MEDICAMENTOS INNOVADORES
Plataforma Tecnológica Española

farmaindustria

Acción experimental para el fomento de la cooperación entre compañías farmacéuticas y empresas y grupos de investigación en el ámbito de la biotecnología

XII Encuentro de Cooperación Farma-Biotech

Santiago de Compostela

Viernes, 26 de septiembre de 2014

El encuentro tiene por objeto estimular 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 iniciativa patrocinada por FARMAINDUSTRIA se propone a través de esta jornada que empresas españolas y grupos de investigación de centros especializados, previamente seleccionados, expongan, ante las compañías farmacéuticas interesadas, productos en desarrollo con el potencial suficiente (innovador, eficaz, protegido) que pueda representar una oportunidad de cooperación para ser explorada por ambas partes.

Tras un cuidadoso estudio de necesidades expresadas por las compañías farmacéuticas y del estado de desarrollo de las investigaciones en curso en las empresas biotecnológicas y los grupos de investigación, se han seleccionado diez propuestas para que realicen su presentación en este encuentro, abierto a todos los asistentes a **BioSpain 2014**, dentro de cuyo marco se celebra este evento.

La jornada se configura como un foro 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 innovador** en el ámbito de las nuevas terapias y los medicamentos avanzados.

Las presentaciones, con una duración cada una de veinte minutos, se centrarán especialmente en los aspectos de **oportunidad de negocio** de cada nuevo producto en desarrollo avanzado, valorizando su *carácter diferencial*, el *ámbito terapéutico* de actuación, *la expectativa de mercado* mundial y el grado de *pruebas superadas*, tanto preclínicas como clínicas.

En las páginas que siguen se presenta la agenda detallada del encuentro así como un breve resumen de cada producto. Este resumen está escrito en inglés para facilitar la difusión entre personas interesadas de distintas nacionalidades.

Las presentaciones durante el encuentro se realizarán en español, soportadas por diapositivas en inglés.

Desde **Farmaindustria**, y a la vista de los exitosos resultados de los once anteriores encuentros celebrados hasta el momento (en Madrid, Barcelona, Zaragoza y Bilbao) recomendamos vivamente la participación en esta jornada, dado el interés como oportunidad de negocio que presentan los productos que se van a exponer.

Para cualquier duda o comentario se puede contactar con:

Amelia Martín Uranga

amuranga@farmaindustria.es

Tfno. 915159350

Agenda prevista

La organización de la jornada pretende dar tanto énfasis a las presentaciones como a la interacción personal entre los asistentes, de acuerdo con la siguiente agenda:

Hora	Entidad	Ponente	Fármaco presentado
09:30 09:50	**Farmaindustria**	***Javier Urzay*** Deputy Director	Opening keynote
09:50 10:10	**Bionure**	***Albert G. Zamora*** Co-founder and CEO	Neuroprotective BN201 for multiple sclerosis and orphan CNS indications
10:10 10:30	**IDIS**	***José Castillo*** Director	Repositioning of the drug CBG000592 for treatment of ischemic stroke
10:30 10:50	**Spherium Biomed**	***Luis Ruiz*** CEO	Oxaloacetate donors as new neuroprotective agents
10:50 11:10	**Oncomatrix**	***Cristina Ferrer*** Preclin. Dev. Director	Recombinant human Cystatin-C for the treatment of invasive triple negative breast cancers
11:10 11:30	**Ankar Farma**	***Ana Martínez*** Co-founder	Innovative pharmacological treatment for retinal dystrophies
11:30 11:50	**NeuronBio**	***Javier Burgos*** CEO	NST0037: a novel statin with high neuroprotective activity
11:50 12:10	**Lilfelength**	***Pilar Najarro*** Director Corp. Sales	Telomere Analysis Technologies: a fit-for-purpose biomarker
12:10 12:30	**Hosp. Virgen del Rocío**	***Pablo Hervás*** Head of Tech. Transfer	Cannabinoid agents for the treatment of multiple myeloma and related conditions
12:30 12:50	**Univ. de Vigo**	***Angel Rguez. de Lera*** Professor	UVI5008, a multiple epigenetic modulator for the treatment of cancer
12:50 13:10	**CIMA**	***Julen Oyarzábal*** Director of Trans. Science	Novel strategy for symptomatic and disease-modifying treatment of Alzheimer's Disease

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: Dentro del marco BioSpain 2014: Pharma-Biotech Meeting. Sala 24, primera planta. Palacio de Congresos y Exposiciones. C/ Miguel Ferro Caaveiro s/n – San Lázaro. 15707 Santiago de Compostela

Fecha: Viernes día 26 de septiembre de 2014. De 9:30 a 13:15 horas.

PRESENTACIONES QUE TENDRÁN LUGAR EN ESTA JORNADA

BIONURE

PROFILE

bionure
Biomedicine heading to future

BIONURE is an early-stage drug development company founded in 2009. We develop neuroprotective therapies for the treatment of neurodegenerative diseases and we focus in Multiple Sclerosis (MS) and two rare diseases: Optic Neuritis (ON) and Neuromyelitis Optica (NMO). The main candidate BN201 is currently at regulatory preclinical stage and it has been granted orphan designation in Europe for the treatment of ON. Bionure also held a pre-IND meeting with the FDA and the outcome was positive. In terms of IP, the patent covering BN201 has already been granted in the United States. The company is led by Albert G. Zamora, CEO, and Pablo Villoslada, CSO, and is supported by an extraordinary SAB led by Joaquim Trias and leading authorities in MS such as Larry Steinman and Stephen L. Hauser, among others.

SPEAKER

Albert G. Zamora, Co-founder and CEO of Bionure, is a pharmacist by training and MBA. Albert is a serial entrepreneur that has been involved in the creation of several companies in the health and tech sectors and a highly experienced professional with more than 15 years of experience in strategic marketing and business development in the healthcare sector.

He is the former CEO at Linkcare (start-up) and former Director of Technology Transfer at the Hospital Clínic at University of Barcelona.

azamora@bionure.com

PRODUCT

Neuroprotective BN201 for multiple sclerosis and orphan CNS indications.

MECHANISM OF ACTION

Neuroprotection refers to the ability to protect neurons from degeneration and promote repair, preventing neurodegeneration. BN201 is a small molecule, New Chemical Entity with a novel MoA. It is a First-in-Class SGK agonist in the SGK/FOXO3 pathway. SGK activates the transcription factor FOXO-3 and induces FOXO-3 translocation out of the nucleus. FOXO-3 promotes apoptosis through up-regulation of programmed cell death genes; therefore translocation of FOXO-3 out of the nucleus inhibits apoptosis program and promotes cell survival. Consistent with the MOA, BN201 has been shown to promote neuroprotection and remyelination in several animal models (MS, ON, Glaucoma) as well as in vitro.

TARGET INDICATIONS

Main indications: optic neuritis, neuromyelitis optica and multiple sclerosis. However, due to the neuroprotective MOA of BN201, it may be potentially useful for several neurodegenerative diseases such as Alzheimer, Parkinson, Amyotrophic Lateral Sclerosis or Glaucoma, among others.

CURRENT STATUS

BN201 is at the regulatory preclinical stage of development. Most relevant milestones achieved:

- Preclinical POC of neuroprotection in several animal models (MS, ON, glaucoma)
- Preclinical POC of remyelination in vitro
- Orphan designation granted to BN201 by European Commission for the treatment of optic neuritis in Europe
- Positive pre-IND meeting with the FDA validating the regulatory, preclinical and clinical development plans designed
- Composition of matter patent covering BN201 granted in the United States

INNOVATIVE ASPECTS

- Current drugs for MS are immunomodulators that only prevent inflammatory relapses: they are only partially effective for relapsing patients (RRMS) while progressive forms (50% of the population) don't respond to therapy.
- Neuroprotection refers to the ability to protect neurons from degeneration and promote repair. It is seen as the holy grail in MS and could open a new era in the treatment of several neurodegenerative diseases.
- Neuroprotective drugs like BN201 able to prevent neurodegeneration are a strong need not only for all MS patients but also for ON and NMO patients (SoC has very limited efficacy). BN201 is a NCE, First-in-Class, small molecule that has proven neuroprotective and remyelinating, with a completely novel MoA through the SGK/FOXO3 pathway.
- Our main goal is to demonstrate neuroprotection in humans. For this purpose we have designed a cost-effective orphan strategy to probe neuroprotection in a rare indication closely related to MS, Optic Neuritis. This clinical POC would then provide the opportunity to extend BN201 to bigger CNS indications such as MS

IPR

Bionure has two patents covering BN201 as well as several other compounds with neuroprotective activity for neurodegenerative diseases. The composition of matter patent covering BN201 has already been granted in the United States and in national phases in Europe and the rest of the world. We continue to work in IP towards new indications, the synthesis process or back-ups

PARTNERING OPPORTUNITIES

We are open to collaborate with pharmaceutical companies in a wide range of options including direct equity investment, licensing, co-development or acquisition deals, among others.

INSTITUTO DE INVESTIGACIÓN SANITARIA DE SANTIAGO DE COMPOSTELA

PROFILE

THE HEALTH RESEARCH INSTITUTE OF SANTIAGO (IDIS), created in January 2008, is one of the 13 health research institutes accredited in the whole of Spain by the Carlos III Health Institute and the only one in Galicia. The IDIS is a system of innovation and knowledge transfer plays a key part in translational matters and optimizes and expands the historical collaboration and synergistic potential between the University of Santiago de Compostela and the Santiago University Hospital Complex, entities to whom over 50 groups from the Institute are linked.

SPEAKER

Professor José Castillo, is Chief of the Neurology Department of University Clinical Hospital of Santiago de Compostela (CHUS), Director of the Department of Medicine of the Faculty of Medicine of the University of Santiago de Compostela (USC) and Director of the Institute for Sanitary Research of Santiago de Compostela (IDIS).

Prof. Castillo possesses an experience of more than 35 years as clinical neurologist, 30 years as researcher in neurosciences, mainly focused on cerebrovascular diseases, and 30 years of teaching at the University of Santiago de Compostela. With more than 400 publications he is making a notable scientific contribution at international level.

jose.castillo.sanchez@sergas.es

PRODUCT

Repositioning of the drug CBG000592 for treatment of ischemic stroke.

MECHANISM OF ACTION

After ischemic stroke, there is a rapid elevation of glutamate into the extracellular space which leads to a neuronal death of the cerebral tissue. Consequently, glutamate antagonists have been widely studied as protective agents; unfortunately, they failed or displayed severe adverse effects when they were tested in clinical trials.

Blood glutamate scavenging has becoming a novel and attractive protecting strategy to reduce the excitotoxic effect of extracellular glutamate released during ischemic brain injury with successful protective effects during acute phase of ischemic stroke. We have discovered that compounds like oxaloacetate are able to acts as a blood scavenger of glutamate; however, administration of oxaloacetate in humans has potential serious limitations as the required effective dosage to induce significant effect.

With the aim to solve this limitation, we have recently performed a screening analysis of repositioning drugs with blood glutamate scavenging activity. As result of this analysis, we have discovered the drug CBG000592 with blood scavenging activity determined either in vitro and in vivo analysis by means of ischemic animal model. We have observed that scavenging activity of CBG000592 is also potentiated in combination with minimal amount of oxaloacetate (non-toxic doses).

TARGET INDICATIONS

The use of CBG000592 is suggested as treatment against ischemic stroke, however this drug could be potentially used in those neurological diseases associated with glutamate neurotoxicity as migraine, Multiple Sclerosis (MS) or Amyotrophic Lateral Sclerosis (ALS), for instance.

CURRENT STATUS

We have already completed all preclinical studies, and recently we have gotten public founding to start a proof of concept in stroke patients to demonstrate the efficacy of CBG000592.

Positive results after this clinical trial will allow us to test the efficacy of CBG000592 in combination with minimal amount of oxaloacetate (as we have described, this combination shows higher protective effects than CBG000592 alone).

INNOVATIVE ASPECTS

- One of the main advantage of the use of the CBG000592 drug as a potential treatment for ischemic stroke is that it has been used previously in humans, therefore human toxicity analysis (Phase I and II) are not needed before to test its effects in stroke patients.
- Other important advantage of this drug is that, it does not require a prior computerized tomography scan therefore, it could be given as early as possible, perhaps even as ambulatory treatment suggesting potential clinical application.
- In comparison with previous glutamate antagonists, another important advantage of serum-scavenging-based treatments is that their effects are not mediated through the neuronal ionotropic glutamate receptors, thereby avoiding problems of poor blood–brain barrier permeability and potential neurotoxic effects

IPR

The use of oxaloacetate as potential drug against stroke is already licensed to Janus Company (Barcelona, Spain,) while the use of the CBG000592 for stroke is in patenting process.

PARTNERING OPPORTUNITIES

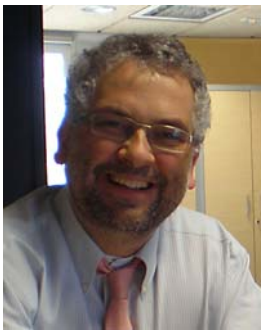
We offer our product and collaboration to the pharmaceutical industry with the to demonstrate the clinical use of CBG000592 in stroke or other neurological pathologies associated to glutamate toxicity.

SPHERIUM BIOMED

PROFILE

SPHERIUM BIOMED is a biomedical project incubator aiming to turn biomedical knowledge into social and economic value, working in the very early stage of the value chain. Spherium acquires licensing rights on university and research institute's patents, invests up to 400K€ taking a leading role in the early stages of product development and subsequently sublicenses these projects to larger companies or to Spherium's spin-offs incorporated to attract VC investment. Revenues are obtained from royalties paid by these companies, sales of participated companies and recurrent revenues related to management or research fees. Spherium's mission is to bridge the gap in early stage developments of biomedical scope. Spherium continues the business model and mission/vision of Janus Developments (www.janusdevelopments.com), which changed the name after a breakthrough deal with the Spanish pharmaceutical company Ferrer Internacional.

SPEAKER



Luís Ruíz Ávila, CEO, PhD (Mol. Bio.) after 10 years of academic research joined the industry in 1997 taking project management and business development roles. In 2001 joined University of Barcelona spinoff Advancell becoming its first CEO.

In 2009 started Janus Developments (now Spherium Biomed), a hands-on, early biomedical project incubator. He is currently CEO in Spherium and in Aquilón Cyl SL, a veterinary EBT located in León. He is one of the founders of CataloniaBio, the catalan association of biomedical companies.

lruiz@spheriumbiomed.com

PRODUCT

Oxaloacetate donors as new neuroprotective agents.

MECHANISM OF ACTION

Brain insults are characterized by a multitude of complex processes, of which glutamate release plays a major role. Our product is a small molecule shown to be an oxaloacetate donor in blood.

Oxaloacetate works as a natural blood glutamate scavenger through the activation of the blood glutamate oxaloacetate transaminase (GOT), reducing the blood glutamate concentration which results in an immediate reduction of brain glutamate levels, thus reducing the acute excitotoxic effects related to the neurotransmitter excessive release.

The use of oxaloacetate donors to scavenge blood glutamate to indirectly reduce brain levels has been shown to be efficacious in anatomic and functional neuroprotection in several animal models of stroke, traumatic brain injury and other well established preclinical models of acute brain glutamate excitotoxicity.

TARGET INDICATIONS

Neuroprotection during HIE and CPB is a clear unmet need with no pharmacological treatment available. HIE has a prevalence around 0.01% (360.000 ww) and therapeutic hypothermia is only partially effective.

A "prophylactic" treatment for all cardiac surgery conducted with CPB has a global incidence around 1.2 M patients worldwide. Besides those, proven neuroprotective therapy has the potential for development in additional conditions like Stroke and Traumatic Brain Injury recovery.

CURRENT STATUS

Preclinical evidence: Neuroprotection has been shown in a rat model of cerebral ischemia (MCAO) after i.v. administration of oxaloacetate ethyl ester as an oxaloacetate donor.

Clinical evidence: Low blood glutamate levels and increased GOT activity are associated with good functional outcome in acute ischemic stroke patients. High blood glutamate levels are found in patients who underwent CAS (Carotid Angioplasty and Stent placement). In another study, glutamate levels increase immediately after CPB respect to basal levels and returned to baseline levels at 24h, with patients showing higher levels being the ones showing also worst neurologica outcomes.

INNOVATIVE ASPECTS

- Reducing brain excess glutamate using a blood target offers unique therapeutic opportunities in a broad range of unmet medical needs, being stroke the most relevant. Spherium's strategy, however, focusses on indications where ischemic episodes causing glutamate release undergo in a controlled hospital setting, where neuroprotective therapy could be anticipated and under control.
- Among them, Hypoxic ischemic Encephalopathy (HIE) (perinatal asphyxia) is a paediatric indication with no pharmacological alternatives, considered a rare disease and could provide an orphan drug designation.
- In parallel, we aim to develop the product for a highly incident adult indication: cardio pulmonary bypass (CPB), a procedure used in the 80% of all cardiac interventions. There are two types of Perioperative Cerebral Injury (PCI) associated with CPB: type I postoperative neurological deficits due to an ischemic stroke (that may affect 2-5% of all patients) and type II Postoperative cognitive decline (POCD). The latter includes impairment of attention, concentration, short-term memory and fine motor function and has an incidence 20-fold greater than type I.
- Our target product profile is a stable oxaloacetate donor, formulated in a precharged syringe for acute bolus administration.

IPR

PCT patent for the use of oxaloacetate ethyl ester in 1-5 ml, 35-500 mg/ml given as an i.v. bolus in a dose higher than 2.4 mg/Kg to treat pathologies associated to high levels of glutamate. Priority date of 2010 and extended in 2013 to the EU and the USA. Current patent expires in 2030. Originated in the University of Santiago and licensed to Spherium to develop and commercialize under an exclusive agreement.

PARTNERING OPPORTUNITIES

We are looking for potential partners to help us forward the project once we complete the pre-GMP development of the candidate and the proof of principle in two preclinical rat models of HIE and CPB.

ONCOMATRIX

PROFILE

ONCOMATRYX BIOPHARMA therapeutic approach is based on the development of new biological drugs that target proteins located in the peritumoral stroma (fibroblasts, endothelial cells and extracellular matrix) a novel and innovative route for cancer treatment, directed not against tumor cells, but the cells that promote their invasiveness and drug resistance.

Oncomatryx develops human-derived proteins, Immunotoxins and Antibody-Drug Conjugates as novel therapies against tumor invasiveness and drug resistance. At the same time Oncomatryx develops companion diagnostics tools for their therapeutic products identifying biomarkers associated to the treatments.

SPEAKER

Cristina Ferrer, Preclinical Development Director, has worked as a research scientist in different Pharmaceutical Companies on the development of recombinant antibodies and other biotech products for therapy, including cancer (ie. Program Resources Inc. ,USA; Menarini Ricerche Sud, Italy; Sanofi-Synthelabo, France; Bio-Rad, France; Biotherapix, Spain). She continued her career working in Preclinical Development service Companies (ie. Harlan Laboratories or Vivotecnia) before joining Oncomatryx.

She holds a PhD in Cell and Molecular Biology and a Master degree in Biotechnology from the Autonomous Univ. of Barcelona (Spain), and B. Sc. degrees in Biological Sciences and in Pharmacy from the Univ. of Barcelona.

cferrer@oncomatryx.com

PRODUCT

Recombinant human Cystatin-C for the treatment of invasive triple negative breast cancers.

MECHANISM OF ACTION

Cystatin-C is a well-known inhibitor of cathepsin B, a cysteine proteinase implicated in stimulating cancer cell invasion and metastasis. Recently we showed that Cystatin-C blocks TGFβ pathway by binding to the TGFβ receptor II; TGFβ is also a stimulatory factor for the metastasis and dissemination of late stage tumors. Overall activity of Cystatin-C in the different compartments of the tumor microenvironment results in an inhibition of tumor invasion and angiogenesis, blocking tumor cells dissemination and metastasis in vivo.

TARGET INDICATIONS

TNBC are aggressive tumors lacking hormone receptors and HER2 overexpression. Patients diagnosed with TNBC have a high rate of mortality and recurrence.

Currently, there is a lack of efficient TNBC therapy and Cystatin-C could be a good candidate to improve the efficacy of standard chemotherapy used in the treatment of TNBC without increasing the toxicity.

Alternatively, due to its anti-angiogenic activity and differential expression into the eye, Cystatin-C could be used locally as anti-angiogenic drug for inflammatory eye pathologies.

CURRENT STATUS

- In vitro characterization of the recombinant human Cystatin-C shows that the protein inhibits cell invasion of different human tumor cells stimulated by TGFβ or cancer associated fibroblasts (CAFs).
- In vivo, preclinical studies in highly aggressive human triple negative breast tumor xenografts show that the recombinant protein has a clear effect in metastasis when combined with standard chemotherapy, mainly due to its anti-angiogenic activity.

INNOVATIVE ASPECTS

- Human Cystatin-C is a natural inhibitor of tumor invasion and metastasis. Cystatin-C has a pleiotropic tumor suppressor activity as inhibitor of cathepsin B and TGF functions.
- The recombinant protein produced by Oncomatryx, almost identical to the natural protein, is a first in class therapeutic product for the treatment of metastasis in aggressive breast tumors.
- Recombinant Cystatin-C is expected to have a good safety profile and we foresee that the product could be a good candidate for triple-negative breast cancer (TNBC) treatment in combination with standard chemotherapy without expecting an increase in toxicity associated to these drugs.

IPR

Oncomatryx has licensed from National Jewish Health (US) a collection of patents claiming the use of Cystatin-C as TGFβ antagonist for the treatment of different diseases including cancer. These patents have already been issued in US and Australia and the main application is pending in Europe.

PARTNERING OPPORTUNITIES

Oncomatryx seeks license agreements or partnership agreements for further development of the product in collaboration with Pharmaceutical Companies.

ANKAR PHARMA

PROFILE

ANKAR PHARMA SL, a CSIC spin-off, born with the aim to fill the gap between basic drug discovery research and clinical trials, increasing the value of innovative drugs candidates. The first project will be focused on ITDZ's small heretocyclic compounds, that targeting simultaneously PDE7 (Phosphodiesterase 7) and GSK3 (Glycogen synthase kinase 3), has demonstrated a promising therapeutic profile for different CNS diseases. The company, was established in February of 2014, and is managed by a team of experienced scientists from the CSIC (Prof. Ana Martinez and Dr. Carmen Gil), as well as recognized business professionals (Michael De Jose, PhD (CEO) and Jose Maria Olbes (Business Development)**.**

SPEAKER

Ana Martinez, co-founder of ANKAR PHARMA, is tenured staff of CSIC since 1990. Having a background formation on Medicinal Chemistry and recently on Bussiness & Administration, her interests are focused on drug design and development of new drugs for neurodegenerative disorders. Since February 2002 till January 2008, to complete a technology transfer process, she joined to NeuroPharma as R&D Director. She provided strategic, leadership management and guidance in R&D activities, reaching two of her research compounds (tideglusib and NP-61) till clinical trials as disease modifying agents for Alzheimer's disease. Author of more than two hundred scientific publications, thirty active patents in the field and editor of several books, she acts as scientific advisory board for several SMEs in the biotech field**.**

ana.martinez@csic.es

PRODUCT

Innovative pharmacological treatment for retinal dystrophies.

MECHANISM OF ACTION

VP3.15 belongs to the 5-imino-1,2,4-thiadiazoles (ITDZs) heterocyclic family. These molecules are innovative dual GSK-3/PDE7 inhibitors. Both targets are expressed in the central nervous system and are related with inflammatory and neuroprotective processes. VP3.15 allosterically modulates both pharmacological receptors. Moreover, GSK-3 and cAMP-specific PDEs, such PDE7, are also involved in neuroregenerative processes. The knowledge gained in neuroscience field should be transferrable to the ophthalmology field, and should allow for innovative results in the near future.

TARGET INDICATIONS

Retinal and Central Nervous System diseases. Based in our pre-clinical data with VP3.15, this molecule may be a good drug candidate for multiple sclerosis (preclinical efficacy shown). Moreover based in VP3.15 mechanism of action, and our previous data with other related molecules, VP3.15 may be a valuable drug candidate for Parkinson and/or Alzheimer disease also.

CURRENT STATUS

VP3.15 is in preclinical regulatory development. Now we have planned the IND-enabling works to reach clinical trials. Important goals for VP3.15:

1. Easy and scale up synthetic method,
2. Negative AMES test,
3. Good in vivo pharmacokinetic profile: VP3.15 reaches the brain after i.p. and oral administration,
4. Remyelinazation properties both in vitro (olygodencrocyte precursors) and ex vivo (cerebellum slices),
5. Photoreceptors protection using retina explants from rd10 mices (a transgenic model for retinitis pigmentosa),
6. Photoreceptors protection using retina explants from wild mices treated with NMDA.

An in vivo study using a NMDA retinal toxicity model is ongoing. Chronically administration of VP3.15 via i.p is used and retinography will be done at the end of the study together with inmmunohistochemical determinations (date of completion September 2014).

INNOVATIVE ASPECTS

- Our compound, VP3.15, is a small molecule that has shown excellent results in different neurological preclinical models.
- As the neuroretina shares common molecular pathways with the brain and other parts of the CNS, we assayed VP3.15 in different models of retinal damage.
- VP3.15 has shown a nice photoreceptors protection in rd10 mice retinas (retinitis pigmentosa model) and in NMDA retinal damage model. Retinal diseases and the associated vision loss is the first cause of personal disability in Europe.
- Presently, only palliative treatments are available, and they do not avoid the photoreceptor's cell loss. Therefore, there is a manifest urgency in the discovery and development of more novel and effective drugs to eradicate the possible blindness associated and VP3.15 may play a key role in the next future.

IPR

5-Imino-1,2,4-thiadiazoles (ITDZs) compounds are protected by Patent Application Number ES200930787 (priority date October 2009). Now the patent is granted in Spain [ES2360783 (B1)], and has been extended to the USA [US2012225879 (A1) (04.02.2012)], EU [EP2484670 (04.27.2012)], Australia [AU2010302536 (A1) (04.27.2012)], and Canada [CA 2780695 (04.27.2012)]. The owner of the patent rights is the CSIC, and a worldwide license with a contractual period of 15 years has been granted to ANKAR PHARMA SL

PARTNERING OPPORTUNITIES

ANKAR PHARMA SL is looking towards a risk-sharing model to seek further expansion within VP 3.15's ophthalmic pathologies, and joint development work in various other therapeutic areas.

NEURON BIO

PROFILE

NEURON BIO specializes in the development of biosolutions for the pharmaceutical, chemical, and agro-food industries via its divisions: Neuron BioPharma and Neuron BioServices. The Neuron BioPharma research division is devoted to the discovery and assessment of pharmaceuticals compounds to treat and combat neurodegenerative illnesses such as Alzheimer's disease.

SPEAKER

Javier S. Burgos Muñoz, CEO, with a background of more than 8 years in the biopharmaceutical industry at the executive level, combined with an excellent basic research background (>12 years) in top-level academic institutions. Senior manager with outstanding knowledge of staff recruitment, budget management (several M€/year), resources assignment, team management and IP & scientific management.

jburgos@neuronbio.com

PRODUCT

NST0037: a novel statin with high neuroprotective activity.

MECHANISM OF ACTION

NST0037 is a member of the statins' family, showing a strong inhibition of the 3-hydroxy-3-methylglutaryl coenzime A reductase (HMGCR) enzymatic activity, that regulates cholesterol metabolism. Additionally, NST0037 has demonstrated a critical regulation of the APP pathway, modulating the β-amyloid deposition in transgenic mouse models. Finally, NST0037 increase the DHCR24 expression, a neuroprotective gene discovered in the brains of the Alzheimer's disease patients.

TARGET INDICATIONS

The therapeutic area for NST0037 will be neurodegenerative diseases, and especially Alzheimer's disease (AD). Because of its other neuroprotective properties as modulation of APP metabolism and protection from neuronal death or neuroinflammation, MCI-to-AD is considered the main indication. Moreover, because of the antiepileptic activity of this compound, epilepsy could be considered as second indication.

CURRENT STATUS

NST0037 is in late preclinical regulatory phase and a Phase I clinical study is scheduled for the next months. The compound's efficacy will be evaluated as a disease modifying neuroprotective treatment to halt or slow down the progression from mild cognitive impairment (MCI) to Alzheimer's disease (AD).

INNOVATIVE ASPECTS

Neuron Bio has developed NST0037, a novel statin with an improved neuroprotective profile and with potential interest as disease-modifiying treatment for Alzheimer's disease (AD).

The molecule presents neuroprotective, antiepileptic, anti-inflammatory and hypocholesterolemic activities, that it has been demonstrated in in vitro and in vivo experimental paradigms.

As an antiepileptic, NST0037 has shown a better profile than simvastatin in acute models of epilepsy and as a long-term neuroprotectant in AD models (transgenic and induced chronic models of neurodegeneration).

NST0037 also reduces neuronal death, peripheral and central inflammation, cognitive impairment, β-amyloid burden and preserves brain metabolic activity

IPR

WO2010119161 ("Antiepileptic, hypocholesterolemic and neuroprotective compound"), priority date: 2009-04-16. International Preliminary Report on Patentability (IPER) obtained at 26/07/2011.

PARTNERING OPPORTUNITIES

Neuron Bio is looking for a partner (a pharmaceutical company or an investment group, etc.) to support the preclinical and clinical development of this promising candidate compound. The degree of involvement of the partner in the development of NST0037 is open to discussion. Profit distribution will depend on the partner's contribution.

LIFE LENGTH

PROFILE

LIFE LENGTH, established in 2010, is a spin-off of Spain's most prestigious research center, the Spanish National Cancer Research Center. Headquartered in Madrid, Life Length has emerged as the world's leading diagnostic testing company for telomere length measurement reports and services for industry, academic research as well as the general public as a biomarker of overall health and biological age**.**

SPEAKER

Pilar Najarro, Director for Corporate Sales**,** is responsible for business building through partnering with pharmaceutical and nutraceutical customers, with an extensive international experience in academia and industry. She obtained her Ph.D. from the State University of New York later expanding her research interests at Imperial College in London. Pilar joined the Biotech industry 9 years ago and has been involved in a wide spectrum of projects both, in biotech and large pharmaceutical companies.

pnajarro@lifelength.com

PRODUCT

Telomere Analysis Technologies: a fit-for-purpose biomarker.

MECHANISM OF ACTION

Telomere length determination is performed using a fluorescence-based assay in which each telomere is measured individually. The results obtained are length-distribution sets - not a mere overall median. These data have proven to be of great relevance as cell senescence is determined by the abundance of critically short telomeres not by their average length.

TARGET INDICATIONS

Telomere attrition is the hallmark to all age-related diseases and, as such, our technology can be applied as diagnostic, biomarker and product development tool to many disease areas including among others oncology, cardiovascular, CNS, immune diseases, infertility, respiratory and metabolic diseases.

We are currently targeting services associated with regenerative medicine and stem cell therapies as well as hematological cancers.

In addition we are pursuing collaborations on frailty studies and oxidative stress managements.

CURRENT STATUS

TAT testing and report for clinical testing in blood samples have undergone a series of improvements with emphasis in its robustness, reproducibility, lower coefficient of variation and versatility in order to provide accurate biological age and to be applied to a range of cellular sources.

We are currently offering TAT v3.0 that has been very well received in the clinic. CLIA certification process applied to dyskeratosis congenita diagnostic is ongoing.

For pre-clinical testing and corporate product development our technology has become part of efficacy studies of anti-oxidants, oncology drugs proof of concept and quality control in regenerative medicine products.

INNOVATIVE ASPECTS

- Life Length offers a range of services related to telomere biology and its analysis. Our proprietary technology is unique as it combines the depth of high content screening data with the throughput of a 384 well-plate format.
- Telomere length measured by our proprietary Telomere Analysis Technology (TAT®) has been established as the most accurate indicator of cellular aging and as the best approach to estimating biological age. Moreover, it has a demonstrated utility as a diagnostic and biomarker for age-related diseases (oncology, cardiovascular, metabolism, CNS…) and in quality control and characterization of cell therapy products.
- TAT® is complemented in house with other highly sophisticated assays such as telomerase activity measurement (TRAP assay), Telomapping® for tissue samples and Terminal Restriction Fragment (TRF). Each and the sum of these techniques represent robust and powerful biomarkers to complement current molecular techniques to evaluate drugs in development. The technology is ripe to commit to co-development proposals, direct investment and joint ventures.

IPR

Patents: Life Length licensed form CNIO the U.S. patent US 8,084,203 B2 which has been granted. This patent relates to Telomapping analysis for telomere measurement in biopsies.

Know-how: TAT is protected by industrial secret.

PARTNERING OPPORTUNITIES

Avenues of collaboration center on feasibility studies for R&D projects to validate telomere biology as a fit-for-purpose biomarker by TAT, Telomapping and TRAP as well as exploring high content screening assays.

Hospital Virgen del Rocío/Instituto de Biomedicina de Sevilla

PROFILE

IBIS is a comprehensive and multidisciplinary biomedical research facility focused on translational research on the most prevalent diseases, with the aim to promote rapid transfer of knowledge to the clinical setting, improving the quality of clinical and epidemiological research. To favor a real translational research, the IBiS is located in the campus of the **Virgen del Rocio University Hospital** (HUVR), one of the largest university hospitals in Spain, and it is functionally tied to it. The HUVR is one of the largest and most reputed health complexes in the Spanish National Health System, for its large volume of medical activity, its infrastructure and its technological facilities.

SPEAKER

Pablo Hervás Ballesteros, is head of the Technology Transfer Unit at Andalusian Public Foundation for Heath Research Management in Seville (FISEVI)/ Institute of Biomedicine of Seville (IBiS). He combines scientific & business background involved in a very wide range of activities in these areas, both in the public and private sector.

The research itself was led by **Dr. José Antonio Pérez Simón**, Director of the Clinical Management Unit of Hematology and Hemotherapy at Virgen del Rocio University Hospital and Head of the Cell Therapy and New Therapeutic Targets in Onco-hematology research group at Institute of Biomedicine of Seville (IBiS). He specialized in the Fred Hutchinson Cancer Research Center in Seattle (Washington, USA) and the Karolinska Institute in Stockholm (Sweden), so he was able to expand their knowledge in the field of transplantation (both in attendance and in research). Prestigious journals such as Blood, Leukemia, Journal of Clinical Oncology, American Journal of Hematology and Cell Transplantation have collected some of the over one hundred articles published research

pablo.hervas.exts@juntadeandalucia.es

PRODUCT

Cannabinoid agents for the treatment of multiple myeloma and related conditions.

MECHANISM OF ACTION

The research group has identified cannabinoids agents as a promising new therapy for multiple myeloma and related conditions. Several pathways have been explored to evaluate their mechanism of action. We have confirmed that these drugs act through the activation of caspase 8, 9, 3 and 2, inhibiting several MAP kinase pathways and modifying the ceramyde composition of cellular membranes. A program for synthetizing new chemical analogs to the original compound is being started through collaboration with the aim of developing new compounds showing improved efficacy and/or toxicity profile.

TARGET INDICATIONS

Agents useful in the treatment of multiple myeloma and other monoclonal gammopathies, such as plasma cell leukaemia, Waldeströn macroglobulinaemia and amyloidosis.

CURRENT STATUS

Mechanisms of action, in vitro toxicity and efficacy studies (both in vitro and in vivo), have been successfully completed with very promising preliminary results.

Proof of efficacy performed in MM animal models with positive results. Significant differences have been observed in terms of tumor growth, mice weigh and survival. In fact, at the highest dose tested, the drug was able to completelly abolish already stablished tumor growth with complete regression of the tumor mass.

INNOVATIVE ASPECTS

Although multiple myeloma (MM) is currently considered an incurable disease, the development of new drugs, such as proteasome inhibitors and immunomodulatory drugs (IMIDs), have allowed increasing survival of patients diagnosed with MM in the last few years.

Current strategies are based on the use of these new drugs as an induction treatment followed by autologous stem cell transplantation (in patients below 65 years) and followed by maintenance therapy.

Thus, we hope to transform MM from an incurable to perhaps a chronic disease. To reach this aim, it would be desirable to develop new drugs which combine both efficacy in terms of disease control but also have a low toxicity profile, so that they do not hamper quality of life, especially if used as maintenance therapy, and can be safely combined with some of the already available drugs to treat this disease.

According to our findings cannabinoids have both characteristics: they display a high cytotoxic effect against MM cells, but do not hamper viability of normal hematopoietic cells.

Furthermore, different types of cannabinoids are currently being tested in several indications, interestingly some of them aimed to avoid chemotherapy-related toxicity. Thus, these drugs have a very low toxicity profile which would allow them to be combined with other drugs and to be used as induction therapy or as maintenance.

IPR

This technology is covered by a Spanish Patent Application, filed on June 13th, 2013, as well as an International Patent application claiming priority from the former filed on June 13th, 2014.

A patentability report has been issued which concludes that, although the cannabinoid compounds originally assayed are publicly known (but are not protected by third intellectual rights), its use for the treatment of multiple myeloma would fulfill with the requirements of novelty and inventive step (second medical use invention).

Furthermore, sponsors, which obtain orphan designation, benefit from a number of incentives, including ten-year market exclusivity once the medicine is on the market.

PARTNERING OPPORTUNITIES

The research group is looking for a partner interested in a license and/or a collaboration agreement to further develop and exploit this innovative technology. It is also open to establishing partnerships for co-development of the technology before reaching the market and highly interested in applying to different funding calls, mainly to Horizon 2020.

UNIVERSIDAD DE VIGO

PROFILE

Universidade Vigo

THE UNIVERSIDADE DE VIGO (UVI) is a teaching and research Institution inaugurated in 1990, which is included amongst the best 400 (and the best 100 of those with less than 50 years; Times Higher Education 2012-2013, 2013-2014), amongst the TOP500 ARWU (Shanghai Ranking 2011, 2012), and occupies position 16th amongst the Spanish Universities in scientific outcome (Scimago Institutions Rankings SIR World Report 2011, 2012, 2013).

SPEAKER

Angel Rodríguez de Lera, Professor of Organic Chemistry**,** PhD from the Universidade de Santiago de Compostela in 1983. Postdoctoral stay at the University of California Riverside (1985-1988).

Assistant Professor Titular at Universidade de Santiago de Compostela in 1987, at Universidade de Vigo in 1996 and Full Professor in 1998. Visiting Professor at Albert-Ludswig-Universität, Freiburg (2000) and Université Paris-Sud (2006).

qolera@uvigo.es

PRODUCT

UVI5008, a multiple epigenetic modulator for the treatment of cancer.

MECHANISM OF ACTION

UVI5008 is the first triple action inhibitor of epigenetic enzymes (HDAC, DNMT and sirtuins). UVI5008 highly efficiently induces cancer cell-selective death in a variety of models and exerts its activities in several human tumor xenografts and genetic mouse models of human breast cancer in vivo.

Its anticancer activity involves activation of death receptors and ROS production in a mutually independent manner. All these studies confirm the drug activities, particularly its tumor selective and intra-tumoral action, and reveal its cancer therapeutic potential.

TARGET INDICATIONS

Cancer treatment. UVI5008 is a pro-drug with strong anti-proliferative and pro-apoptogenic potential. UVI5008 action is not critically dependent on p53, Bcl-2 modifying factor, BMF, and/or TNF-related apoptosis inducing ligand, TRAIL, because cell death is efficiently induced in cells mutated or deficient for these factors, limiting the risk of drug resistance development.

CURRENT STATUS

UVI5008 is a derivative of the natural product psammaplin A optimized after SAR studies, and has a disulfide bond, similarly to romidepsin. This drug candidate has been thoroughly characterized and its activities have been validated in enzyme assays, in in vitro (tumor cells, stepwise tumorigenesis systems), ex vivo (leukemia patients' blasts) and in vivo (human colon and breast cancer xenografts, and two murine genetic models of breast cancer) models of cancer.

UVI5008 induced both tumor-selective apoptosis in the stepwise tumorigenesis model and human hematopoietic cells (patients' blasts vs. CD34+ cells).

This epi-drug activates both TRAIL and ROS-mediated apoptosis. Intra-tumoral drug action was validated in all in vivo models. Pharmacokinetics supports favorable drug-compatible features. Initial mouse pharmacotoxicology revealed no significant toxicity at effective doses.

INNOVATIVE ASPECTS

- UVI5008 is an entirely novel type of anti-cancer drug with unique activities that have been validated in large number of pre-clinical settings.
- Currently there are only 4 epi-drugs that have been approved by the FDA (vorinostat, romidepsin, 5-azacytidine and decitabine), but none of them has the capacity to target multiple epigenetic enzymes as UVI5008. Moreover, vorinostat is, according to our own studies, dramatically more toxic that UVI5008 (in nude mice).
- UVI5008 addresses critical needs that are unmet by current cancer therapies. At present, we did not yet find a cancer cell line in our pre-clinical tests that would be insensitive to UVI5008. This concerns tumor cells that have lost the tumor suppressor p53, as well as leukemic blast that have been tested so far in ex vivo assays

IPR

Novel derivatives of psammaplin a, a method for their synthesis and their use for the prevention or treatment of cancer; Pub. No.: WO/2008/125988; Priority Data: 07290253.9 28.02.2007 EP; International Application No.: PCT/IB2008/001887; Publication Date: 23.10.2008; International Filing Date: 28.02.2008. Extended to USA, China, Israel, Australia, Japan, Canada and Europe

PARTNERING OPPORTUNITIES

Open to discussion for licencing or support for toxicity pre-clinical assays. The development of UVI5008 up to the stage of preclinical studies is a joint effort of four institutions: Universidade de Vigo (30%); Centre National de la Recherche Scientifique (30%), Seconda Universitá degli Studi di Napoli (30%) and University of Nijmegen (10%).

CIMA, CENTER FOR APPLIED MEDICAL RESEARCH

PROFILE

cima
CENTER FOR APPLIED MEDICAL RESEARCH
UNIVERSITY OF NAVARRA

The Center for Applied Medical Research (CIMA) is a biomedical research institution of the University of Navarra, based in Pamplona, Spain. CIMA performs high quality scientific work with a strong translational focus. These groups are interested in deciphering the complex molecular mechanisms underlying Alzheimer's Disease (AD) and drug discovery, efficacious and safe therapeutic agents to treat AD patients.

SPEAKER

Dr Julen Oyarzabal, Senior Scientist and Director of Translational Science and Head of the Molecular Therapeutics Program, got his PhD in Pharmaceutical Chemistry. After finishing his PhD in 1998, he moved to the University of California, San Francisco; and later, he joined the University of Southampton. In November 2001 he started working at Johnson & Johnson Pharma R&D in Toledo (Spain), and in 2006 he joined Spanish National Cancer Research Centre (CNIO) where he set up and led the Computational Medicinal Chemistry Section. Dr. Oyarzábal joined CIMA in 2010 and he is co-inventor of 15 published patents.

julenoyarzabal@unav.es

PRODUCT

Novel strategy for symptomatic and disease-modifying treatment of Alzheimer's Disease.

MECHANISM OF ACTION

Novel MoA, systems therapeutics approach: two independent and synergistic pathways, epigenetic and non-epigenetic, involved in synaptic plasticity and memory. Hitting these two different target classes leads to over-express memory-related genes as well as to reduce β-amyloid and tau pathology.

TARGET INDICATIONS

Therapeutic Area: CNS.

Indication: Alzheimer's Disease.

CURRENT STATUS

Lead compound, CM - 414, was tested in aged Tg2576 mice; in-vivo efficacy and safety:

- After chronic treatment (3 weeks), CM-414 reversed memory impairments in two different behavioral tasks: the Fear Conditioning and the Morris Water Maze (MWM) test.
- Memory recovery was maintained after a washout period of 4 weeks.
- AD pathological marks analysis. Treated Tg2576 mice showed a reduction in β–amyloid and Tau pathology as well as reversion in dendritic spine loss.
- Gene expression profile, in hippocampus of treated Tg2576 mice leads to identify the over-expression of memory related genes.

INNOVATIVE ASPECTS

First-in-class novel proprietary compounds simultaneously acting at two independent and synergistic pathways: epigenetic and non-epigenetic targets.

Multifactorial optimization process, from initial proprietary hits to lead compounds (e.g. CM-414):

- Design and synthesis of dual inhibitors: Hitting two independent targets, IC50 at low nM range,
- In–vitro efficacy, using cellular and Tg2576 (AD transgenic mice) primary neuronal cultures, according to AD related markers: C99, pTau, … EC50 at low nM range,
- Acceptable solubility, P450s profiling, permeability, hERG and plasma protein binding (unbound fraction). There is still room for improvement,
- Crossing BBB and showing in-vivo functional response at brain level (hippocampus), looking at biomarkers for each independent pathway,
- Therapeutic window, efficacy vs toxicity, >3 log units,
- Acceptable pharmacokinetics,
- In-vivo proof-of-concept.

IPR

Two patent applications for novel proprietary compounds, four different chemical series, are filed.

PARTNERING OPPORTUNITIES

CIMA is open to various types of partnerships with academia and biopharmaceutical companies in order to facilitate the advancement of the research, with the ultimate goal of improving patient quality of life. By joining capabilities and resources, this win-win cooperation facilitates the advancement in the different research stages, from target validation to lead optimization or early candidate development.