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Organoid Models for Preclinical Drug Screening: A Systematic Review Protocol

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We use this protocol and it's working

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Keywords: organoid models for preclinical drug screening, preclinical drug screening, organoid model, organoid type, organoid, assays with conventional preclinical model, toxicity testing, based assay, conventional preclinical model, animal model, applications in efficacy

Abstract

To describe organoid types, experimental methods, and applications in efficacy and toxicity testing. To compare organoid-based assays with conventional preclinical models (2D cultures, animal models). To identify methodological challenges, reproducibility issues, and potential improvements for translational use.

Guidelines

- All records will be imported into Rayyan.ai for blinded screening.
- **Step 1**: Title and abstract screening by two independent reviewers.
- **Step 2**: Full-text review to confirm eligibility.
- Discrepancies will be resolved by discussion or a third reviewer.
- Selection will be summarized using a **PRISMA 2020 flow diagram**.

Quality Assessment / Risk of Bias

- *In vitro studies*: Checklist adapted from *ToxRTool* or OECD standards.
- *In vivo studies*: *SYRCLE's Risk of Bias tool.*
  Each study will be rated as *low, unclear, or high* risk of bias.

Data Synthesis

- *Narrative synthesis*: Results will be summarized descriptively and organized by organoid type, drug category, and outcome.
- *Quantitative synthesis*: A meta-analysis will *not* be conducted due to expected heterogeneity in study designs and outcomes.
- *Tables and figures* will present study characteristics, outcomes, and gaps.

Materials

A structured Excel sheet will be used to extract the following data:
- Author, year, country
- Study design (in vitro / in vivo)
- Type and source of organoid (tissue, species)
- Purpose of study (efficacy, toxicity, etc.)
- Methods of drug screening
- Comparators used
- Main results and limitations

Objectives

1 Primary Objective: To systematically review and synthesize current evidence on the use of organoid models for preclinical drug screening between 2015 and 2025.

2 Secondary Objectives:

2.1 To describe organoid types, experimental methods, and applications in efficacy and toxicity testing.

2.2 To compare organoid-based assays with conventional preclinical models (2D cultures, animal models).

2.3 To identify methodological challenges, reproducibility issues, and potential improvements for translational use.

Research Question (PICO)

3

Element	Description
P (Population/Problem)	Organoid models used in preclinical drug screening (derived from human or animal tissues; both in vitro and in vivo).
I (Intervention/Exposure)	Use of organoid models for evaluating drug efficacy, toxicity, pharmacokinetics, and pharmacodynamics.
C (Comparator)	Conventional preclinical models, such as 2D cell cultures, animal models, or other 3D culture systems.
O (Outcomes)	Predictive accuracy, reproducibility, translational relevance, scalability, limitations, and

Research Question: What are the applications and challenges of organoid models in preclinical drug screening for evaluating drug efficacy and toxicity in vitro and in vivo?

Eligibility Criteria

4 Inclusion Criteria

Population: Studies using human- or animal-derived organoid models applied in preclinical drug screening or testing.
Intervention: Any drug exposure, compound testing, or therapeutic evaluation (efficacy, toxicity, pharmacokinetics, pharmacodynamics).
Comparator: Studies that include 2D cultures, animal models, patient-derived xenografts (PDX), or no comparator if the design is justified.
Outcomes: Must report quantitative or qualitative outcomes related to drug efficacy, toxicity, pharmacodynamics, or predictive performance.
Study Design: Experimental or preclinical studies conducted in vitro or in vivo.

5 Exclusion Criteria
Population: Studies not involving organoid models (e.g., 2D cultures, spheroids, organ-on-chip, or animal-only experiments).
Intervention: Studies not related to drug testing, e.g., focusing only on disease modeling, stem cell differentiation, or genetics.
Study Design: Reviews, commentaries, case reports, conference abstracts, or theoretical studies without experimental data.
Document Type: Full text unavailable or data insufficient for extraction.

Information Sources

6 Electronic databases:
- **PubMed / MEDLINE**
- **Scopus**
- **Embase**
- **Web of Science**
- **Cochrane Library**

Grey literature sources:
- **bioRxiv, medRxiv, ProQuest Theses, Google Scholar, and conference proceedings.**

Reference lists of all included studies will be screened manually to identify additional relevant publications.

Search Strategy

7 ("organoid"[Title/Abstract] OR "organoids"[Title/Abstract] OR "3D organoid" [Title/Abstract] OR "mini-organ"[Title/Abstract]) AND ("drug screening"[Title/Abstract] OR "drug discovery"[Title/Abstract] OR "preclinical screening"[Title/Abstract] OR "preclinical testing"[Title/Abstract]) AND ("2015/01/01"[Date - Publication] : "2025/12/31" [Date - Publication]) NOT (review[Publication Type] OR "systematic review"[Title])
Equivalent search strings will be adapted for Embase, Scopus, and Web of Science.

Study Screening and Selection

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Data Extraction

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- Purpose of study (efficacy, toxicity, etc.)
- Methods of drug screening
- Comparators used
- Main results and limitations
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Quantitative synthesis: A meta-analysis will *not* be conducted due to expected heterogeneity in study designs and outcomes.
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Timeline

12

Stage	Estimated Completion
Protocol writing	November 2025
Database search	December 2025
Screening & extraction	January–February 2026
Analysis & synthesis	March 2026
Manuscript preparation	April 2026

Registration

13 This protocol will be registered on **OSF Registries**, as the topic is primarily preclinical and may not qualify for PROSPERO registration. Any future amendments will be documented and justified according to PRISMA 2020 guidelines.

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