XXV Encuentro de Cooperación Farma-Biotech

Jueves, 3 de julio de 2025

El Programa Farma-Biotech tiene por objeto establecer un punto de encuentro para la cooperación entre compañías farmacéuticas nacionales e internacionales, empresas españolas de biotecnología y grupos de investigación, en torno al desarrollo de nuevos medicamentos innovadores.

La presente jornada, que hace la número 25 desde que se inició el programa en el año 2011, está enfocada a la presentación de proyectos de medicamentos innovadores desarrollados en distintos centros de investigación y pequeñas empresas *spin-offs*, que han sido seleccionados por su potencial y prometedor estado de desarrollo, dentro del ámbito de la investigación preclínica.

En esta jornada se presentarán y discutirán **siete propuestas** que se considera han alcanzado un **grado de madurez** razonable, lo que permite estudiar posibles **acuerdos de cooperación** con la industria farmacéutica en condiciones ventajosas técnico-económicas. Consecuentemente, pensamos que esta jornada reviste especial interés para las compañías farmacéuticas invitadas, incluyendo responsables de sus **unidades de desarrollo de negocio** e inversiones.

El grado de información manejado durante la jornada se clasifica como “no confidencial” por lo que no se requiere ningún acuerdo previo al respecto.

La jornada se configura como un foro individualizado no abierto a terceras partes, y en donde se desea generar un **clima de interacción** suficiente que permita identificar el valor añadido derivado del intercambio de información entre demanda y oferta, con suficiente contenido diferencial e innovador en el ámbito de las nuevas terapias y los medicamentos avanzados.

Esta jornada tendrá lugar de forma presencial en la Delegación de Farmaindustria en Cataluña. Para cualquier duda o aclaración adicional por favor contactar con:

Amelia Martín Uranga

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Tfno. 915159350

Agenda

El programa Farma-Biotech, patrocinado por FARMAINDUSTRIA, pretende dar énfasis tanto a las presentaciones como a la interacción personal entre los asistentes, de acuerdo con la siguiente agenda:

Hora	Presentación	Ámbito terapéutico	Ponente
09:00	Recepción, contactos informales, café		
09:30 10:00	*Bienvenida y presentación de la jornada*	***Fina Lladós*** Presidenta de FARMAINDUSTRIA ***Robert Fabregat*** Director general de BIOCAT	
10:00 10:30	*Del-01 ATG4B, Oligonucleótido antisentido para tratar ELA*	SNC	***Pascual Torres Cabestany*** UNIVERSIDAD DE LLEIDA
10:30 11:00	*Terapia basada en RNA circular para el tratamiento de enfermedades incurables*	Varias patologías	***Miriam Corredor*** NOCTURNA THERAPEUTICS
11:00 11:30	*Biomarcadores diagnósticos de hipertensión portal*	Enfermedad hepática	***Sergi Guixé Muntet*** CIBER/IDIBAPS/H.CLINIC
11:30 12:00	*Células CAR-T autólogas contra CD1a en leucemia de células T con recaída o refractaria*	Oncología	***Wilmar Castillo Ávila*** ONECHAIN IMMUNOTHERAP.
	DESCANSO. Café y refrescos. Contactos informales		
12:15 12:45	*Oxapi-27, terapia epigenética first-in-class para desórdenes del Sistema Nervioso Central*	SNC	***Christian Griñán Ferré*** UNIVERSIDAD DE BARCELONA
12:45 13:15	*Onconeovac, una inmunoterapia personalizada para el melanoma maligno*	Melanoma maligno	***Olga Rué Clarós*** NEMA HEALTH
13:15 13:45	*Nuevos analgésicos de acción dual*	Dolor postoperatorio	***Fermín Goytisolo*** UNIVERSIDAD DE BARCELONA
	FINAL. Aperitivos y refrescos. Contactos informales		

Todas las presentaciones se harán en español, si bien la documentación escrita se dispondrá en inglés para facilidad de circulación interna entre los órganos de las compañías internacionales

Lugar de celebración: **sede de Farmaindustria en Barcelona. Avda. Diagonal, 514, planta 1ª**

Fecha: **jueves día 3 de julio de 2025**

La Plataforma Tecnológica Española Medicamentos Innovadores cuenta con la Ayuda (PTR2024-002838) financiada por MCIU/AEI/10.13039/501100011033

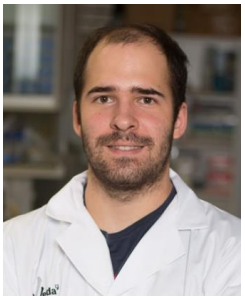
PRESENTACIONES QUE TENDRÁN LUGAR EN ESTA JORNADA

PROFILE

Universitat de Lleida

The **Metabolic Physiopathology Group** at Universitat de Lleida–IRBLleida investigates how metabolism and oxidative stress—including redox biology and antioxidant systems—contribute to aging and age-related diseases. Using both animal models and human studies, the group explores altered aging conditions and neurodegenerative diseases. In ALS, special emphasis is placed on metabolic and RNA dysregulation to uncover novel therapeutic strategies.

SPEAKER



Pascual Torres *is a Postdoctoral researcher with over a decade of experience in neurodegeneration, particularly ALS, and a strong focus on RNA metabolism and antisense-based therapeutic strategies. Lead inventor of a patented antisense oligonucleotide for ALS, developed during a research stay at the University of Oxford. A track record in translational research, with 25 publications and leadership in collaborative therapeutic development projects.*

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PRODUCT

Del-01 ATG4B, a novel antisense oligonucleotide to treat ALS.

MECHANISM OF ACTION

Del01-ATG4B is a 25-mer phosphorodiamidate morpholino oligonucleotide (PMO) designed for exon-skipping at the TDP-43 binding site of the cryptic exon in the ATG4B gene. The PMO is conjugated to the Del01 cell-penetrating peptide (CPP) via amide linkage through a 5′-amino linker, enhancing cellular uptake and endosomal escape. The Del01 peptide, with its positive charge, further promotes efficient RNA binding by electrostatic interaction, optimizing the oligonucleotide's delivery and efficacy in target cells.

TDP-43 dysfunction leads to the inclusion of ATG4B cryptic exon, disrupting its normal expression and impairing autophagic activity. By promoting exon-skipping, Del01-ATG4B restores normal ATG4B function, thereby correcting the autophagy defect. ATG4B has been shown to be crucial for the survival of motor neurons in preclinical ALS models, highlighting its therapeutic potential. The conjugated Del01 cell-penetrating peptide enhances delivery, allowing efficient cellular uptake and endosomal escape, improving the therapeutic potential across various TDP-43-related conditions.

TARGET INDICATIONS

Del01-ATG4B is primarily intended for the treatment of amyotrophic lateral sclerosis (ALS), both sporadic and familial forms, by targeting a TDP-43-related splicing defect. Its mechanism also holds therapeutic potential for other neurodegenerative diseases characterized by TDP-43 pathology, such as frontotemporal dementia (FTD), LATE (limbic-predominant age-related TDP-43 encephalopathy), and select cases of Alzheimer's disease.

CURRENT STATUS

- The PMO sequence of Del01-ATG4B effectively suppresses cryptic exon inclusion, normalizing transcript expression in cells with TDP-43 loss-of-function, a key pathological feature in ALS.
- In vivo, intracisternal administration in mice showed good safety and biodistribution, with predominant accumulation in spinal cord neurons, nuclear localization, and no histopathological damage in brain or spinal cord.
- Acute toxicity studies (1-week follow-up) revealed only mild, transient clinical effects at high doses.
- These results support both the molecular mechanism and delivery approach for central nervous system targeting.

INNOVATIVE ASPECTS

- Compared to existing treatments like Tofersen, an ASO with less than 5% eligibility in ALS patients, Del01-ATG4B shows a 97% eligibility rate.
- QRL-201, a first-in-class candidate in phase 1, targets the STMN2 cryptic exon, important in ALS. Both therapies focus on exon-skipping but target different cryptic exons, with Del01-ATG4B offering enhanced cellular uptake and improved pharmacokinetics due to its CPP-PMO design. These therapies could be complementary, targeting distinct pathological processes in ALS.
- Riluzol, the only drug approved for sporadic ALS (95% of cases), offers minimal benefit, extending survival by only 3-6 months.

IPR

The technology is protected under a priority patent application (PCT/EP2024/079389), filed internationally on 17 October 2024. The patent is co-owned 50/50 by Oxford University Innovation Limited and Universitat de Lleida.

PARTNERING OPPORTUNITIES

We are seeking strategic partnerships to support preclinical development and advance Del01-ATG4B toward clinical trials, with potential licensing or co-development opportunities.

PROFILE

NoctuRNA develops first-in-class therapies based on circRNAs for the effective treatment of currently uncurable diseases, including infectious and genetic disorders. Our circRNAs are designed to specifically target secondary RNA structures critical to the progression of several diseases. The platform has been validated against viral infections such as Covid19, Dengue, West Nile Virus and Hepatitis C. Additionally, our technology has shown promising results for a rare genetic muscular dystrophy called Steinert disease, as well as in vitro efficacy targeting other CNS diseases (ALS, FTD, FXTAS and Huntington's disease).

SPEAKER

Miriam Corredor, *CEO of NoctuRNA Therapeutics, holds a PhD in Medicinal Chemistry and a Master's degree in Business Innovation (MBI). Following her postdoctoral work, she served as Project and Business Development Manager at the Nb4D group within CSIC. She later became Director of Singular Initiatives at Fundación Leitat, where she led strategic projects in the health sector. Since June 2023, she has been the CEO of NoctuRNA Therapeutics.*

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PRODUCT

Circular RNA-based therapy for treating incurable diseases

MECHANISM OF ACTION

RNA viruses contain secondary structures in their genome that are essential for replication, as they serve as binding sites for critical factors. NoctuRNA's circRNAs bind near these sites, disrupting the secondary structure and thereby preventing viral replication. This MoA has been validated through the preclinical development of circRNAs targeting infectious diseases caused by viruses such as HCV, DENV, WNV, and COVID-19.

Secondary structures are not exclusive to viral genomes. Certain genetic diseases cause aberrant secondary structures in human RNA that sequester proteins or disrupt splicing, as seen in Myotonic Dystrophy Type 1 (Steinert, DM1), ALS or Huntington's disease.

NoctuRNA has developed a platform for the design and synthesis of circular RNAs (circRNAs) that target RNA secondary structures which are key to the development of a wide range of diseases. It has been demonstrated that its circRNAs drastically reduce the infectivity of multiple human pathogenic viruses in cell culture within 48 hours. Their potential as broad-specturm antiviral drugs, targeting more than one virus, has also been confirmed. The in vivo experiment in a SARS-Cov-2 model show that the viral load was reduced 90% in the lungs.

TARGET INDICATIONS

This platform has therapeutic applications in both infectious diseases (Covid-19, Dengue, West Nile Virus and Hepatitis C) and genetic disorders, including neuromuscular conditions such as Myotonic Dystrophy Type 1, and neurodegenerative disease like ALS, Huntington's disease and Frontotemporal Dementia. Additional fields have been identified, such as ophthalmology, where there are also genetic diseases that could be targeted by circRNAs.

CURRENT STATUS

- Lead candidate selection and validation: NTX-DM1 has been identified as the lead circRNA therapeutic for DM1, showing strong efficacy in vitro by correcting splicing defects in patient-derived cells and in vivo by improving phenotypes in 83% of treated Drosophila. In vivo studies in mouse are currently ongoing.
- In vivo antiviral proof of concept: In a SARS-CoV-2-infected Syrian hamster model, intranasal circRNA delivery reduced lung viral load by 90% at six days post-infection, demonstrating effective delivery and therapeutic action in vivo.
- Platform versatility: circRNA platform has been validated in vitro in multiple indications beyond DM1, including ALS, FTD, FXTAS, SCA8, Huntington's disease, and viral infections such as Hepatitis C, Dengue, and West Nile virus, supporting a broad therapeutic pipeline.
- Scalable manufacturing: Production protocol has been transferred to a CDMO, which successfully produced 1 mg of NTX-DM1 under RUO conditions, confirming process scalability and enabling future GMP manufacturing.

INNOVATIVE ASPECTS

- There are currently no effective treatments for any of the diseases mentioned above. Existing therapies are solely focused on alleviating some of the symptoms they cause. NoctuRNA's therapeutic strategy offers multiple competitive advantages. Its innovative MoA is particularly suited for rare diseases like DM1, ALS, or FXTAS, potentially qualifying for ODD.
- circRNAs, being endogenous, offer superior safety over exogenous approaches. Their circular structure provides exceptional stability without costly chemical modifications.
- NoctuRNA's platform delivers lead candidates with in vitro efficacy in just 3–4 weeks, with high success rates.
- Broad-spectrum antiviral potential and reduced risk of resistance are added benefits.

IPR

NoctuRNA holds an exclusive, indefinite, worldwide, and for any application, license of patent PCT/EP2021/067756 that protects circRNAs and the underlying MoA, originally owned by UPF. Additionally, NoctuRNA has the right to sublicense such patent. In 12/22, PCT entered in national phases extending protection to the USA, EU, AUS, CAN, JPN, KOR, CHN, ISR, MEX, BRA. The ISR reported no concerns.

NoctuRNA patented in May 2024 (EP24382561.9) a key component of the manufacturing protocol that enhances the circularization yield and reduce costs.

NoctuRNA patented in August 2024 (EP24382889) artificial RNAs for treating repeat expansion disorders

PARTNERING OPPORTUNITIES

The most feasible approach would be an option-to-license agreement for circRNAs targeting diseases of interest, with shared development costs. We are also open to R&D collaborations where NoctuRNA explores specific indications, and the pharma partner funds in vitro validation in exchange for the results. We remain flexible and open to any proposal that creates mutual value.

PROFILE

The **Liver Vascular Biology research group,** led by Dr. Gracia-Sancho, has extensive international experience in studying vascular liver diseases at both pre-clinical and clinical levels. The group's main focus is to advance the knowledge of the pathophysiology of the main complications of chronic liver diseases such as portal hypertension, identify new therapeutic targets and develop non-invasive biomarkers to improve patients' management.

SPEAKER

Sergi Guixé Muntet is a post-doctoral researcher of the Liver Vascular Biology research group at IDIBAPS, led by Dr. Jordi Gracia. He earned his PhD at the University of Barcelona and conducted a Postdoc at the University of Bern (Switzerland). He has published 28 articles in high impact journals. He is currently a teacher in the Master in Translational Research on Hepatic and Intestinal Disease (MITH), from the Universidad Complutense de Madrid.

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PRODUCT

PH-Care: Biomarker signature for early diagnostic of portal hypertension

MECHANISM OF ACTION

Method based on the detection and quantification of circulating proteins regulated by CBX7, such as E-cadherin (ECAD) and/or SPINK1, in body fluid samples. Such proteins have been shown to be released by hepatic vessels in response to elevated portal pressure (portal hypertension), and can be easily detected in systemic circulation.

PH-Care is a novel NON-INVASIVE method for the early diagnosis of portal hypertension. It detects two deregulated circulating proteins in body fluids, such as blood, plasma or serum. It could also provide information on disease severity due to the good correlation between circulating protein levels and portal pressure levels, allowing stratification of patients as normal portal pressure, clinically non-significant PH or clinically significant PH. The technology also has prognostic value, allowing safe time course monitoring of the markers. Protein detection can be performed by ELISA or lateral flow based medical device, making the technology accessible and adaptable to clinical practice.

TARGET INDICATIONS

PH-Care focuses on the diagnosis of chronic liver disease and portal hypertension and is applicable to all cirrhotic patients (~100 million worldwide) regardless of aetiology. Its strong correlation with the hepatic venous pressure enables the identification of high-risk patients for better clinical management planning. Additionally, PH-Care could have potential as companion diagnostic to guide and monitor therapeutic response in clinical trials and targeted treatments.

CURRENT STATUS

- The results presented have been obtained and confirmed both in animal models and in patients.
- Identification of the genes of interest was performed in studies in a Wistar rat animal model and in liver-on-a-chip hydrodynamic pressure study models, followed in both cases by mRNA sequencing studies.

- A developmental cohort of 64 patients (18 healthy donors and 46 cirrhotic patients with varying degrees of portal hypertension) was also used.
- Finally, the markers were validated in a prospective, independent validation cohort including 57 patients with portal hypertension of different etiology.

INNOVATIVE ASPECTS

- The hepatic venous pressure gradient (HVPG) is the gold standard to assess portal hypertension (PH) and allows stratification of patients into normal pressure (NP, HVPG ≤ 5mmHg), PH (HVPG > 5mmHg) and clinically significant PH (HPCS, HVPG ≥10mmHg).
- Despite its accuracy, HVPG is invasive, requires hospitalization and specialized clinicians, and is only available in specialized hospitals. This implies a high cost for the National Health System: 3500-4000 Euro per test (even higher cost in the USA).
- Elastography is a non-invasive alternative, it has a very wide grey area that does not allow patients risk stratification.

IPR

The Technology proposal is protected by the patent entitled "Informative biomarkers of portal hypertension", priority number: EP21382731, which is in national phases in the USA and Europe.

PARTNERING OPPORTUNITIES

Currently, the research group is willing to explore different collaboration models, such as exclusive licenses, R&D contracts for further developments or application for public-private research projects.

PROFILE

Onechain Immunotherapeutics is a clinical stage biotech company based in Barcelona (Spain) focused on the development of CAR T therapies for cancer. OC-1 is the lead product described below. The Company is also developing dual-CAR products using both autologous (patient derived) and allogeneic (donor-derived) cells, targeting haematologic malignancies and solid tumours.

SPEAKER

Wilmar Castillo *holds a PhD in Biomedicine from the University of Barcelona and brings over 20 years of experience in biomedical research and pharmaceutical development. Over the past decade, she has specialized in managing drug development projects across various therapeutic areas. For the last five years, she has served as Clinical Operations Director at OneChain, where she leads all clinical activities.*

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PRODUCT

OC-1; Autologous CAR T CD1a Immunotherapy for coT-ALL

MECHANISM OF ACTION

OC-1 is an autologous CAR-T product targeting CD1a, a marker normally used to diagnose cortical T-ALL/LL patients. The mechanism of action for OC-1 involves the specific recognition of CD1a-expressing tumor cells, leading to the activation and expansion of cytotoxic T lymphocytes. These effector cells mediate targeted tumor cell lysis, resulting in antitumor immune responses. CD1a is normally only expressed briefly by a small sub-population cells in the thymus and the skin and is therefore considered a safe target antigen with very little scope for off-tumour toxicities.

OC-1 is a CART CD1a Immunotherapy developed as a treatment for refractory or relapsed patients with cortical T-ALL, a T-cell leukaemia subgroup that represents a very high unmet medical need. OC-1 consists of autologous T cells that are genetically modified by using a lentivirus to express a chimeric antigen receptor (CAR) with specificity for CD1a. OC-1 has received Orphan Drug Designation (ODD) from the EMA and the FDA. T-ALL comprises 10–15% of all acute leukemias diagnosed in children and 20–25% in adults, with a median diagnostic age of 9 years. Despite intensive chemotherapy, overall survival in T-ALL patients remains suboptimal, particularly in adults. R/R T-ALL has a particularly poor outcome of 6% and 20% in 5 and 10 years respectively.

TARGET INDICATIONS

Lead indication, currently in Phase 1 clinical trial: Relapsed/Refractory (R/R) cortical T-cell Acute Lymphoblastic Leukemia/Lymphoma (coT-ALL). Additional indications: Langerhans cell histiocytosis; Histiocytic sarcoma.

CURRENT STATUS

- The first-in-human clinical trial CARxALL (NCT05679895) to assess the safety and efficacy of OC-1 in R/R coT-ALL patients is ongoing and recruiting at Hospital Clinic Barcelona (adult patients) and Hospital Sant Joan de Deu (pediatric patients). This is a dose escalation trial, evaluating four dose levels (3+2 design) and currently recruiting patients for the second dose level.

- The automated manufacturing process and QC assays have been approved by the AEMPS and a long-term stability study has been completing demonstrating a 36-month stability of the product.
- Non-clinical package demonstrating safety and efficacy of OC-1 has been completed, including the in vivo POC in mouse models, showing very high levels of efficacy and persistence using cell lines and patient-derived leukaemia cells.
- Orphan Drug Designation for the OC-1 therapy has been granted by both EMA and FDA based on preclinical data.

INNOVATIVE ASPECTS

- Out of approximately 70 ongoing competing clinical trials in T-ALL, around 20 are CAR-T therapies targeting CD7, or CD5, two pan-T-cell markers. Such approaches lead to a condition known as lymphopenia, where the T-cells in the patient are depleted by the CAR-T therapy. The patients therefore require a haematopoietic stem cell transplant (HSCT).
- This is associated with risks (e.g. GvHD), and most patients do not have access to a HSCT or are not eligible for one. The majority of the other competing trials are based on various chemotherapy combinations.
- OC-1 is the first CAR T therapy against CD1a marker and is not expected to lead to toxicities, due to its specificity and will be applicable to all CD1a+ patients and has the potential to be the best-in-class product for this indication.

IPR

The first patent protecting the product very broadly (EP19382104.8A; priority date 14/02/2019) has been granted in the USA and certain other countries and is still in application stage in the EU and several additional countries. The second application, protecting specifically the humanised version of the antibody and CAR molecule used on the product (EP23708467.8A; priority date 28/02/2023) is still in the application stage.

PARTNERING OPPORTUNITIES

The company intends to complete the ongoing Phase 1 clinical trial but is open to earlier partnerships. For the next trials, and commercialization, it plans to partner with a larger pharmaceutical company. The company is flexible and remains open to various collaboration options and licensing models for OC-1 and its other products.

PROFILE

UNIVERSITAT DE BARCELONA

The Neuroepilab develops novel chemical entities targeting epigenetic mechanisms to treat central nervous system (CNS) disorders. The group investigates how gene expression is regulated by epigenetics and its role in diseases such as Alzheimer's, Parkinson's, and Huntington's. The main focus is on discovering and optimizing new drug candidates for neurodegenerative conditions.

SPEAKER

Crhistian Griñán *is an Associate Professor and Principal Investigator of the Neuroepigenetics Laboratory at the Faculty of Pharmacy, University of Barcelona (UB), since November 2023. He is a leading expert in neuroscience, epigenetics, and drug discovery for neurodegenerative diseases, with over ten years of experience in translational research. He is also part of the founding team of the future spin-off, Flavii Therapeutics.*

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PRODUCT

Oxapi-27, a first-in-class epigenetic therapy for CNS disorders

MECHANISM OF ACTION

Oxapi-27 is a novel small molecule drug developed to treat Alzheimer's disease and other CNS disorders such as Parkinson's and Huntington's disease. It acts through a unique epigenetic mechanism, targeting the G9a enzyme, which is involved in chromatin modification and gene regulation. The compound was identified through virtual screening and refined via hit-to-lead optimization. Oxapi-27 has demonstrated high potency and selectivity, as well as excellent pharmacokinetic and pharmacodynamic profiles, with proven efficacy through both intraperitoneal and oral administration.

Oxapi-27 is a selective inhibitor of G9a, competing with S-adenosyl methionine (SAM) at the enzyme's cofactor binding site. Unlike other inhibitors, it avoids off-target effects by not affecting the G9a homolog GLP. G9a, also called Euchromatic Histone-Lysine N-Methyltransferase 2 (EHMT2), is a histone methyltransferase that catalyses the di-methylation of lysine 9 on histone H3 (H3K9me2), a repressive mark associated with gene silencing and chromatin reprogramming. In the CNS, G9a regulates neuronal differentiation, synaptic plasticity, and memory. Its dysregulation has been directly associated with neurodegeneration.

TARGET INDICATIONS

Oxapi-27 is intended for the treatment of neurodegenerative diseases, with a primary focus on Alzheimer's disease. Due to the involvement of G9a in multiple brain disorders, Oxapi-27 also shows efficacy in treating Parkinson's disease and rare conditions such as Huntington's disease, highlighting its broad therapeutic potential across multiple brain disorders.

CURRENT STATUS

- Oxapi-27 have a proof-of-concept validated in laboratory settings.
- It belongs to a new class of G9a inhibitors demonstrating high in vitro potency, strong selectivity, and no significant toxicity.
- The compound has a favourable pharmacokinetic profile and excellent blood-brain barrier penetration.

- Toxicological studies confirm its safety, oral bioavailability, and therapeutic range.
- In disease models, Oxapi-27 has shown neuroprotective effects, improving cognitive performance, memory, and behavioral deficits in both in vitro PD and in vivo AD/HD models.
- Results also showed reduced neuroinflammation and enhanced neuronal activity. Next steps include regulatory preclinical studies and GMP manufacturing.

INNOVATIVE ASPECTS

- Oxapi-27 offers significant advantages over current G9a inhibitors, including exceptional selectivity, an improved safety profile, and strong blood-brain barrier permeability, crucial for CNS therapies.
- Its novel epigenetic mode of action reprograms gene expression without altering the DNA sequence, a mechanism largely unexplored in neurodegenerative disease treatment.
- Unlike conventional therapies that target beta-amyloid or tau proteins with limited success, Oxapi-27 addresses underlying epigenetic dysregulation, opening new therapeutic pathways and approaches to treat neurodegenerative diseases.

IPR

Oxapi-27 is protected under European patent EP:23382641.1, filed on June 26, 2023, by Hoffmann Eitle, S.L.U., with a favorable search report issued in December 2023. A PCT application was filed in June 2024, expanding coverage to new compounds and biological data. The international publication (WO/2024/261340) occurred in December 2024, with national phase entry expected by late 2025.

PARTNERING OPPORTUNITIES

We are seeking strategic partnerships with pharmaceutical companies and investors to support the clinical development of Oxapi-27, accelerate its market entry, and maximize its impact on patient outcomes and global healthcare.

PROFILE

NEMA HEALTH is a spin-off from the University of Lleida and the Vall d'Hebrón Research Institute (VHIR) that aims to develop immunotherapies for the treatment of cancer, both in human and animal health. As a licensee of a family of immunostimulatory peptides patented by various institutions, it uses these to produce ONCONEOVAC, an immunotherapy targeted at advanced-stage melanoma.

SPEAKER

Olga Rué, *CEO, holds a degree in law. She has dedicated the last 15 years of her career to the biotechnology industry, holding executive positions in companies such as Archivel Farma, where she was CEO for eleven years, during which she led the development of an immunotherapeutic agent for the treatment of tuberculosis.*

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PRODUCT

Onconeovac: a personalized immunotherapy for the treatment of cancer

MECHANISM OF ACTION

ONCONEOVAC is a personalized immunotherapy generated by the addition of proprietary immunostimulatory peptides (known as DIF peptides) to tumor neoantigens obtained through a biopsy of the patient (which gives it its personalized nature). The final product is designed to induce a specific immune response that recognizes the unique mutational profile of each patient's tumor.

DIF peptides are small molecules capable of stimulating and activating the immune system, promoting the production of cytokines and the activation of T lymphocytes. ONCONEOVAC takes advantage of the patient's immune system to generate a precise and sustained antitumor response, while minimizing systemic toxicity.

TARGET INDICATIONS

ONCONEOVAC is being developed for the treatment of advanced stage melanoma as the first selected indication, but other anticipated applications are related to solid tumors with a high mutational burden and poor prognosis, such as lung, ovarian, and pancreatic cancer.

CURRENT STATUS

- Both in vitro and in vivo studies have demonstrated the potential of DIF peptides as powerful immunostimulatory agents.
- Preclinical experiments in mouse models show that ONCONEOVAC achieves antitumor efficacy comparable to anti-PD1 therapy, and that the combination of ONCONEOVAC with anti-PD1 results in a synergistic increase in therapeutic outcomes.
- Additionally, preliminary data suggest that ONCONEOVAC may also play a preventive role in reducing tumor recurrence.
- Work is currently underway to define the regulatory roadmap, having already made contacts with the AEMPS in collaboration with a specialized consulting firm, and in various aspects of product development.

INNOVATIVE ASPECTS

- It is worth noting the customization offered by ONCONEOVAC and the potential to increase the efficacy of anti-PD1 when both are combined are its most notable aspects.
- But what sets ONCONEOVAC apart is the low complexity of its manufacturing process, which consequently impacts the manufacturing times that are far below what any other immunotherapy requires.
- Additionally, the fact that NEMA HEALTH holds the patent on the DIF-P peptides, and therefore, on setting their price, could mean that the costs of immunotherapy could be significantly lower than other current or future proposals.

IPR

DIF peptides are protected by a patent (PCT/EP2020/068052) that covers a family of peptides and their use as anti-cancer agents and as new vaccine adjuvants. The patent was filed in 2019 and has progressed through international phases, with national entries in key markets, including the United States, Europe, and Japan, where it has already been granted.

PARTNERING OPPORTUNITIES

Our interest is in identifying a pharmaceutical company that has some affinity with our product to accompany us in the preclinical (and future clinical) development of ONCONEOVAC, contributing its expertise in the field to do a co-development.

PROFILE

This research group forms a multidisciplinary team from several institutions. Prof. Vázquez brings expertise in medicinal chemistry and project valorization. Pharmacologists Prof. Cobos and Dr. Ruiz-Cantero are experts in S1R pharmacology, pain models, and technology transfer. The team is supported by Dr. Arbulu and Dr. Goytisolo which are business experts, with extensive mentoring experience from the pharma sector. Finally, Ms. Íñiguez (TTO) is our expert in IP protection and spin-off creation.

SPEAKER

Fermin Goytisolo *holds a PhD in Biochemistry and an MBA from IE Business School, with extensive experience in diagnostics, drug discovery, and medical devices. He was Evaluation Director at Najeti and Uninvest SCR, assessing over 150 biotech projects. At Esteve pharmaceutical, he served on the R&D Executive Committee and led business development. He later joined Pangaea Oncology, where he licensed the PAK program from Cancer Research Technologies and co-led joint optimization teams. He has been CEO of several biotech startups, including Kusudama Therapeutics, Adan Medical, and Miramoon Pharma.*

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PRODUCT

First-in-class dual-acting analgesics

MECHANISM OF ACTION

This product consists of first-in-class molecules that act on two key targets involved in the pathophysiology of pain, offering a novel dual mechanism of action. This approach results in significantly improved analgesic efficacy compared to conventional monotherapies.

The development of the compound is based on a dual mechanism of action involving sigma-1 receptor (S1R) antagonism and soluble epoxide hydrolase (sEH) inhibition. Both the S1R and sEH are novel drug targets that have been independently identified as key players in pain development. S1R antagonists and sEH inhibitors have shown promising results in preclinical studies, and both targets have been validated in clinical trials for other pain conditions, demonstrating a favorable safety profile. Our dual approach improves pain management, offering a potential breakthrough in pain therapeutics.

TARGET INDICATIONS

The main indication is the treatment of moderate-to-severe postoperative pain. It also shows promising potential for the treatment of pain during rheumatoid arthritis, which could be a potential secondary indication.

CURRENT STATUS

- The compounds have shown strong analgesic effects in validated preclinical models, including a murine model of postoperative pain (laparotomy) and a rat model of rheumatoid arthritis pain. These findings support the therapeutic potential of our product for the treatment of both acute and chronic pain conditions.
- Preclinical development stage is ongoing. A first family of dual compounds was identified with excellent potency, target selectivity, favorable in vitro DMPK, and strong in vivo efficacy.

- A selected lead showed significant analgesic effects in rodent models of postoperative and rheumatoid arthritis pain, supporting its therapeutic potential.
- Pharmacokinetic studies confirmed brain penetration after oral dosing, and selectivity assays indicated minimal off-target activity.
- To further improve the profile, a second generation of compounds was synthesized, showing promising results in vitro and in a mouse model of pain hypersensitivity.

INNOVATIVE ASPECTS

- The product stands out as an innovative drug with excellent potency for treating postoperative pain, offering a new dual mechanism of action.
- It demonstrates strong efficacy with fewer side effects than opioids, potentially reducing the need for opioids after surgery, which could help address concerns about their addiction potential, particularly in the USA.
- Additionally, this dual compound offers competitive advantages over drug combinations, such as simpler pharmacokinetics, avoiding potential undesired interactions, and promoting better patient compliance.
- These differential characteristics position the product as a promising alternative in pain management.

IPR

Three patents protect the technology: WO2024105225A1 (the combined use of an sEH inhibitor and an S1R antagonist), WO2024105234A1 (the Markush structure of our first family of dual molecules) with national phase entry in progress, and the PCT application PCT/EP2025/060895 (which protects the second family of our dual compounds).

PARTNERING OPPORTUNITIES

We are looking for a licensee or a potential partner for doing a co-development of the product.