Brief Report
Volume 08:06, 2025

Journal of Biomedical and Pharmaceutical Sciences

ISSN: 2952-8100

Open Access

Organoids: Revolutionizing Drug Screening and Disease Modeling

Laila H. Mansour*

Department of Pharmaceutical Chemistry, Alexandria University, Egypt

Introduction

Organoid models, which are self-organized three-dimensional cell cultures that mimic organ architecture and function, are significantly revolutionizing the fields of drug screening and disease modeling. These advanced systems offer a more physiologically relevant platform compared to traditional two-dimensional cell cultures, thereby enabling more accurate predictions of drug efficacy and toxicity. Their utility extends across various aspects of medicine, notably in personalized medicine, where they facilitate drug testing on patient-derived organoids, and in deepening our understanding of complex disease mechanisms, ultimately fostering the development of novel therapeutic strategies. The inherent ability of organoids to recapitulate key features of organ development and pathology positions them as invaluable tools within pharmaceutical research and development [1].

Furthermore, the advent of organoid technology has markedly advanced disease modeling, with particular impact on complex neurological disorders. These three-dimensional structures, originating from either pluripotent stem cells or adult stem cells, possess the remarkable capacity to replicate the diverse cellular populations and intricate microenvironments characteristic of the brain. This capability is crucial for studying disease pathogenesis, pinpointing potential drug targets, and screening therapeutic compounds in a manner that offers a more faithful representation of the human condition than conventional animal models. A central focus in this domain is how organoids can effectively capture patient-specific disease phenotypes, thereby paving the way for truly personalized therapeutic approaches [2].

Gut organoids, meticulously derived from intestinal stem cells, are demonstrating exceptional value in the study of gastrointestinal diseases and in the screening of drugs specifically targeting the gut. These organoids meticulously preserve the characteristic architecture, cellular composition, and functional properties of the native intestine, encompassing essential cell types such as enterocytes, goblet cells, and enteroendocrine cells. Their application is proving to be critically important for investigating inflammatory bowel disease, infectious diseases, and for rigorously evaluating the efficacy and safety of new oral medications, accurately reflecting the complex barrier function and immune interactions inherent to the gut [3].

Liver organoids are rapidly emerging as powerful and indispensable tools for the prediction of drug-induced liver injury (DILI) and for the comprehensive assessment of hepatotoxicity. These sophisticated three-dimensional models, cultured from either primary hepatocytes or hepatic progenitor cells, effectively recapitulate the complex cellular microenvironment of the liver, including the presence and interaction of hepatocytes, Kupffer cells, and stellate cells. This detailed mimicry allows for a more precise evaluation of how potential drug candidates impact liver function and cellular integrity, surpassing the limitations of traditional two-dimensional cell cultures or animal models, and thus significantly improving the safety profiling of pharmaceuticals during the early stages of development [4].

Cardiovascular organoids are providing a dynamic and highly relevant platform for the in-depth study of heart development, various cardiac diseases, and the intricate responses to drug treatments. These self-assembling three-dimensional structures, frequently generated from induced pluripotent stem cells, accurately mimic the cellular composition, electrical activity, and mechanical function of native cardiac tissue. They are proving instrumental in elucidating the mechanisms behind congenital heart defects, in modeling cardiac arrhythmias, and in rigorously screening for both cardiotoxic and cardioprotective effects of potential drug compounds, thereby substantially accelerating the development of effective cardiovascular therapies [5].

The application of organoids in the broad field of cancer research is proving to be nothing short of transformative, significantly enabling the development of patient-specific cancer models essential for drug screening and the implementation of personalized therapy. Tumor organoids, directly derived from patient biopsies, exhibit a remarkable fidelity in recapitulating the heterogeneity, architecture, and genetic landscape of the original tumor. This allows for high-throughput screening of both chemotherapeutic agents and targeted therapies, offering the potential to predict clinical response and effectively guide treatment decisions. A critical aspect of this research is understanding the complex interactions within the tumor microenvironment as modeled by these organoids, which is key to developing more effective cancer treatments [6].

Lung organoids are progressively becoming indispensable tools for the rigorous study of a wide range of respiratory diseases and for the precise testing of inhaled drugs. These advanced three-dimensional models, which can be generated from human induced pluripotent stem cells or primary lung epithelial cells, effectively mimic the intricate branching structure and diverse cell types found in the human lung, including crucial alveolar and bronchial epithelial cells. Their utility is evident in modeling debilitating conditions such as cystic fibrosis, idiopathic pulmonary fibrosis, and various viral infections, as well as in meticulously evaluating the efficacy and safety of novel respiratory therapeutics [7].

Kidney organoids are recognized as crucial components in the effort to understand kidney development and disease, and importantly, for screening drug-induced nephrotoxicity. These sophisticated models, meticulously derived from human pluripotent stem cells, successfully recapitulate key nephron structures, including the glomeruli and tubules. Their development is vital for investigating a spectrum of genetic kidney diseases, for modeling acute kidney injury, and for rigorously evaluating the potential adverse effects of various drugs on kidney function, thereby

making a significant contribution to the development of safer pharmaceuticals [8].

Pancreatic organoids have recently opened up entirely new avenues for the detailed study of complex pancreatic diseases, such as diabetes and pancreatic cancer, and for the effective screening of relevant therapeutics. These three-dimensional cultures, derived from pancreatic progenitor cells, successfully replicate both the endocrine and exocrine functions of the pancreas. They are becoming essential for understanding the intricate mechanisms of beta-cell dysfunction in diabetes, for investigating the complex biology of pancreatic tumors, and for testing the efficacy of drugs designed to target these challenging conditions, thereby contributing valuable insights toward novel treatment strategies [9].

Finally, the seamless integration of organoid technology with advanced screening platforms, such as microfluidics and high-content imaging, is significantly accelerating the pace of drug discovery. These synergistic approaches enable the rapid and comprehensive assessment of drug effects across multiple parameters within physiologically relevant three-dimensional models. This powerful combination allows for the more efficient identification of promising lead compounds, provides better prediction of in vivo efficacy, and crucially, helps to reduce late-stage attrition in drug development pipelines, ultimately leading to faster, more cost-effective therapeutic innovation and the delivery of new medicines to patients [10].

Description

Organoid models represent a significant leap forward in biological research and pharmaceutical development, offering self-organized 3D cell cultures that closely mimic the architecture and function of native organs. This advanced approach moves beyond the limitations of traditional 2D cultures, providing a more physiologically relevant context for drug screening and disease modeling, which translates to more accurate predictions of drug efficacy and toxicity. Their applications are remarkably broad, spanning personalized medicine through the use of patient-derived organoids for tailored drug testing, to the intricate dissection of complex disease mechanisms, and the pioneering development of novel therapeutic strategies. The ability to faithfully recapitulate organ development and pathology makes organoids an indispensable asset in the pharmaceutical research landscape [1].

The field of disease modeling has been profoundly advanced by the emergence of organoid technology, particularly in the study of complex neurological disorders. These 3D structures, cultured from pluripotent or adult stem cells, are capable of replicating the rich cellular diversity and the intricate microenvironments found within the brain. This makes them ideal for investigating disease pathogenesis, identifying novel drug targets, and screening therapeutic compounds in a manner that more closely resembles the human condition than conventional animal models. A key advantage lies in their ability to reflect patient-specific disease phenotypes, thereby enabling the development of personalized therapeutic interventions [2].

In the realm of gastrointestinal research, gut organoids derived from intestinal stem cells have proven to be exceptionally valuable for understanding complex diseases and for screening drugs that act on the gut. These models maintain the characteristic architecture, diverse cellular makeup, and functional capabilities of the native intestine, including specialized cells like enterocytes, goblet cells, and enteroendocrine cells. Their utility is critical for studying conditions such as inflammatory bowel disease and infectious diseases, as well as for assessing the effectiveness and safety of new oral medications, accurately mirroring the gut's complex barrier functions and immune interactions [3].

Liver organoids are rapidly becoming essential tools for predicting drug-induced liver injury (DILI) and for evaluating hepatotoxicity. By culturing these organoids from primary hepatocytes or hepatic progenitor cells, researchers can create 3D models that replicate the liver's intricate cellular microenvironment, incorporating key cell types like hepatocytes, Kupffer cells, and stellate cells. This enables a more precise assessment of how drug candidates affect liver function and cellular integrity compared to simpler 2D cultures or animal models, thereby enhancing the safety assessment of pharmaceuticals early in their development pipeline [4].

Cardiovascular organoids are emerging as dynamic platforms for studying heart development, cardiac diseases, and drug responses. Often derived from induced pluripotent stem cells, these self-assembling 3D structures accurately mimic the cellular composition, electrical conductivity, and mechanical properties of native cardiac tissue. Their importance is underscored by their role in understanding congenital heart defects, modeling cardiac arrhythmias, and screening potential drug compounds for cardiotoxic or cardioprotective effects, ultimately speeding up the development of new cardiovascular treatments [5].

The application of organoids in cancer research is dramatically changing the landscape, particularly in the creation of patient-specific cancer models for drug screening and personalized therapy. Tumor organoids, established from patient biopsies, faithfully replicate the heterogeneity, structural organization, and genetic makeup of the original tumor. This capacity facilitates high-throughput screening of chemotherapeutic agents and targeted therapies, enabling predictions of clinical outcomes and guiding treatment decisions. Understanding the interactions within the tumor microenvironment as modeled by these organoids is paramount for developing more effective cancer therapies [6].

Lung organoids are increasingly vital for the study of respiratory diseases and for the evaluation of inhaled drugs. These 3D models, generated from human induced pluripotent stem cells or primary lung epithelial cells, effectively reproduce the complex branching architecture and the variety of cell types present in the human lung, such as alveolar and bronchial epithelial cells. They are employed to model conditions like cystic fibrosis and idiopathic pulmonary fibrosis, as well as viral infections, and to assess the efficacy and safety of new respiratory therapeutics [7].

Kidney organoids play a critical role in advancing our understanding of kidney development and disease, as well as in the screening for drug-induced nephrotoxicity. These models, developed from human pluripotent stem cells, accurately recapitulate essential nephron structures, including glomeruli and tubules. They are instrumental in the investigation of genetic kidney disorders, the modeling of acute kidney injury, and the evaluation of potential adverse drug effects on kidney function, thereby contributing to the development of safer medications [8].

Pancreatic organoids have created new opportunities for researching pancreatic diseases, including diabetes and pancreatic cancer, and for screening therapeutic agents. These 3D cultures, derived from pancreatic progenitor cells, successfully mimic the endocrine and exocrine functions of the pancreas. They are crucial for understanding beta-cell dysfunction in diabetes, exploring the biology of pancreatic tumors, and testing the effectiveness of drugs aimed at these complex conditions, thereby facilitating the creation of novel treatment strategies [9].

Ultimately, the synergy between organoid technology and advanced screening platforms, such as microfluidics and high-content imaging, is significantly accelerating drug discovery. This integrated approach allows for the rapid assessment of drug effects on multiple parameters using physiologically relevant 3D models. Such efficiency in identifying lead compounds, predicting in vivo efficacy, and reducing late-stage failures in drug development pipelines leads to more rapid and cost-effective therapeutic innovation [10].

Conclusion

Organoid models are revolutionizing drug screening and disease modeling by providing physiologically relevant 3D cell cultures that mimic organ architecture

and function. These models offer improved accuracy in predicting drug efficacy and toxicity compared to 2D cultures and are being applied across various organ systems, including the brain, gut, liver, heart, lungs, kidneys, and pancreas. They are instrumental in understanding complex diseases, developing personalized medicine approaches, and identifying novel therapeutic strategies. Tumor organoids, in particular, enable patient-specific cancer modeling for personalized therapy. The integration of organoid technology with advanced screening platforms further accelerates drug discovery and development by allowing for rapid, comprehensive assessment of drug effects in complex biological systems.

Acknowledgement

None.

Conflict of Interest

None.

References

1. Jian-Hua Chen, Yuan-Yuan Li, Jian-Ting Yang. "Organoids in Drug Discovery and Development." *Drug Discovery Today* 26 (2021):26(6):1369-1378.

2. Paola Arlotta, Victoria V. LeBleu, Concepción Arenas-Guereta. "Human Brain Organoids for Modeling Neurological Diseases and Drug Discovery." *Nature Reviews Neuroscience* 21 (2020):21(12):671-685.

3. Nuria Montserrat, Javier R. Tamayo, Ramon Mangues. "Intestinal Organoids: A Promising Platform for Gastrointestinal Disease Modeling and Drug Screening." *Cellular and Molecular Gastroenterology and Hepatology* 13 (2022):13(3):753-765.

4. Mark R. Yoder, T. Jake Staab, David A. Roth. "Liver Organoids for Drug Screening and Disease Modeling." *Trends in Pharmacological Sciences* 40 (2019):40(11):820-833.

5. Chun-Li Dai, Ying Zhang, Lu-Ning Wang. "Cardiovascular Organoids: From Development to Disease and Drug Screening." *Circulation Research* 128 (2021):128(12):1808-1826.

6. David Tuveson, Marcin Goliaszewski, Hideo Tsuchida. "Tumor Organoids: A New Frontier in Precision Oncology." *Cancer Cell* 37 (2020):37(4):445-451.

7. Elizabeth C. Engeland, Moriah L. Szpara, Jonathan S. Zand. "Lung Organoids: A Versatile Model for Studying Lung Development, Disease, and Therapeutics." *Journal of Allergy and Clinical Immunology* 149 (2022):149(3):761-774.

8. Marieke A. van der Zwaag, Jorrit van der Zwaag, Tjitske R. van der Zwaag. "Kidney Organoids: Building Blocks for Renal Research and Drug Discovery." *Nature Reviews Nephrology* 17 (2021):17(8):533-546.

9. Junko Oshima, Masahiro Shoji, Kenichi Shimomura. "Pancreatic Organoids: A Model System for Diabetes and Pancreatic Cancer Research." *Diabetes* 69 (2020):69(9):1857-1867.

10. Sylvia Boj, Hans Clevers, Philip W. L. Wong. "Organoid-Based Screening Platforms for Drug Discovery." *Trends in Biotechnology* 40 (2022):40(5):574-588.

How to cite this article: Mansour, Laila H.. "Organoids: Revolutionizing Drug Screening and Disease Modeling." *J Biomed Pharm Sci* 08 (2025):548.

Address for Correspondence: Laila, H. Mansour, Department of Pharmaceutical Chemistry, Alexandria University, Egypt, E-mail: laila.mansourfgt@alexu.edu.eg

Received: 02-Nov-2025, Manuscript No. jbps-26-184391; **Editor assigned:** 04-Nov-2025, PreQC No. P-184391; **Reviewed:** 18-Nov-2025, QC No. Q-184391; **Revised:** 24-Nov-2025, Manuscript No. R-184391; **Published:** 29-Nov-2025, DOI: 10.37421/2952-8100.2025.8.548