Partner Search Form
Horizon Europe
Health

Date 01/07/2025 Deadline … …

CONTACT

Organisation	Axxam s.p.a.	**Department**	Scientific Innovation
Contact person	Silvia Cainarca	**Email**	silvia.cainarca.sc@axxam.com
City	Milan	**Website**	Axxam.com
Country	Italy		

Organisation type

Research organisation type	☐ Research Organisation ☐ University **X** Company ☐ Other	**Is your company a Small and Medium Sized Enterprise (SME*)?** **Number of employees:**	☐ YES **X** NO

Your enterprise is an SME if:
- it is engaged in **economic activity**
- it has **less than 250 employees**
- it has either an **annual turnover not exceeding €50M, or an balance sheet total not exceeding €43M**
- it is **autonomous**

For the definition of SMEs, look at: http://ec.europa.eu/growth/smes/business-friendly-environment/sme-definition_en

Short introduction of key areas of institute's research:
Axxam is an innovative research and discovery organization providing early discovery services across the life sciences industry. Our mission is to support clients and partners transforming ideas into actionable discoveries and translating innovative biology into bioactive molecules. We strive to push the boundaries of science to solve tough challenges and make a meaningful difference in people's lives.
We have a remarkable experience on European projects granted by the European Commission both as coordinator and as partner.

Former participation in an FP European project?	**X** YES ☐ NO
Project title / Acronym:	European Commission – Horizon 2020-Innovative Medicines Initiative 2 (IMI2): Title: REsolution – Add medical genetic solutions to RESOLUTE
Activities performed:	Start: June 2021-ongoing European Commission – Horizon 2020-Innovative Medicines Initiative 2 (IMI2): Title: ReSolute – Research empowerment on solute carriers Start: July 2018-ongoing Here you can find the complete list of secured grants

Partner Search Form
Horizon Europe
Health

Expertise / Commitment offered

Description of your expertise:

We are a leading provider of integrated discovery across Life Sciences industries with a major focus on pharmaceuticals, and with access to novel modalities such as VHH/nanobodies.
We have consolidated expertise across a broad range of discovery disciplines and innovative technologies including biophysical, biochemical and cell-based assay development, high-content image-based screening, electrophysiology on whole cells and on isolated organelles, in vitro disease modelling, including use of recombinant and patient-derived iPSC cells, virtual, synthetic and natural compound collections, high-throughput screening (HTS) and compound profiling, hit identification, validation, and medicinal chemistry-assisted hit-to-lead and lead optimization.

EXPERTISE OFFERED FOR THE SPECIFIC CALL:

HORIZON-HLTH-2025-01-TOOL-01: Enhancing cell therapies with genomic techniques

- **Advanced target identification and validation:** Deep biological expertise aimed at the identification and validation of novel therapeutic targets, including challenging and "undruggable" classes, and at identifying the pathophysiological mechanism of medical conditions. Support to pre-clinical and clinical research studies is achieved through cutting-edge CRISPR/Cas9 genome editing technologies, and functional validation in model systems bearing increasing level of complexity, from cell-free biophysical and biochemical assays and cell-based assays on tailored recombinant cell lines, to physiologically relevant human iPSC-derived cell models.
- **Innovative assay development:** Designing robust, high-sensitive and disease-relevant in vitro assays—both cell-free and cell-based—tailored for high-throughput and high-content image-based compound testing and screening, structure-activity relationship (SAR) analysis and hit-to-lead optimization. Development and configuration include clinically relevant, non-human and human model systems and advanced assays using recombinant and patient-derived human iPSC-derived cells in 2D and 3D cultures, enabling disease-relevant phenotypic readouts and precise genome editing for target modulation to complement clinical investigations.
- **Medicinal chemistry, compound libraries and comprehensive solutions for identification and characterization of bioactive compounds and biologicals.** Access to diverse and innovative compound collections, including: (1) A proprietary collection of drug-like molecules of 450.000 compounds, including sub-libraries tailored for specific purposes e.g., collection of covalent compounds, RNA-binders, protein-protein interaction, CNS-favoured scaffolds; (2) AI-assisted virtual library of 19M readily available chemical entities; (3) Design, screening and evolution of target-directed VHH/nanobodies, to offer alternative modalities for specific therapeutic indications.
- Medicinal chemistry, cheminformatics, and AI-supported molecular modelling assist hit identification, characterization and progress through the lead optimization process. We integrated automated high-throughput screening of compounds and biomolecules (in 384/1536-well formats)

	with genetic screening—including phenotypic, functional, and omics approaches (e.g., RT-qPCR, high-content imaging)—to rapidly identify and characterize promising candidates with suitable bioactivity and safety profiles for developing new diagnostics, therapeutics, or preventive strategies. Low-, mid-, and high-throughput electrophysiology assay development, compound testing and screening on ion channels and protein targets eliciting signal transduction roles across plasma membrane and intracellular organelles, i.e., mitochondrial, lysosomal, nuclear target proteins. • **Integrated hit validation:** Supporting hit follow-up with mechanism of action studies applying functional enzymology and in vitro pharmacology, and with pathophysiological relevant models, advanced molecular and phenotypic assays, multi-parametric data analyses, target engagement studies, biophysical, biochemical, cellular and electrophysiological technologies and in vitro ADMET characterization to support informed decisions at early stage on favourable features for clinical development of compounds under investigation. • Among the others, Axxam has developed advanced assays targeting serotonin receptors and transporters, as well as critical modulators of neurodevelopment and behavior. • Axxam expertise includes studying the neuronal excitatory/inhibitory imbalance in ASD. Using patient-derived iPSC neuronal we perform electrophysiological and phenotypic assays to elucidate functional disruptions and screen for therapeutic modulators. • This integrated approach enables comprehensive mechanistic insights and supports the development of preclinical and clinical candidates targeting the complex neurobiology of ASD and related disorders
Keywords specifying your expertise:	1. Novel target identification and validation for cell therapies. 2. Study of disease mechanisms to inform therapy. 3. Genome and epigenome editing of therapeutic cells. 4. iPSC and 2D/3D models for disease study. 5. Cell assays, imaging, and electrophysiology (including MEA). 6. HTS, hit validation, hit to lead 7. Virtual/synthetic compound libraries and medicinal chemistry. 8. AI, data analysis, and automation. 9. Omics and biomarkers identification for therapy and diagnosis. 10. Preclinical and clinical development support, including ADMET and mechanism of action studies
Commitment offered:	X Research ☐ Demonstration ☐ Training X Technology ☐ Dissemination ☐ Other:

Interested in participation in project types:			
	X Research & Innovation Action	**X** Innovation Action	X EIC Pathfinder

Work Programme research areas: indicate your interest

Partner Search Form
Horizon Europe
Health

Cluster Health

Do you have other partners for this topic (which partners/country)?	No yet

Profile of partner sought

Role	**X** technology development	**X** research	☐ training
	☐ dissemination	☐ demonstration	☐ other _____
Country /region	☐ Italy		
Expertise required	We would like to offer our technical expertise to a consortium		

I agree with the publication of my contact data: **X** YES ☐ NO