

General description: OZ Biosciences (OZB) is a French corporation founded in 2003 that creates and develops new research tools focused on delivery technologies of bioactive materials (nucleic acid, protein, peptide...) into living organisms intended for the life sciences research world and pharmaceutical industries. The proprietary technologies of OZB are based on new lipids and magnetic-based methods. OZB employs 8 persons and has a turnover of > 500K€.

Website: www.ozbiosciences.com

Expertise: OZB develops research tools based on multiple technologies such as lipids, polymers and nanoparticles. OZB proposes various generic and specific transfection reagents for nucleic acids and proteins (Magnetofection: magnetic assisted transfection based on iron oxide nanoparticles and TEE-technology: cationic lipids based formulations). OZB current R&D activities are focused on the synthesis of new lipids, polymers, targeting ligands and nanoparticles for the delivery of various nucleic acids, proteins and other molecules. OZB major expertise in non viral gene therapy. The current research activities of OZB R&D department are: development of siRNA delivery systems for in vitro and in vivo applications, design of transfection technology for haematopoietic cells, ex vivo gene therapy and for neurosciences applications (neurons, organotypic slices), development of new lipids and polymers for cell specific molecular delivery systems and expansion of OZB Magnetofection technology to specific cells, tissues and in vivo applications.

Facilities: OZB is located in the Scientific Park of Marseille-Luminy, France and has about 200 m2 of laboratory and offices. OZB is equipped with modern facilities for cell biology, molecular biology, chemistry and biochemistry. OZB has access to many technological platform located in the scientific park such as P2-P3 laboratory, FACS, imaging (confocal, electron) and animal facilities. OZB has established a convention with INSERM.

Other European projects: OZB participates in three FP6—LSH programs: Improved precision of nucleic acid based therapy of cystic fibrosis. Contract. LSHB-CT-2004-005213, Combined isolation and stable non-viral transfection of hematopoietic cells – a novel platform technology for ex vivo hematopoietic stem cell gene therapy. Contract. LSHB-CT-2006-8, Functional genomics and neurobiology of epilepsy: a basis for new therapeutic strategies. Contract. LSHM-CT-2006-037315

Role in the project: OZ Biosciences develops innovative delivery reagents for biologically active substances, in particular nucleic acids and proteins, and commercializes delivery systems to research laboratories worldwide. In this context, OZB transforms research protocols from scientific laboratories into robust, quality controlled protocols applicable with high reproducibility for general use. OZB contributes to this project and provides to all Partners proprietary cationic lipid and magnetic nanoparticle transfection reagents for the formulation of nonviral and adenoviral nucleic acid vectors. OZB will optimise these reagents towards the use in gene activated matrices, in particular in view of in vivo use. GAM-mediated transfection/transduction will be optimised in the presence of full blood and blood components. Transgene expression levels in relevant cells (primary chondrocytes) will be followed over time. One important role of OZB is in dissemination: Most techniques, protocols and reagents developed in specialized research laboratories emerging during basic research never become available to the broad scientific community. Based on licensing agreements with the Partners, OZB aims at transforming research protocols and reagents to research tools and kits that can be commercialized for use in basic research. This activity will contribute to a broad dissemination of the results of this project within the scientific community.

Workpackages responsibility: Magnetic nanovectors; Assembly of components <u>WP01</u>, <u>WP02</u>, <u>WP03</u>, <u>WP09</u> (WP = Workpackage)

Key personnel

Olivier Zelphati : (M), CEO of OZB, is a specialist of molecular delivery systems. He has a PhD degree in Immunology, carried out his post-doctorate at the UCSF (USA) and worked at Vical Inc (USA). He founded Gene Therapy Systems (USA). He has many publications and patents in the field of drug delivery systems and received several awards and grants.

<u>Cédric</u> <u> Sapet</u>: (M) has a PhD in cell biology. He worked 4 years at University of Pharmacy, Marseille and two years at the Army Topical Medicine Institute. He has 7 years

experiences in cell biology, virology and microarrays. Research interests are gene therapy, endothelial physiopathology, cell signalling, vector targeting and high throughput screening.

Nicolas Laurent : (M) has a PhD in chemistry. He worked 4 years at the University of Lyon, 1 year at Idealp'Pharma (France) and 3 years at University of Manchester (UK). He is working in the field of chemistry since 8 years. Research interests are: organic & analytical chemistry, lipids synthesis, polymers, glycoconjugates and peptide chemistry.

Patents:

1. Bergemann C. & Plank C. (2001). "Method for transfecting cells using a magnetic field"; European Patent N°2001/0949436; US Patent N°2002/0086842; International (PCT) WO02/00870.

2. Chan, D. "Magneto-Biolistic Methods". US Patent number 5,753,477 issued May 19th, 1998, taking priority from USPTO application number 60/617,685 on March 19th, 1996.

3. Jean Haensler "Use of a cationic amphipathic compound as a transfection agent, vaccine additive or drug", French Patent #FR9504615, US#6,124,270, EP#9504615 and worldwide WO96/32102.

4. Zelphati O. and Moutard S. New class of cationic lipids for delivering active agents into cells». Patent filed 19/12/2007, FR Patent N° 07/08861

5. Felgner P. and Zelphati O. (2000) "Chemical Modification of DNA Using Peptide Nucleic Acid Conjugates". US Patent # 6,165,720and International Patent # WO 99/13719.

Specific awards and certifications: OZB is winner of several prestigious awards:

1. 2003, National Award for innovative company from the French Minister of Research & New Technologies.

2. 2004, "Masters de la Création d'Entreprise" for inventive company, awarded by the French Sénat.

3. 2004, 3rd National award for innovative and durable development from the Young Economic Chamber of Aix, France.

4. 2003, PACA- Entreprendre Award (French organisation of entrepreneurs)

5. 2006, Créa 13 XV departmental Award from the Conseil General of Bouches du Rhone