We care about cancer

PRESS RELEASE

Oncodesign presents novel LRRK2 inhibitor jointly discovered with Ipsen at the 2015 Neurosciences meeting in Chicago

- Molecule was discovered in strategic research collaboration with Ipsen for the development of new therapeutic agents against Parkinson's disease started in 2012
- Additional information is presented by clinical experts on inhibitory activity of the molecule on patient material

Dijon (France), October 21, 2015 – ONCODESIGN (FR0011766229 - ALONC), a biotechnology company serving the pharmaceutical industry in the discovery of new therapeutic molecules to fight cancer and other serious illnesses with no known effective treatment, will present today with Ipsen (Euronext: IPN, ADR: IPSEY), a global specialty-driven pharmaceutical company, the discovery and characterization of ODS2005294, a novel LRRK2 inhibitor they jointly discovered in a research collaboration started in January 2012. The molecule will be presented at the 45th annual meeting of the Society for Neurosciences that is taking place October 17-21 in Chicago, IL; this leading global meeting for presentation of novel discoveries in neuroscience is attended by over 30,000 experts in the field from over 80 countries.

ODS2005294 is a potent and selective small molecule derived from Oncodesign's Nanocyclix® technology. It shows good in vivo oral exposure and reaches high levels in the brain, where it demonstrates dose-related inhibition of LRRK2 phosphorylation with no obvious adverse effect at pharmacological effective doses. ODS2005294 is the most fully characterized lead of a series of Nanocyclix® inhibitors that have the potential to address the relevance and the application of LRRK2 kinase inhibition in the human brain as a potential treatment for Parkinson's disease. More advanced molecules in the series are currently under investigation.

Specific mutations in LRRK2 were identified in 2004 in familial cases of Parkinson's disease. Since then, this kinase is considered as one of the highest potential molecular targets against this neurodegenerative disorder. Despite being the subject of massive research efforts since 2005, no inhibitors have yet entered clinical stages of development, because of the difficulty to find potent, selective inhibitors that readily cross the Blood-Brain Barrier and that are completely safe for use in a chronic disease setting.

At the same meeting, in a French multi-center approach, clinical scientists from the Brain and Spine Institute (ICM, Paris) and the French clinical research network for Parkinson's disease and Movement Disorders (NS-Park, FCRIN), showed strong inhibition of LRRK2 induced phosphorylation on human peripheral blood mononuclear cells (hPBMCs) from Parkinson patients by ODS2005294.

"This new exciting LRRK2 inhibitor shows the progress we have made in the collaborative program with Ipsen since 2012. The combination of Oncodesign's Nanocyclix® technology for next generation kinase inhibitors with Ipsen's recognized expertise in CNS research has allowed us to identify a class of molecules that has the potential to advance towards candidate selection. Both Oncodesign and Ipsen are committed to continue their efforts to improve the lives of Parkinson's patients", said **Philippe Genne, Ph.D., Chief Executive Officer and founder of Oncodesign.**

"ODS2005294 is a potent and selective orally active molecule that reaches high levels in the brain and that has been shown to inhibit the phosphorylation of LRRK2 in the brain in animal models without side effects at efficacious doses. The work by clinical scientists on human blood cells shows that our compound also potently blocks the kinase activity of the human form of LRRK2, an essential condition to further advance the program", added **Jan Hoflack, Ph.D., Chief Scientific Officer of Oncodesign.**

About Parkinson's disease

Parkinson's disease (PD) is the most common neurodegenerative movement disorder afflicting 1% of the population aged 65 years and older. Clinical features include bradykinesia, rigidity and tremor. PD is characterized by progressive loss of dopaminergic neurons and accumulation of aggregation of α–synuclein protein in the brain. Only dopamine replacement therapy which compensates the dopamine neuronal loss reduces with some efficacy motor symptoms in PD patients but does not stop or slow the neurodegenerative process. At present, there are no proven neuroprotective or neurorestorative therapies. Disease modification is thus the most important goal in PD.

About LRRK2 target

Although PD is regarded as a sporadic disorder, 5-10% of PD cases are genetically inherited as familial. LRRK2 mutations represent the highest risk of familial PD and are also observed in sporadic patients. Pathological characteristics and clinical symptoms observed in patients carrying LRRK2 mutations are indistinguishable between familial and sporadic patients. LRRK2 is a multidomain protein which contains both GTPase and Kinase enzymatic activities where most pathogenic mutations are located. LRRK2 inhibition represents a potential neuroprotective therapeutic target for the treatment of PD.

About Oncodesign: www.oncodesign.com

Founded 20 years ago by Dr. Philippe Genne, the Company's CEO and Chairman, ONCODESIGN is a biotechnology company that maximizes the pharmaceutical industry's chances of success in discovering new therapeutic molecules to fight cancer and other serious illnesses with no known efficient treatment. Backed by unique experience acquired through more than 600 clients, including the world's largest pharmaceutical companies, and relying on a comprehensive technological platform combining state-of-the-art medicinal chemistry, advanced animal modeling and medical imaging, ONCODESIGN is able to predict and identify for every molecule, very upstream, its therapeutic use and its potential to become an efficient drug. Applied to kinase inhibitors, molecules that represent a market estimated at over 40 billion dollars in 2016 and accounting for almost 25% of the pharmaceutical industry's R&D investments, ONCODESIGN's technology has already enabled the targeting of 7 promising molecules with substantial therapeutic potential, in oncology and elsewhere, and the signing of partnerships, potentially worth €350 million in upfront payments should predefined milestones be reached, with pharmaceutical groups Sanofi, Ipsen and UCB. Based in Dijon, France, in the heart of the town's university and hospital hub, ONCODESIGN has 95 staff.

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