



Axxam S.p.A. collaborates with GenSight Biologics S.A. to support Clinical Trials of an optogenetic-based gene therapy drug for sight-threatening diseases

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Axxam S.p.A. announced today a collaboration with GenSight Biologics S.A. in the field of optogenetic gene-therapy drug development, with the goal to support GenSight effort of establishing innovative therapies to preserve or restore vision in patients suffering from sight-threatening ophthalmic diseases, such as Retinitis Pigmentosa.

GenSight is an innovative clinical-stage gene therapy company with an initial focus on discovering, developing and commercializing novel therapies for severe retinal neurodegenerative diseases.

Its unique know-how in using optically controlled gene products for orchestrating cellular functions in miniaturized HTS-compatible functional assays, made Axxam the research partner of choice for the development of a cell-based potency assay to be used to validate GenSight GS030 drug product batches for the use in clinical-stage experimentation.

Axxam started the collaboration with GenSight with the establishment of a miniaturized *in-vitro* assay aimed at robustly and quantitatively measuring the biological effects linked to GS030 therapeutic properties, namely photoactivated cellular excitability. The potency assay, specially developed by Axxam for GenSight, is now ready to process for the method qualification in order to meet the regulatory requirements for clinical and further commercial stages.

Within the framework of the collaboration, Axxam will continue to collaborate and support GenSight throughout its GS030 drug product manufacturing stages by providing technological development and support to transfer, qualify and validate this new *in vitro* based assay which will serve for testing of production lots, and the quantitative assessment of quality and consistency of future drug product batches manufactured by GenSight.

The exact pharmacological assay and financial terms of this collaboration were not disclosed.

This is the first time that Axxam collaborates in a Chemistry, Manufacturing, and Control (CMC) phase of drug development process for international regulatory submissions, confirming its expertise in conducting strictly controlled and high quality technological investigations.

The project also represents a remarkable achievement for Axxam investments in innovative technologies, demonstrating its capabilities in conducting HTS-grade experimentations which use optogenetic tools to support drug discovery and development process.

Dr. Stefan Lohmer (Chairman and CEO of AXXAM) commented: *“We are proud to collaborate in such a pioneering drug development program, the impressive scientific commitment and CMC process management by GenSight inspires and motivates our efforts in validating our optogenetic assay platform to assist HTS operations and drug product development. The fact that we are contributing to one of the first optogenetic drug approvals make us even more convinced that optogenetics will shine light on the future of human medical needs”*



About Axxam S.p.A.

Axxam is an innovative Partner Research Organization (iPRO). We are a leading provider of integrated discovery services across Life Sciences industries including: pharmaceuticals, crop protection, animal health, cosmetics, fragrances, food and beverages. We have world-class expertise across a broad range of discovery disciplines and innovative technologies including: assay development, high-throughput screening of both the Axxam high quality compound collections (synthetic and natural) or those provided by our clients, compound management, hit identification and hit validation.

In recent years, Axxam has been engaged in several international programs for applied research and development projects with the goal of developing novel assets of HTS-compatible optogenetic assays to study cellular excitability.

Our performance-driven approach is recognized by our clients as key to the success for their discovery programs. Axxam is also engaged in alliance based research towards innovative small molecule therapies for diseases with a high unmet medical need. Axxam's business terms are flexible, ranging from fee-for-service to risk-sharing deal structures. For more information, please visit www.axxam.com.

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