

Theranexus, Diverchim and Inserm awarded €4.7 million in funding under the “Innovations in biotherapies and biomanufacturing” call for proposals from France 2030 to develop a novel antisense oligonucleotide



Lyon, April 23rd, 2024

Sponsors of the PickASO project, a winner of the “Innovations in biotherapies and biomanufacturing” call for proposals from France 2030, Theranexus, Diverchim and Inserm’s ARNA laboratory were awarded €4.7 million in funding from French Government to develop a revolutionary autophagy activation therapy.

The PickASO consortium is led by Theranexus, a specialist in rare neurological diseases, and will include Diverchim, expert in the synthesis of active pharmaceutical ingredients, and Inserm’s ARNA laboratory, France’s leading academic institution in the field of antisense oligonucleotides (ASOs).

PickASO won the “Innovations in biotherapies and biomanufacturing” call for proposals under the France 2030 investment plan. The €4.7 M, 3-year funding package is attributed by the French government and operated by Bpifrance and breaks down into grants and repayable advances. With this funding Theranexus will handle development (up to entry into clinical development) of an antisense oligonucleotide, an innovative drug candidate targeting TFEB¹, the protein identified as the autophagy² regulator. The project draws on Theranexus’ capabilities in the areas of molecular biology, neuroscience and drug development and ARNA and Diverchim’s expertise in RNA chemistry and industrial active ingredient synthesis, respectively.

“We sincerely thank the French government for its financial support via France 2030 for the PickASO project. In line with our major strategic shift to rare neurological diseases initiated two years ago, via this collaboration with two leading players, Theranexus will be the first company to develop and bring to clinical trials an antisense oligonucleotide targeting a central autophagy pathway – the TFEB pathway – for patients with rare neurological diseases”, explained Theranexus Chairman and CEO, Mathieu Charvériat.

“The PickASO project is an opportunity for Inserm (ARNA unit) to showcase its expertise in the area of oligonucleotides for therapeutic applications and give international visibility to its Optoligo platform. Moreover, this project will allow Inserm to validate and illustrate the value of its innovations in the fields of bioconjugation and modification of nucleic acids, notably in the development of therapies targeting rare neurological diseases. In terms of commercialization, the transfer of patents developed at Inserm to French industries and biotech firms will foster the development of new technologies essential for the drug candidates of tomorrow”, said Philippe Barthélémy, Director of the Inserm ARNA unit.

¹ The protein TFEB is the recognized molecular switch for autophagy; its activation has been reported as an effective therapeutic remedy for numerous diseases, as shown in various *in vitro* or *in vivo* models.

² Autophagy is a cellular process involving small intracellular vesicles known as lysosomes used by cells to recycle part of their own material and destroy certain residues which accumulate and may become toxic for them.

“At Diverchim, we are very excited to collaborate on the PickASO project with such prestigious players as Theranexus and Inserm. Diverchim will be the first French company to offer ASO production capability that meets Good Manufacturing Practices (GMP) requirements to manufacture clinical and commercial batches. We see this collaboration as a unique opportunity to apply our expertise to a novel therapy. The combination of our expertise in the chemistry of active pharmaceutical ingredients and the cutting-edge capabilities of our partners promises significant advances in biotherapies. We are looking forward to tackling this challenge together and to helping provide innovative solutions to patients in need”, continued **Diverchim CEO, François Macarez**.

“The PickASO project embodies the collective commitment to offer new hope to patients with lysosomal diseases and boost the excellence of research and innovation in innovative therapies in France. Over and above this development, the PickASO project aims to structure a production sector for antisense oligonucleotides as there is currently none available in France for this very promising molecule that has great potential for the booming international market. This project is completely in the spirit of what the State is pursuing with France 2030, and in particular the health aspect: supporting and supporting health innovation to strengthen our sectors and our production capacities, and thus regain our sovereignty in health » specifies **Bruno Bonnell, general secretary for investment in charge of France 2030**.

TFEB, an autophagy regulator

Autophagy is a cellular process involving small intracellular vesicles known as lysosomes used by cells to recycle part of their own material and destroy certain residues which accumulate and may become toxic for them. This process, which earned the Japanese scientist Yoshinori Ohsumi the Nobel Prize in 2016, is essential for cell and particularly neuron function, and is disrupted in a group of rare diseases called lysosomal diseases. Niemann-Pick type C, Sanfilippo and Batten diseases are examples of lysosomal diseases. But beyond these rare diseases, autophagy generally decreases with age and an autophagy deficiency is now identified as a determinant of diseases such as Parkinson’s, amyotrophic lateral sclerosis (ALS) or age-related macular degeneration (AMD). The protein TFEB is the recognized molecular switch for autophagy³; its activation has been reported as an effective therapeutic remedy for numerous diseases, as shown in various *in vitro* or *in vivo* models.

Overview of the PickASO project

Firstly, Theranexus had already identified an ASO promoting autophagy via direct action on TFEB. The first step in the PickASO project will involve assessing Inserm’s lipid conjugation technology for improving the pharmacokinetic profile of ASOs and their ability to penetrate cells. Meanwhile Diverchim and Inserm will work together to implement semi-automated GMP (Good Manufacturing Practice)-compliant production of this ASO; and this will be a big first for France. Theranexus will also handle regulatory preclinical characterization. On project completion, the drug candidate’s formulation will be approved, paving the way for clinical development.

About Theranexus

Theranexus is an innovative biopharmaceutical company that emerged from the French Alternative Energies and Atomic Energy Commission (CEA). It specializes in the treatment of rare neurological diseases. The company has a unique platform for the identification and characterization of advanced therapy drug candidates targeting rare neurological disorders and an initial drug candidate in clinical development for Batten disease.

Theranexus is listed on the Euronext Growth market in Paris (FR0013286259 - ALTHX).

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³ <https://pubmed.ncbi.nlm.nih.gov/33259088/>; <https://pubmed.ncbi.nlm.nih.gov/27252382/>

About Diverchim

Diverchim is a renowned specialist in the synthesis of sophisticated small-scale organic molecules. As a high value-added service provider for the pharmaceutical, cosmetics and biotechnology industries for over two decades, Diverchim offers its clients an extensive range of services from design and manufacturing of active substances to the drafting and filing of regulatory applications.

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About Inserm

The ARNA laboratory (Nucleic Acids: Natural and Artificial Regulations) in Bordeaux includes approximately 120 researchers across 6 interdisciplinary teams (chemistry, biology, biophysics) and 3 core facilities, half of which are permanent staff of Inserm, the CNRS and the University of Bordeaux (INSERM U1212 / UMR CNRS 5320, UB, <https://arna.cnrs.fr>). ARNA is a structure unique to Europe that implements research focused on the study of nucleic acids. The laboratory boasts expertise that is unique to France and offers proprietary oligonucleotide bioconjugation technologies to improve the pharmacological and biodelivery properties of ASOs, as shown through numerous collaborations.

About France 2030

- **Governed by Bruno Bonnell Secretary General of Investments (SGPI)** on behalf of the Prime Minister.
- **A double ambition:** Sustainably transform our key economic sectors (energy, healthcare, automobile, aeronautics, space...) whilst increasing the competitiveness of French companies in these respective sectors. France 2030 will support all cycles of innovation up until industrialisation: from research to development of ideas, to prototypes, to production of new services and products.
- **Unprecedented funding:** €54 billion will be invested into companies, universities and research centers. France 2030 is in possession and has control over its €54 billion fund and will invest 50% of the budget into the decarbonisation of French economy and the latter 50% in sustainable innovations and ground-breaking technologies.
- **Collective action:** Strategic actions and plans will be thought of and deployed in coordination with economic actors, academia and field experts, as well as local and European institutions.

For more information: <https://www.gouvernement.fr/france-2030>

About Bpifrance

About Bpifrance Bpifrance Investissement is the management company that handles Bpifrance's equity investments. Bpifrance is the French national investment bank: it finances businesses – at every stage of their development – through loans, guarantees, equity investments and export insurances. Bpifrance also provides extra financial services (training, consultancy) to help entrepreneurs meet their challenges (innovation, export...). For more information, please visit: <https://www.bpifrance.com/>

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