



#06 Organoid-based functional assays for efficacy and toxicity assessment in relevant human disease models in neuroscience and rare diseases



EXPECTED DELIVERABLE

Indicative duration: 6 – 9 months

Development of human organoid models (e.g., brain organoids, liver organoids or others—depending on partner capabilities) to evaluate:

- **Pharmacological efficacy** of Ipsen compounds on biological pathways of interest (e.g., proliferation, apoptosis, differentiation, network activity, secretion).
- **Structural and functional toxicity**, using 3D morphometry, viability, imaging, and functional readouts.
- Differential sensitivity between healthy and disease-specific organoids (when applicable).

The project may include (to be adapted by the partner based on expertise):

- Establishment or optimization of organoid generation protocols (iPSC-derived or patient-derived, according to ethical requirements).
- Development of a robust analytical pipeline: 3D morphological features: size, density, lumen formation, polarity
- Functional biomarkers: calcium imaging, metabolic activity, electrophysiology, secretion assays
- High-content or high-throughput 3D imaging
- Exploratory testing of Ipsen reference and test molecules to assess model sensitivity and translational relevance.



LONG-TERM COLLABORATION POTENTIAL

Subject to scientific and strategic alignment

Human organoid models represent a strategic opportunity for Ipsen in several key therapeutic areas:

- Neuroscience: brain organoids to study neurotoxicity or efficacy in human-relevant neurological phenotypes.
- Rare diseases: iPSC-derived pathological organoids to explore disease mechanisms and therapeutic response in small patient populations.

If the model demonstrates robustness and translational value, Ipsen would be highly interested in developing a long-term collaboration, including:

- expanding internal organoid biobanks,
- creating scalable efficacy/toxicity testing platforms,
- co-developing disease-specific organoid models with strong predictive value.



CANDIDATE SELECTION

Initial eligibility check by MPR. Final selection by the challenge provider.



Completion of EDUCATE

Core Module



Company status

SME under EU criteria



Maximum number of supported companies

1 – 3



Minimum required TRL

5–6 minimum (functional models established with preliminary validation data)



Confidentiality
NDA/ CDA required

Yes

Selection by the challenge provider based on fit, relevance, readiness and innovation potential.

Additional selection criteria



Geographic
area

SME from across EU are welcome.
SMEs from Interreg NWE are prioritized,
particularly partner regions

Applicants with expertise in human organoid culture, functional analysis capabilities, 3D imaging access and long-term culture capacity will be positively considered.



APPLICATION

Application directly via the STEP4NAMs Moodle platform



<https://step4nams.moodlecloud.com/>



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