

2. Organoid Models

Organoid and 3D models offer advanced platforms to investigate drug response in human-like systems. These cell-based tools improve the relevance of efficacy & safety studies. Organoid models are increasingly used to reduce dependency on traditional models & reduce costs while preserving scientific integrity & translational value.

4. Material & Excipient safety

All materials & excipients used in production must be generally recognised as safe (GRAS). This includes testing for biological compatibility, purity & stability. Use of pharmacopeia materials can help to ensure suitable purity & safety, and are essential in both drug substance & drug production in order to meet regulatory & quality expectations for product safety.

1. Mode of Action

Understanding the mode of action (MoA) is foundational in preclinical development. This involves characterising how a therapeutic interacts at cellular or molecular levels. Clear insights into MoA support safety, efficacy & potency assessments, guiding the design of relevant in-vitro & in-vivo models.

3. Safety & Efficacy

Preclinical studies assess the balance between safety & efficacy using accepted methodologies. The data generated can help to identify appropriate dosing ranges & detect potential side effects. Generating reliable data at this stage supports ethical progression into clinical trials as well as building regulatory confidence.

5. Pharmacokinetics (PK) & Pharmacodynamics (PD)

Pharmacokinetics (PK) and pharmacodynamics (PD) modelling provides insight into Absorption, Distribution, Metabolism & Excretion (ADME). PK/PD data supports dose selection & helps predict human response. These studies are key for mechanistic understanding & clinical trial design, aiding key decisions.

6. Early Positive Preclinical Data

Early indicators of efficacy, safety & MoA from preclinical studies guide the development strategy and attract investment. Robust early data builds confidence, supports regulatory engagement as well as informing stage-gate, Go/No-Go decisions. Clarity at an early stage helps align timelines & expectations for stakeholders.

