Proceedings of the 6th International Conference on Biological Engineering and Medical Science
DOI: 10.54254/2753-8818/2026.38460

From Cell Culture to Patient Benefit: Understanding Drug Development Through Regulatory Science

Mingshan Jiang

The Second High School Attached to Beijing Normal University, Beijing, China
15110261925@163.com

Abstract. The development of a new oncology drug is often described as a sequence of laboratory experiments, animal studies, clinical trials, regulatory approval, and reimbursement. However, this view does not explain why each stage is necessary or why promising therapies often fail. This paper argues that each stage addresses a distinct scientific question that previous evidence cannot fully answer. As development progresses from cell culture experiments to clinical trials, regulatory review, post-marketing surveillance, and reimbursement, the definition of a "successful" drug evolves from biological activity to meaningful patient benefit. Using regulatory practices in the United States, Japan, and China, this paper examines how different systems manage scientific uncertainty while protecting patient safety. Regulatory policies are understood not as isolated administrative procedures, but as responses to evidence gaps throughout drug development. Finally, the paper discusses how this perspective may improve oncology drug development in China through stronger evidence generation, earlier regulatory communication, and improved patient access without compromising scientific rigor.

Keywords: oncology drug development, regulatory science, evidence generation, translational medicine, patient access

1. Introduction

Recent developments in cancer biology, genomics, immunotherapy and precision medicine have led to an unprecedented pace of discovery of potential anticancer therapies. High-throughput screening, organoid models, antibody-drug conjugates and CAR-T therapy are examples of technologies that enable researchers to identify therapeutic targets quickly and with high accuracy. However, the development of new oncology drugs is still the most time-consuming, expensive and unpredictable process in contemporary medicine. Although many compounds exhibit promising lab activity, few receive official approval. Most candidate drugs fail between preclinical studies and late-stage clinical trials [1, 2]. This raises a crucial question: if a drug has already shown promising results in the laboratory, why does it require so many further steps before reaching patients?

The success in the lab is characterized by stopping tumors or killing cancer cells, which cannot be assured of clinical benefits. These biological responses are not enough to clinicians and patients, however. A therapy that has proven to shrink tumors might still not be able to increase survival time, enhance quality of life, decrease symptoms or create a reasonable ratio between benefit and toxicity.

© 2026 The Authors. This is an open access article distributed under the terms of the Creative Commons Attribution License 4.0 (https://creativecommons.org/licenses/by/4.0/).

DOI: 10.54254/2753-8818/2026.38460

As such, laboratory efficacy does not always result in significant patient benefit [3]. This changing meaning of success is the reason why oncology drug development goes through Oncology drugs need to have several phases: cell-based experiments, animal studies, clinical trials, regulatory review, post-marketing surveillance, and reimbursement assessment. The stages deal with unanswered scientific questions as opposed to being administrative procedures. Efficiency-driven policies have been initiated by global regulators. The United States has created accelerated programs including Breakthrough Therapy Designation, Fast Track, Accelerated Approval, and Priority Review to help achieve earlier access to promising therapies [4, 5].

In Japan, it is the early consultation and bridging studies that are considered in order to identify when the foreign clinical evidence can be applied to the Japanese people [6]. China has also been developing a system of priority review processes and conditional approval as an alternative to the accelerated development of new drugs and improved regulation.

This paper will not compare the systems of regulation, but it is a question about why all the stages of oncology drug development are present and what scientific uncertainty should be addressed in each of them. The approach to drug development as such can help to have a more consistent perspective on the innovation in regulatory matters and find new ways of enhancing the future oncology drug development within China.

2. Why does drug development require so many stages

2.1. From cell culture to biological evidence

The process of drug discovery starts with the most basic question: Is it possible to make a candidate compound generate the desired biological effect? Cell culture experiments thus examine whether a drug binds its target molecular, prevents tumor growth or modifies particular cellular pathways. These studies are also important in providing necessary mechanistic evidence and enable researchers to screen out compounds that exhibit minimal biological activity prior to proceeding to more elaborate experiments [2]. Nevertheless, positive results of laboratory experiments do not prove that a therapy will be useful to patients. Cultured cells under controlled conditions do not possess physiological complexity of living organisms such as immune responses, vascular systems, organ interactions and metabolic regulation. This means that only the question of whether a drug is biologically effective can be answered by laboratory studies instead of whether it is clinically effective.

2.2. From animal models to human patients

The next step of the evidence generation is the animal studies, which are the question in a different way: Can we use the results of the laboratory to a living organism? The advantage of using animals as models is that it enables the researchers to study pharmacokinetics, pharmacodynamics, systemic toxicity, metabolism and other interactions between various organs. Such experiments give us an idea about the functioning of a drug inside a biological system. However, there are still some issues that have not been addressed. The fact that animals are genetically distinct, they do not have the same immune systems, their tumors do not respond to drugs the same way, and so on, makes it impossible to predict what would happen to humans based on the results of the experiments with animals. Most promising results achieved in mice can be unsuccessful in human trials due to the inability of the mouse physiology to reflect all aspects of human disease [1]. Thus, the uncertainty will never go away by means of animal research.

DOI: 10.54254/2753-8818/2026.38460

2.3. From clinical efficacy to patient benefit

It is still in the process of clinical trials, and the questions that are asked are becoming more patient-centered. The first phase involves the measurement of safety and dosage of the drug whereas the second phase measures the effectiveness of the drug to be used and the third phase determines whether the drug will work on a larger scale and with a broader population. Notably, it is important to note that the concept of success is not fixed at these levels. The initial research in the laboratory is concerned with biological processes but the later one tends to consider results directly related to the patients such as the survival rate, progression-free survival, toxicity of the therapy, improvement of symptoms, quality of life [3]. As a result, a medicine which can effectively kill cancer cells in vitro does not necessarily offer adequate clinical efficacy so as to warrant its use by many people. The development of drugs is thus not just an advancement of the experimental process, but rather a slow shift of the evidence based on biology to the patient-based evidence.

3. How do regulatory systems reduce scientific uncertainty

3.1. Determining when evidence is sufficient

Even with the successful clinical trials, regulators need to answer another important question: Has enough evidence been generated to justify approval? Regulatory agencies must balance two competing priorities providing timely access to innovative therapies while protecting patients from ineffective or unsafe treatments. To address this challenge, the U.S. Food and Drug Administration (FDA) has established several expedited programs including Breakthrough Therapy Designation, Fast Track, Accelerated Approval, and Priority Review. Rather than lowering scientific standards, these programs aim to improve communication between regulators and developers, prioritize promising therapies for serious diseases, and allow earlier patient access while requiring continued evidence generation after approval [4, 5].

3.2. Can evidence be shared across populations

With the increase in globalization of pharmaceutical research, a different question has also been posed: Can clinical evidence developed in one population be used in another? Separate Phase III clinical trials would take much longer to reach patients and development costs would skyrocket. Nonetheless, direct application of foreign clinical evidence can fail to consider significant ethnic variations in genetics, epidemiology of disease, medical practice, and drug metabolism. In order to overcome this challenge, International Council for Harmonisation (ICH) came up with E5 guideline which focuses on the acceptability of foreign clinical data but taking into account possible ethnic factors that might affect response to treatment [6]. Instead of demanding absolute replication of all clinical trials, the guideline promotes scientific assessment of whether available evidence can reasonably justify regulatory actions across populations. This is an important example offered by Japan.

The PMDA has created a consultation system in advance of the clinical trials, which means that the regulators and developers can consult with each other on the evidence needed before they are put into practice. When there is an international data that is already believed to be scientifically sound, it is possible to conduct bridging studies as well, in order to identify whether or not a limited amount of further evidence is required in Japan patients and avoid the unnecessary duplication of clinical research [7]. This is one more rule of regulatory science that this method demonstrates. It is not

DOI: 10.54254/2753-8818/2026.38460

about making sure that all the uncertainties will not be addressed prior to the approval but rather about what kind of uncertainty needs new evidence and what can be resolved by a careful scientific analysis of the available evidence. As a result, the efficiency of regulation is attained not through the diminution of the level of science but through the diminution of repetition.

3.3. Does regulatory approval mean the end of evaluation

The granting of marketing authorization is usually seen as the last stage in drug development. In fact, approval is a transition between controlled clinical research and routine clinical practice, with many important questions left unanswered. Clinical trials are carried out under strictly controlled conditions and often include highly selected patient populations. As a result, rare adverse events, long-term safety issues, treatment adherence, and effectiveness in larger patient populations may not be evident until thousands of patients begin using a newly approved therapy. For this reason, post-marketing pharmacovigilance, real-world evidence (RWE), and post-approval confirmatory studies have become increasingly significant components of modern regulatory science [5]. Many expedited regulatory pathways reflect this philosophy. Programs such as Accelerated Approval permit patients with serious diseases earlier access to promising therapies based on surrogate clinical endpoints but require manufacturers to continue generating confirmatory evidence after approval [4].

These programs are not a replacement of the scientific work but they transfer the generation of evidence to all stages of a medicine. The same ideas have been implemented in China, where conditional approval and the development of new drugs is done on a regular basis and is followed by an ongoing process of regulation that aims at increasing the pace of innovation and ensuring the safety of the patients [7]. It is however not sufficient to approve the regulatory measures and be sure that the patient will get any benefit out of it. Post-approval there are further tests such as health technology assessment (HTA) or reimbursement analysis, cost-effectiveness study and healthcare resource distribution to determine the extent to which the innovative therapies are introduced into the healthcare systems. This means that the last step of the drug production is no longer the one with the focus on science but the one that has the focus on the patients. There can never be a successful medicine, even though it has proven its clinical efficiency, when it is not available due to high price, slow payment, and low coverage of the medical facilities.

In other words, the goal of developing a drug in oncology is not only to gain approval but also to make it available to the patients.

4. Discussion

4.1. Drug development is a process of answering scientific questions

The conclusion that I have come up with in this review is the fact that oncology drug development cannot be perceived as a series of increasingly complicated experiments. Rather, all these steps are there since they answer the question of a different scientific issue which is not answered at the previous step. Cell culture research questions the activity of a therapy and animal studies determine the application of these findings on a living organism. Clinical trials test clinical benefit to patients and tolerable safety. The evidence has been evaluated by regulatory authorities to decide whether it should be used more broadly or not, whereas post-marketing surveillance was conducted to evaluate long-term use and infrequent adverse effects. The reimbursement systems also measure the value of approved therapies to the point where they can be invested in healthcare extensively. Therefore, the process of developing drugs is complicated because the issues of science are complex and not due to

DOI: 10.54254/2753-8818/2026.38460

the superfluous administrative measures. It will also give an insight into why laboratory discoveries are promising but fail when it comes to clinical development: the level of proof required varies.

4.2. Regulatory policies exist because scientific uncertainty exists

Comparing regulatory systems also indicates that individual policies should be considered not as stand-alone administrative innovations, but as approaches to managing scientific uncertainty. For instance, Breakthrough Therapy Designation responds to the desire for earlier access to promising therapies for patients with life-threatening diseases. Bridging studies were created to avoid repeating identical clinical trials in each country when such repetition might add limited scientific value to high-quality international evidence. Post-marketing surveillance reflects the recognition that some safety questions cannot be resolved prior to extensive clinical use. Similarly, reimbursement evaluations recognize that proving clinical efficacy does not necessarily demonstrate societal value. Instead of removing all uncertainty, modern regulatory systems seek to decide when the available evidence is adequate to enable informed decision making while continuing to generate further evidence post-approval where appropriate.

This view takes the focus off regulatory speed per se and onto enhancing efficiency of evidence generation across the whole lifecycle of a medicine.

4.3. Implications for future drug development in China

China has achieved a significant progress through priority review, conditional approval and involvement in the international clinical research. However, future regulatory reform may gain less by directly imitating individual foreign policies and more by comprehending why those policies were originally created. The communication between regulators and developers earlier could make evidence requirements clear and unnecessary studies fewer. More use of real-world evidence would enhance post-marketing evaluation and allow selected therapies to reach patients faster. Better coordination between regulatory review, health technology assessment and reimbursement can reduce the time lag between approval and patient access. A greater focus on patient-reported outcomes and quality of life could also make sure that regulatory decisions are more representative of what is important to patients.

5. Conclusion

It is not a process of cell culture and then the patient treatment, but rather it is an endless stream of experiments and regulatory measures with the ultimate aim to decrease the level of scientific uncertainty in the process. The stages are different questions that are answered by the previous studies which can be answered in part only. This view helps understand why the systems of regulation take various policies even though they share the same purpose of public health. The innovations of the regulatory field like fast-track, bridging study, conditional approval and post-marketing surveillance should hence be regarded as the scientific ways of dealing with the uncertainty and ensuring the safety of patients. In China, there are still areas where oncology drug development needs to be improved so that it does not only reduce the time of approval, but also enhance the quality, consistency and effectiveness of evidence generation. In fact, it is not about the drugs being passed through the processes of the regulators, but about how the facts on the basis of science can be converted into something useful for the patient.

DOI: 10.54254/2753-8818/2026.38460

References

[1] Hay, M., Thomas, D. W., Craighead, J. L., Economides, C., & Rosenthal, J. (2014). Clinical development success rates for investigational drugs. *Nature Biotechnology, 32*(1), 40–51. https://doi.org/10.1038/nbt.2786

[2] Paul, S. M., Mytelka, D. S., Dunwiddie, C. T., Persinger, C. C., Munos, B. H., Lindborg, S. R., & Schacht, A. L. (2010). How to improve R&D productivity: The pharmaceutical industry's grand challenge. *Nature Reviews Drug Discovery, 9*(3), 203–214. https://doi.org/10.1038/nrd3078

[3] Blumenthal, G. M., Kluetz, P. G., Schneider, J., Goldberg, K. B., McKee, A. E., & Pazdur, R. (2017). Oncology drug approvals: Evaluating endpoints and evidence in an era of breakthrough therapies. *The Oncologist, 22*(7), 762–767. https://doi.org/10.1634/theoncologist.2017-0152

[4] Kern, K. A. (2016). Trial design and efficacy thresholds for granting breakthrough therapy designation in oncology. *Journal of Oncology Practice, 12*(8), e810–e817. https://doi.org/10.1200/JOP.2016.012161

[5] Michaeli, D. T., Michaeli, T., Albers, S., Boch, T., & Michaeli, J. C. (2024). Special FDA designations for drug development: Orphan, fast track, accelerated approval, priority review, and breakthrough therapy. *The European Journal of Health Economics, 25*(6), 979–997. https://doi.org/10.1007/s10198-023-01639-x

[6] International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use. (1998). E5(R1): Ethnic factors in the acceptability of foreign clinical data. https://database.ich.org/sites/default/files/E5_R1__Guideline.pdf

[7] Franco, P., Jain, R., Rosenkrands-Lange, E., Hey, C., & Koban, M. U. (2023). Regulatory pathways supporting expedited drug development and approval in ICH member countries. *Therapeutic Innovation & Regulatory Science, 57*(3), 484–514. https://doi.org/10.1007/s43441-022-00480-3