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Human Cells Are Rewriting the Rules of Drug Development

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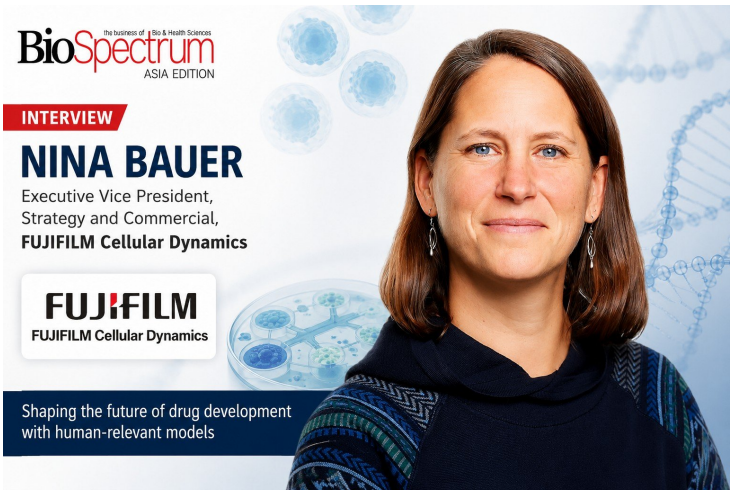
Nina Bauer of FUJIFILM Cellular Dynamics explains why the convergence of iPSCs, organoids, AI and regulatory momentum could accelerate the pharmaceutical industry's shift from animal models towards more predictive, scalable and human-relevant drug development.



Drug development is approaching a significant inflection point. Advances in induced pluripotent stem cells (iPSCs), organoids and organs-on-chips are converging with artificial intelligence and a rapidly changing regulatory environment to challenge pharmaceutical R&D's longstanding dependence on animal models.

In this conversation with BioSpectrum Asia, Nina Bauer discusses what is driving the rise of non-animal methods, where human-relevant models are already making an impact, and why the next competitive frontier could lie in combining scalable human biology with AI to fundamentally rethink how medicines are discovered, tested and brought to patients

Non-animal methods (NAMs) have gained significant momentum in recent years. What scientific, regulatory, and technological developments have finally enabled this shift away from traditional animal models?

The shift towards NAMs represents a convergence of scientific validation, technological maturity, and regulatory modernization. One milestone was an FDA-led workshop in 2013 that brought to life an initiative named CiPA (comprehensive in vitro proarrhythmia assay). It proved truly groundbreaking in that it ultimately demonstrated that human iPSC-derived cardiomyocytes, combined with in silico modeling, could predict cardiac safety better than animal models. Importantly, the regulators accepted it. This proof-of-concept opened the doors to the development of what is now commonly thought of as NAMs.

In parallel, we have reached a genuine critical mass of off-the-shelf iSPC-derived cell types and organoid systems, which are now standardized, scalable, and available across multiple tissues. Overall, this is making broad screening and toxicology testing in human cells practical instead of aspirational.

Lastly, regulatory support has been transformative over the past few years, with the FDA Modernization Act 2.0 in 2022 eliminating the animal testing mandate, explicitly recognizing NAMs as valid alternatives. The EMA followed suit with 3Rs

implementation strategies and guidance on iPSC-based models, while the MHRA created expedited pathways for NAMs submissions post-Brexit. And in the last few weeks, China's NMPA has announced that they would accept validated NAMs for specific endpoints.

We are at a significant inflection point, where the underpinning scientific rigor exists, the technology is accessible and scalable, and regulators globally are not just accepting but actively encouraging these human-relevant approaches. It is fundamentally changing how we think about drug development.

Induced pluripotent stem cells (iPSCs) are increasingly viewed as the foundation of many next-generation human-relevant models. What unique advantages do iPSCs offer over conventional in vitro systems, and where are they delivering the greatest impact today?

iPSCs are becoming the backbone of the next-generation human-relevant models because they solve three of the biggest limitations of conventional in vitro systems. First, they are created off of stable, scalable master cell banks that can be differentiated on demand into many different cell types. This means you can generate large numbers of consistent, well-characterized cells with the same genetic background, and actually industrialize assays rather than relying on more fragile primary tissue or poorly defined immortalized lines.

Second, they allow us to capture human genetics much more faithfully. We can build panels of iPSC lines that reflect diverse patient genotypes, specific disease mutations, or even rare variants, and then read out differential responses in a way that animal models or generic lines simply can't.

Third, the combination of iPSCs with gene editing tools such as CRISPR has dramatically accelerated model creation. Engineering isogenic disease and control lines can be done in weeks to months, instead of the year or more needed to generate and backcross a comparable rat or mouse model.

We see the impact of these technologies everywhere. In addition to the CiPA cardiac safety measures, which have been incorporated into International Council for Harmonization guidelines, developers are using iPSC-based CNS disease modeling and neurotoxicity assays, liver metabolism and DILI (drug-induced liver injury) risk assessments, and increasingly, multi-organ microphysiological systems, where consistent and defined human cells are essential.

These NAMs allow for high-throughput screening approaches at the discovery stage of drug development, for off-target effect testing by assessing a compounds impact on individual cell types and tissues or a gene therapy's on-target specificity, and, finally, can be used in routine quality control and release testing once a drug has been approved. \

Human organoids and organs-on-chips are transforming preclinical research. How are these technologies improving disease modelling, drug discovery and toxicity assessment, and which therapeutic areas are seeing the most rapid adoption?

Human organoids, assembloids (composed of defined ratios of specific cell types), and organs-on-a-chip are transforming preclinical research by giving us 3D, multicellular systems that much more closely recapitulate human tissue architecture, function, and microenvironments than flat (2D) cell cultures or animal models.

For disease modeling, these technologies let us recreate patient-specific and genetically defined pathologies, some examples being tumor microenvironments, neurodevelopmental disorders, and fibrotic lung pathologies. These can be leveraged to observe human-relevant molecular, cellular, and disease mechanisms in real time.

In drug discovery, these platforms support more predictive efficacy screens and target validation, including complex readouts like barrier integrity, electrophysiology, immune-tumor interactions, and even organ-to-organ crosstalk, in more complex linked "multi-organ" chips.

For safety testing, we now have access to more sensitive and mechanistic toxicity assessments, for example human liver, kidney, cardiac, and brain models that can capture delayed toxicities and idiosyncratic ones that are unpredictable because they're either patient-specific or rare, all of which are often missed in animals.

The broadest availability of in vitro models is in the cardiac and neuronal space (based on the availability of off-the-shelf cells), lending itself to those disease areas. However, it would be too limiting to say that these are the areas of broadest adoption due to the relevance in early discovery, as all disease areas and therapeutic developments leverage cardio and neurotox models to understand safety.

From a mechanistic/mode of action perspective, some of the fastest adoption is seen in oncology (tumor organoids and immune co-cultures), neurology and psychiatry (brain organoids and assembloids), cardiometabolic disease (heart, liver, and vascular chips), and respiratory and GI indications, where organoid biobanks and standardized chips are already being integrated into pharma pipelines and early regulatory dialogues. Many of these do not rely on iPSC-derived cells alone, often adding primary cells or immortalized lines.

AI is becoming an integral part of biomedical research. How do you see AI and iPSC-derived models complementing one another, and what new opportunities could this convergence unlock for drug development?

AI and iPSC-derived models are highly complementary as they each solve a critical weakness of the other. AI is undoubtedly very powerful, but fundamentally limited by the lack of extensive, comprehensive, high-quality datasets – the so-called “garbage in, garbage out” problem. Most historical drug discovery data is sparse, heterogeneous, and biased toward non-human systems. There is also almost no negative data available, of experiments that have not worked.

iPSCs, by contrast, can now provide a stable, scalable, and genetically well-defined source of human cells across tissues and patient genotypes, generating cleaner, more comprehensive, and longitudinal datasets.

Therefore, by pairing standardized iPSC models and platforms with high-content readouts and multimodal omics, we can now create exactly the kind of rich, high-quality training data that AI requires. This will enable us to build out digital twins and over time move early discovery, as well as novel molecular and chemical design, into an in silico environment. I would expect much more accurate prediction of human efficacy and toxicity, as well as the assessment of dose and combination options. Iterative design-test-learn cycles, which today take a long time and are a key limiting financial factor in drug development, can be moved to an AI model to propose candidates or mechanisms that can then be quickly validated in iPSC-based systems, thus closing the loop between computation and biology.

Despite the progress, what are the biggest scientific, manufacturing or regulatory challenges that still need to be addressed before NAMs become the global standard across pharmaceutical R&D?

Despite the rapid progress over the last several years, a number of hurdles remain before NAMs can become the global default approach for drug development. Scientifically, many models still lack full tissue complexity, including vascularization, immune components, stromal interactions, and the ability to mimic chronic exposure. It is therefore important to develop better integrated systems, and clearer links between complex in vitro readouts and their clinical outcomes. Considering iPSC-based NAMs specifically, scaling them into robust, cost-effective, GLP compliant products with tight QC, batch-to-batch consistency, and standard manufacturing protocols, is still a work in progress – especially if we consider that these cells need to be frozen in a manner that maintains their functionality and viability post-thaw.

Regulators are increasingly supportive, but for the most part global harmonization is still missing, which leads to an uneven acceptance across regions and decision points.

In addition, validation is also considered a central bottleneck for broad adoption. We need robust, specific evidence to demonstrate that NAMs can be as predictive, reliable, and reproducible as the existing animal models we are intending to replace. This also depends on the “context of use”, which has to be clearly defined and demonstrated. We still need standardized protocols, multi-site trials, well-designed concordance studies against historical animal and clinical data like CiPA, and transparent performance metrics.

For global harmonization, ICH/OECD-style frameworks between the different regulatory agencies might help with streamlining development and data acceptance. Without this level of rigorous, shared validation, NAMs will remain attractive supplements rather than true replacements, especially for high-stakes decisions like first-in-human-enabling toxicology and release testing.

Looking ahead over the next five years, which innovations or industry trends do you believe will most accelerate the adoption of iPSC-based platforms, and what should biopharma companies invest in today to remain competitive?

With the current level of regulatory drive and innovation, the next five years will be marked by three main trends.

First, iPSC-based NAMs will experience a significant increase in maturity and standardization, which will be marked by the commercial availability of many more cell and especially tissue types, higher functional maturity, as well as GLP-aligned, ready-to-use assay kits.

In addition, cell manufacturing and NAMs application will see an increased level of automation. It will enable cheaper and faster cell product development (think differentiation protocols) and optimized cell manufacturing (think fully closed and automated with in-process controls), but also high content assay performance and their read-outs, as well as associated hardware tools. This will be enabled by AI, using iPSCs as more consistent “data generators” for both target discovery and safety pharmacology, but also for the development of digital twins.

Lastly, as regulatory comfort with iPSC-based drug development grows, we should see a gradual replacement of animal-based testing and increased adoption overall.

To stay competitive, pharma needs to stay informed on the growing body of regulatory acceptance so they can move in lockstep with these developments. While some might want to develop in-house capabilities for their more routine needs, building a strong partnership approach with trusted suppliers will be critical. Internal assay capabilities built on industrialized workflows using robotics and automation will be key.

Beyond this, building out analytical and bioinformatics capabilities that can dissect the increasing amounts of data in a meaningful way should be a core focus area. Ensuring that data is captured, structured, and stored in an interrogatable manner will be an invaluable competitive advantage so that AI and machine learning can be fully leveraged. Lastly, proactive regulatory engagement around specific iPSC use cases can ensure that relevant platforms have been validated, are scalable, and credible, once they are ready for adoption.

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