightest logical and scientific brains are necessary for success. It also requires luck and perseverance [4].

***Author for Correspondence**
Chandani B. Patil
E-mail: patilchandani836@gmail.com

[1]Student, M. Pharm, Department of Quality Assurance, P. S. G. V. P. Mandal's College of Pharmacy, Shahada, Maharashtra, India
[2]Associate Professor, Department of Pharmaceutical Chemistry, P. S. G. V. P. Mandal's College of Pharmacy, Shahada, Maharashtra, India
[3]Principal, Department of Pharmacognosy, P. S. G. V. P. Mandal's College of Pharmacy, Shahada, Maharashtra, India

Received Date: September 01, 2025
Accepted Date: November 04, 2025
Published Date: November 16, 2025

Citation: Chandani B. Patil, Jagruti G. Khairnar, Sunila A. Patil, Sunil P. Pawar. Pathways in Drug Discovery and Development: From Molecular Targets to Market Approval. Research & Reviews: A Journal of Drug Design & Discovery. 2026; 13(1): 1–4p.

DRUG DISCOVERY AND DEVELOPMENT STAGES

Target Identification

Finding a disease's biological cause and possible intervention targets is the first stage in the medication development process. Finding a potential therapeutic target—a gene, nucleic acid, or protein—and evaluating its significance in the illness constitute the first step in target discovery [5]. After the target has been identified, the molecular processes it targets must be described. The perfect target should be "druggable," safe, efficient, and in line with commercial and clinical guidelines. The target identification techniques may be based on the domains of molecular biology, biochemistry, genetics, biophysics, and others [6].

Methods

- Potential disease targets are identified, selected, and ranked using bioinformatics data mining.
- Modifications to mRNA/protein expression profiles.
- Pathway and phenotypic analysis, as well as mechanistic study based on in vitro cells.
- re screening using knockdown, knockout, or target-specific techniques [7].

TARGET VALIDATION

This involves checking how similar molecules behave, looking at the signals that follow the suspected target, making versions of the target that are resistant to drugs, and changing the levels of the target to see the effects [8]. The main goal is to prove that the target plays a real role in the disease. Even though it's important to test how safe and effective a drug is in lab models and animals, the final proof comes from seeing if the drug works in real patients [9].

IDENTIFICATION OF LEAD

A molecule that is stable to make, useful, and resembles real drugs, and that works well with the target receptor in the first and second rounds of testing is known as a chemical lead. To find such a molecule, you need to check if it can be made, connect its structure to how it works, and show that it interacts with the target and works in living animals. These are the key features of a chemical lead [10].

LEAD OPTIMISATION

After finding a promising molecule, the next step is called lead optimization. This process involves repeatedly making and testing new versions of the molecule to better understand how its structure affects its ability to interact with the target. The goal is to improve the molecule's performance while keeping its good qualities. Lead optimization is part of the early stages of drug discovery, where scientists look for better compounds from those found through high-throughput screening. During this phase, the focus is on making and testing new molecules to refine their structure. The main aim is to fix any issues with the molecule's design while keeping its beneficial properties. To do this, the chemical structure of the molecule—whether it's a small molecule or a biological compound—may need to be altered to make it more specific and effective against the target. Scientists in the lab collect important information about the molecule, such as how toxic it is, how well it works, how stable it is, and how well it moves through the body. They also study its effects on the body, how it is absorbed and processed, and its potential harmful effects [11].

PRE - CLINICAL TRIALS

Pre-clinical drug research can focus on either toxicology or pharmacology, and both areas play important but different roles. Pharmacology mainly looks at how a drug moves through the body and how it interacts with biological targets. This includes understanding how the body takes in, spreads, breaks down, and gets rid of the drug, as well as how it affects the body's functions. Toxicology, on the other hand, checks for any harmful or negative effects of the drug. Scientists use animals to test the drug's safety and identify any potential risks. Pharmacokinetic studies are very important for figuring out how safe and effective a drug is. These studies look at how fast the drug is absorbed through different ways of taking it, which helps decide the best form, dose, and schedule for giving the drug. It also helps understand how quickly the drug spreads through the body, is processed, and leaves the body [12]. This

information is key to determining the drug's half-life, which is important for assessing safety and getting approval from regulatory bodies. How a drug is spread around the body greatly affects how well it works. This depends on how well the drug binds to certain tissues and how much of it actually reaches the bloodstream. Metabolism studies show how the drug is changed in the body, which enzymes are involved, and how active or inactive products are formed. All these findings are crucial for creating better, safer drugs that meet the high standards set by regulatory agencies [13].

EXAMINATION OF NEW DRUG APPLICATION

Medicine researchers must first submit an application to the FDA for an experimental innovative medicine The following must be included in the IND application by developers Information about clinical research techniques for planned trials in medication manufacture, data from preclinical and toxicological studies, and, if accessible, information about the inventor or investigator [14].

CLINICAL TRIALS

To give accurate information about the safety and effectiveness of medicines, vaccines, other treatments, or new ways to use existing treatments, clinical studies are done with volunteer made by the drug maker, researcher, or investigate. which is required before any clinical research can begin. They also think about their goals for each of the different stages of clinical research [15].

Phases Includes

- *Phase I:* Investigates a new medication's safety, side effects, and optimal dosage in a small group of 20–100 healthy volunteers, or occasionally patients. focuses on how the drug is metabolized in the body to identify the maximum safe dosage. Side effects and tolerability are routinely monitored.
- *Phase II:* determines the optimal dosage and monitors any adverse effects 100 to 300 patient involved.
- *Phase III:* 300 to 3000 patients is involves
- *Phase IV:* more realistic,everyday situations [16].

APPROACHES IN DRUG DISCOVERY AND DEVELOPMENT

- Multiple Target Agents Changing from "one-drug-one-target" to multi-target modulation (e.g., Alzheimer's drug possibilities) is a better way to treat complex diseases [17].
- Drug Repurposing Reusing licensed drugs (like thalidomide for multiple myeloma) saves time and money by avoiding early research [18].
- Allostery Modulation Drugs bind to particular allosteric sites (e.g., BQCA for schizophrenia models) to provide tailored targeting and minimize side effects [19].
- Organic Products Modified natural compounds are significant leads, but they must be optimized for drug-like properties [20].
- Products Made of Biologicals Although they are costly and challenging to produce, monoclonal antibodies, vaccines, and other therapies target specific proteins or genes [21].
- *Teamwork Techniques Crowdsourcing:* Early R&D innovation is fostered by partnerships between academia and industry. Network pharmacology looks at therapeutic synergies and disease-related protein networks for more intelligent targeting [22, 23].

CONCLUSION

Therapy, pharmaceutical repurposing, and multi-target modulation. In addition to increasing process efficiency, these ongoing developments increase the likelihood of developing novel, safe, and effective drugs for patients worldwide.

REFERENCES

1. Shayne CG. Introduction: Drug discovery in the 21st century. Drug Discovery Handbook. Wiley; 2005. p. 1–10.
2. Smith GC, O'Donnel JT. The Process of New Drug Discovery and Development. 2nd ed. New York: Informa Healthcare; 2006.

3. Moffat J, Vincent F, Lee J, Eder J, Prunotto M. Opportunities and challenges in phenotypic drug discovery: an industry perspective. Nat Rev Drug Discov. 2017;16(8):531–543.
4. DiMasi JA, Hansen RW, Grabowski HG. The price of innovation: new estimates of drug development costs. J Health Econ. 2003;151–185.
5. Lindsay MA. Target discovery. Nat Rev Drug Discov. 2003;2:831–838.
6. Terstappen G, Schlüpen C, Raggiaschi R, Gaviraghi G. Target deconvolution strategies in drug discovery. Nat Rev Drug Discov. 2007;6(11):891–903.
7. Peet NP. What constitutes target validation? Targets. 2003;2:125–127.
8. Imming P, Sinning C, Meyer A. Drugs, their targets and the nature and number of drug targets. Nat Rev Drug Discov. 2006;5:821–834.
9. Osakwe O. Chapter 6: Preclinical in vitro studies – development and applicability. In: Social Aspects of Drug Discovery, Development and Commercialization. Elsevier; 2016.
10. Patidar AK, Selvam G, Jeyakandan M, Mobiya AK, Bagherwal A, Sanadya G, et al. Lead discovery and lead optimization: a useful strategy in molecular modification of lead compound in analog design. Int J Drug Des Discov. 2011;2(2):458–463.
11. Huber W. A new strategy for improved secondary screening and lead optimization using high-resolution SPR characterization of compound-target interactions. J Mol Recognit. 2005;18:273–281.
12. Barile FA. Principles of Toxicological Testing. CRC Press; 2008.
13. Vogel HG. Drug Discovery and Evaluation. 2nd ed. Springer; 2002.
14. Karara AH, Edeki T, McLeod J, et al. PhRMA survey on the conduct of first-in-human clinical trials under exploratory investigational new drug applications. J Clin Pharmacol. 2010;50:380.
15. Fitzpatrick S. The Clinical Trial Protocol. Buckinghamshire: Institute of Clinical Research; 2005.
16. Advarra. Phases of clinical research: a detailed overview. Available from: https://www.advarra.com/blog/phases-of-clinical-research-a-detailed-overview.
17. Zhang HY. One-compound-multiple-targets strategy to combat Alzheimer's disease. FEBS Lett. 2005;579:5260–5264.
18. Azvolinsky A. Repurposing existing drugs for new indications. The Scientist. 2017 Jan 1.
19. Grover AK. Use of allosteric targets in the discovery of safer drugs. Med Princ Pract. 2013;22:418–426.
20. Robles O, Romo D. Chemo- and site-selective derivatizations of natural products enabling biological studies. Nat Prod Rep. 2014;31(3):318–334.
21. Morrow T, Felcone LH. Defining the difference: what makes biologics unique. Biotechnol Healthc. 2004;1(4):24–29.
22. Lessl M, Bryans JS, Richards D, Asadullah K. Crowdsourcing in drug discovery. Nat Rev Drug Discov. 2011;10(4):241–242.
23. Hopkins AL. Network pharmacology: the next paradigm in drug discovery. Nat Chem Biol. 2008;4(11):682–690.

