Innovations in Translational Research: Organoids as Versatile Tools for Therapeutic Development

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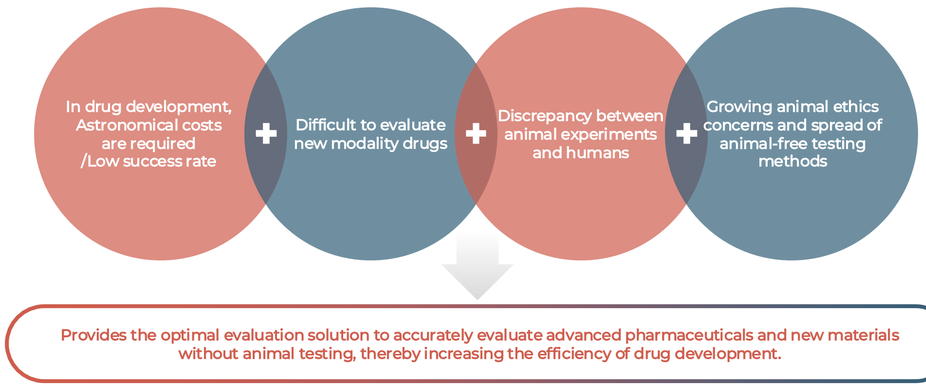
Background & Purpose

On April 10, 2025, the U.S. FDA and the European Medicines Agency (EMA) jointly announced a phased withdrawal of mandatory animal testing for drug development, opening the door for validated, human-relevant in vitro models. In this new regulatory landscape, *organoid technology* has emerged as a transformative tool in translational research.

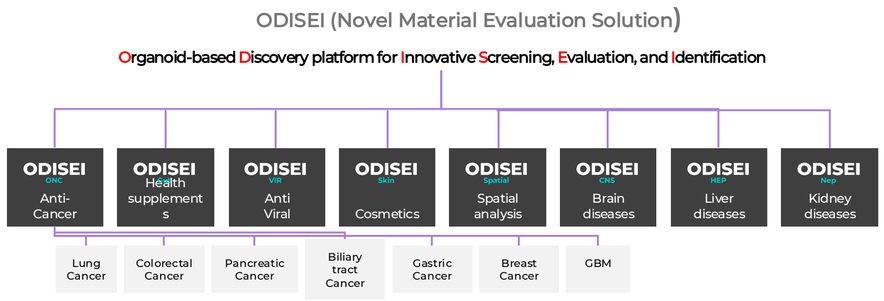
Organoids have long been recognised as physiologically relevant models that replicate patient-specific drug responses with remarkable fidelity. Numerous studies have demonstrated that organoid-based assays closely mirror clinical outcomes, offering a powerful alternative to traditional cell lines and animal models.



ODISEI (Organoid-based Discovery platform for Innovative Screening, Evaluation, and Identification)

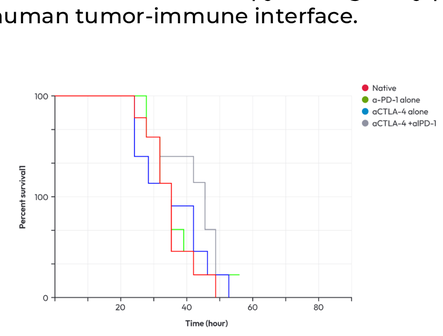
ORGANODISCIENCES has developed a wide array of **efficacy evaluation models** tailored to specific therapeutic modalities and disease contexts. These models support preclinical decision-making by simulating the complex interactions between tissues, immune cells, and microbiota.
At the core of this effort is our proprietary **ODISEI platform**, the **Organoid-based Discovery platform for Innovative Screening, Evaluation, and Identification**. ODISEI integrates human organoids with co-culture systems and advanced analytical workflows to provide:

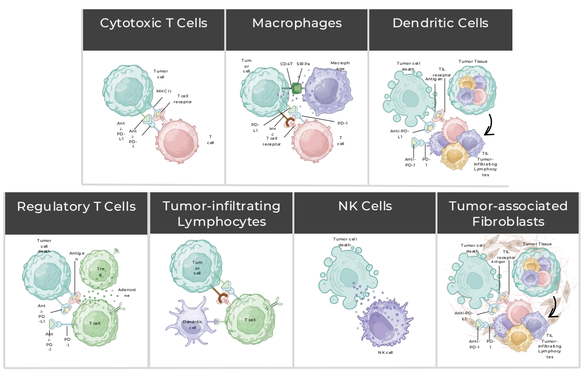
ODISEI designed to model patient-specific biology and drug response with unprecedented accuracy. The ODISEI pipeline supports applications from high-throughput drug screening to personalized therapeutic response modeling, offering a credible alternative to animal models and conventional 2D assays.



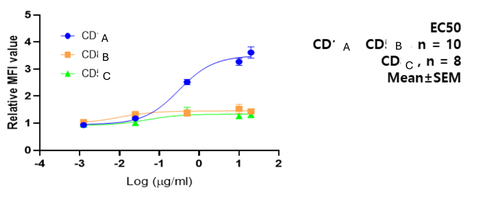
Immuno-Oncology Drug

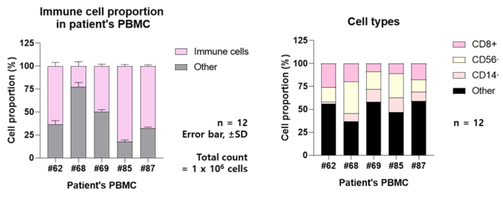
Immune checkpoint inhibitors have demonstrated robust antitumor activity in preclinical animal models, contributing significantly to the development of immuno-oncology drugs. However, fundamental differences in immune system regulation and tumor microenvironment between animals and humans often limit the translational relevance of these models. In particular, they fail to adequately reflect immune-related adverse events and the variability of patient-specific immune responses.
To overcome these limitations, we have developed a co-culture platform utilizing patient-derived tumor organoids and autologous peripheral blood mononuclear cells (PBMCs). By leveraging matched tumor and immune cells from the same patient, this system enables direct evaluation of drug efficacy and personalized immune responses. This approach facilitates the development of precision immunotherapy strategies by providing a clinically relevant model that closely mimics the human tumor-immune interface.





Beyond evaluating drug efficacy alone, the organoid-based ODISEI_ONC platform enables comprehensive immune profiling before and after drug treatment. This includes the quantification of immune cell populations, analysis of surface marker expression, and profiling of activation or exhaustion markers. By integrating these immunological endpoints with functional tumor response data, the platform provides a multidimensional view of how therapeutic agents modulate both the tumor and the immune microenvironment. Such detailed immunophenotyping enhances mechanistic understanding and supports the selection and optimization of immuno-oncology strategies tailored to patient-specific responses.





The ODISEI_ONC platform leverages patient-derived cancer organoids co-cultured with immune cells to bridge the gap between conventional preclinical models and real-world patient heterogeneity. This system enables detailed investigation of tumor-immune interactions and allows for the personalized evaluation of drug responses across diverse therapeutic contexts.

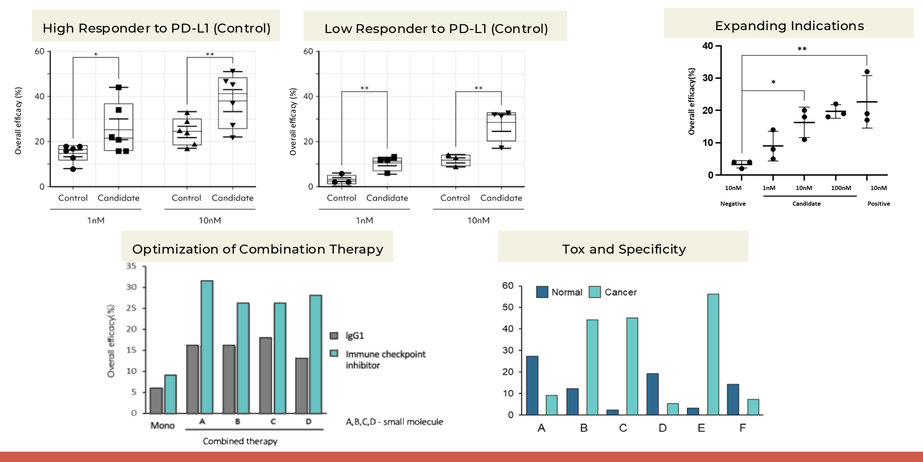
Key differentiators of the platform include:

- **Patient heterogeneity modeling**: By using matched patient samples, the platform reflects individual immune responses, enabling personalized therapy testing.
- **New combination strategies**: Beyond monotherapy evaluation, the system supports the testing of dual, triple, or SM (small molecule) combinations, offering new possibilities for refractory cancers.
- **Emerging modalities**: The platform allows preclinical validation of novel approaches such as cancer vaccines, mRNA therapeutics, and bispecific antibodies.
- **Immune-oncology integration**: Through real-time co-culture of immune cells and tumor organoids, mechanisms of immune activation and anti-tumor response can be directly visualized and analyzed.

Representative case applications include:

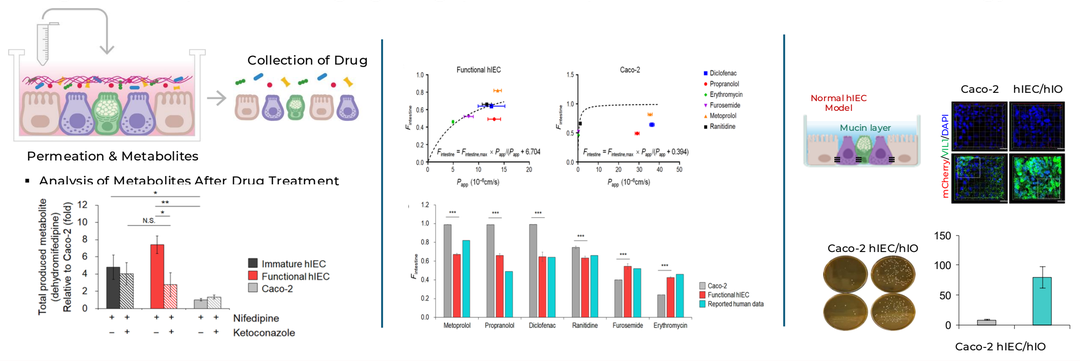
- Case 1: Screening optimal **combination therapies** in patient-specific contexts
- Case 2: Evaluation of **bispecific antibody** efficacy
- Case 3: Testing **new modalities** such as cancer vaccines, MDSC-targeted agents, or mRNA-based therapies
- Case 4: **Differentiating between drugs** with similar clinical outcomes
- Case 5: Supporting **indication expansion** through mechanistic validation
- Case 6: Providing human proof-of-concept (**POC**) data prior to **IND filing**

Moreover, the platform has demonstrated strong performance in validating agents that show superior efficacy over existing drugs and has been instrumental in predicting combination therapy responses in a patient-specific manner. These capabilities position the ODISEI_ONC platform as a powerful translational tool for drug discovery, precision oncology, and clinical decision-making.



Microbiome

The gut organoid platform, an advanced human intestinal model, enables precise evaluation of drug absorption and permeability. When tested using this system, results showed a high degree of similarity to human clinical data, significantly outperforming the conventional Caco-2 model in predictive accuracy. This highlights its value as a reliable tool for assessing drug permeability, metabolism, and overall pharmacokinetic behavior in a physiologically relevant environment.
In addition to pharmaceutical applications, the gut organoid model plays a critical role in the development of health functional foods and probiotic products. The platform allows for the quantification of lactic acid bacteria adhesion to the intestinal epithelium using fluorescence visualization and smear methods. Furthermore, it facilitates detailed analysis of the interaction between probiotics and intestinal epithelial cells, supporting the scientific validation of efficacy and safety before entering clinical or consumer use. This dual capability positions gut organoids as a next-generation platform bridging the gap between preclinical studies and human biology.



Cosmetic

Research into the mechanisms of active compounds for hair loss treatment has long been hindered by the translational gap between in vitro cell-based assays and clinical studies. To address this issue, a physiologically relevant evaluation platform was needed—one that can effectively bridge the gap between basic research and human application. While 3D hairy skin models and skin explants have been considered, their commercialization remains technically limited.
In this study, we aimed to develop a hair-bearing organoid model capable of evaluating the efficacy of hair loss prevention agents, particularly in the context of androgenic alopecia (male pattern baldness). Using embryonic stem cell-derived organoids that form nascent hair follicles, we investigated the effects of dihydrotestosterone (DHT) and soybean embryo extract—two agents known to modulate hair growth and loss.
Our findings confirm the viability of hair-bearing organoids as a reliable, translational model. The observed congruence between organoid-based and tissue-level responses underscores the utility of organoids in dermatological research. In particular, soybean embryo extract demonstrated consistent efficacy in preventing DHT-induced hair loss and promoting follicular regeneration.
This pioneering work supports the use of organoids not only as preclinical screening tools but also as potential platforms for hair transplantation research. The scalability, ethical viability, and physiological relevance of organoids mark a significant step forward in developing next-generation therapies for hair loss.