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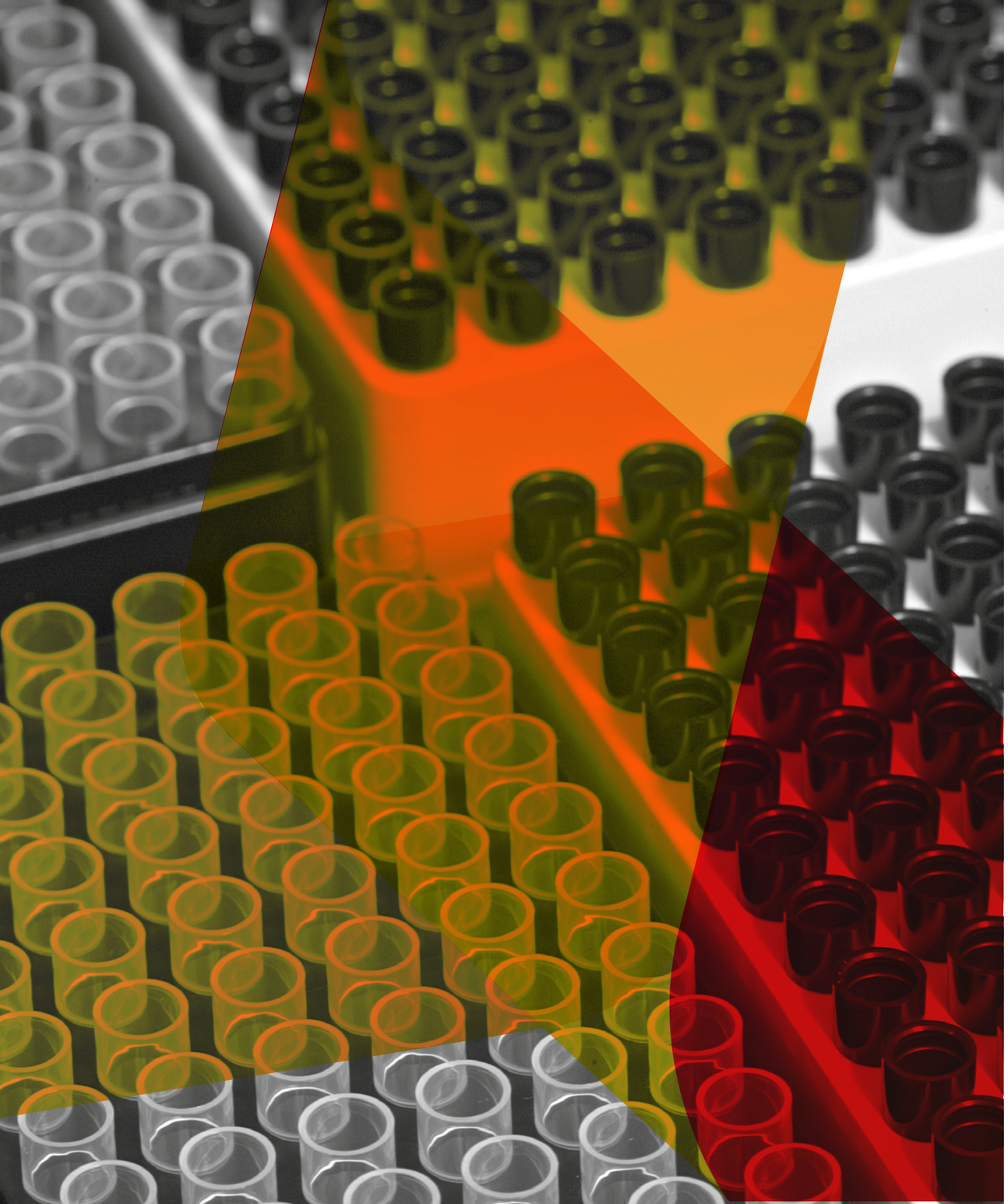
LES ENTREPRISES DU MÉDICAMENT

LES RENCONTRES INTERNATIONALES DE *BIOTECHNOLOGIES* 2015



LES RENCONTRES INTERNATIONALES DE *BIOTECHNOLOGIES* 2015

A brave new world for health innovation !

In today's virtual world, actual meetings and exchanges in finite time and space are becoming the exception, and this accounts for our decision to organize the first International Biotechnology Meeting, as we strongly believe that close links must be encouraged and strengthened, since therapeutic innovation will naturally flow from flourishing partnerships between “biotech” and “pharma”, between small and big companies.

The first International Biotechnology Meeting focused on rare diseases is especially designed to bring together selected French biotechnology companies and major worldwide pharmaceutical companies. Not surprisingly, the biotechnology industry is spotting plenty of opportunities in the field of rare diseases. Several companies which sprouted up in recent years aim at developing therapies that fight Duchenne muscular dystrophy, amyotrophic lateral sclerosis as well as Retinitis Pigmentosa.

These start-ups significantly fuel the shift towards innovation in rare disease therapies and cast much light on the complementary nature of projects conducted by biotech and research and development undertaken by industrials.

French life science research and biotechs have yet demonstrated their potential and attractiveness towards international pharma and investors. This first International Biotechnology Meeting is the result of a growing ecosystem, the bio-side of the French Tech, fostering in the last years several unicorns and international leaders, yet to be multiplied.

For patients, for innovative healthcare, the clear choice is to build a vibrant, collaborative and modern scientific ecosystem that underpins great health and technological innovation.

That is our key ambition which in turn will ensure greater competitiveness for the French healthcare industry as a whole.

Nicolas DUFOURCQ
General Manager BpiFrance

Philippe LAMOUREUX
General Manager Leem

Bpifrance, filiale de la Caisse des Dépôts et de l'État, partenaire de confiance des entrepreneurs, accompagne les entreprises, de l'amorçage jusqu'à la cotation en bourse, en crédit, en garantie et en fonds propres. Bpifrance assure, en outre, des services d'accompagnement et de soutien renforcé à l'innovation, à la croissance externe et à l'export, en partenariat avec Business France et Coface.
Bpifrance propose aux entreprises un continuum de financements à chaque étape clé de leur développement et une offre adaptée aux spécificités régionales.
Fort de 42 implantations régionales (90 % des décisions prises en région), Bpifrance constitue un outil de compétitivité économique au service des entrepreneurs. Bpifrance agit en appui des politiques publiques conduites par l'État et par les Régions pour répondre à trois objectifs :
• Accompagner la croissance des entreprises ;
• Préparer la compétitivité de demain ;
• Contribuer au développement d'un écosystème favorable à l'entrepreneuriat.

Avec Bpifrance, les entreprises bénéficient d'un interlocuteur puissant, proche et efficace, pour répondre à l'ensemble de leurs besoins de financement, d'innovation et d'investissement.

Plus d'information sur : www.bpifrance.fr - http://investissementsdavenir.bpifrance.fr/ - Twitter : @bpifrance

Bpifrance, a subsidiary of the French state and the Caisse des Dépôts and the entrepreneurs' trusted partner, finances businesses from the seed phase to IPO, through loans, guarantees and equity investments. Bpifrance also provides operational services and strong support for innovation, export, and external growth in partnership with Business France and Coface.
Bpifrance offers to businesses a large range of financing opportunities at each key step of their development, including offers adapted to regional specificities. With its 42 regional offices (90% of decisions are made locally), Bpifrance represents a strategic tool for economic competitiveness dedicated to entrepreneurs. Bpifrance acts as a back-up for initiatives driven by the French State and the Regions to tackle 3 goals:
• Contribute to SME's growth
• Preparing tomorrow's competitiveness
• Contributing to the development of a positive entrepreneur ecosystem.

With Bpifrance, businesses benefit from a powerful, efficient and close representative, to answer all their needs in terms of financing, innovation and investment.

More info @ www.bpifrance.fr - Follow us on Twitter : @bpifrance

Le Leem en quelques mots
Organisation professionnelle fédérant les entreprises du médicament, le Leem (Les Entreprises du Médicament) s'inscrit au cœur des grands enjeux de santé. Dans un contexte sans précédent de mutation scientifique et industrielle, il se mobilise, avec ses 270 adhérents et avec leurs 100 000 collaborateurs, pour promouvoir l'innovation et le progrès au service des patients, et pour renforcer l'excellence française en termes de recherche et de production. Promoteur de comportements responsables au sein du secteur, le Leem contribue, par une démarche de qualité, de sécurité et de transparence, à renforcer la confiance dans le médicament ;

Le Comité Biotechnologies du Leem représente la composante biotechnologies de l'activité des entreprises du médicament réunies en France au sein du Leem. Il se positionne comme un think tank sur la recherche et l'innovation.
Il alimente dans ce cadre les réflexions du Leem par des études, des analyses et des propositions, avec une attention particulière donnée aux tendances en matière d'innovation dans le domaine des médicaments afin de développer l'attractivité de la France. Il est aussi à l'initiative de conférences grand public et de rencontres B to B entre grandes entreprises pharmaceutiques et sociétés de biotechnologies.

Plus d'information sur : www.leem.org –- Twitter : @LeemFrance

Leem in a few words
Leem (Les Entreprises du Médicament) is the professional organization representing drug companies operating in France.
With a 100,000-strong workforce, these high-tech companies are striving to discover and develop drugs and vaccines to treat diseases.Their presence in 300 manufacturing and R&D sites throughout the country makes them key drivers of France's economic vitality.

Therapeutic innovation, drug safety, industrial and scientific attractiveness, economic efficiency and responsible practices are the core areas of its activity and commitment.

Aware of the innovative impact of biotechnologies in the health field, and wishing to take advantage of France's specific biosciences bases, Leem created the Leem Biotechnology Committee to help and promote the development of biotech activities in France.

More info @ www.leem.org - Twitter : @LeemFrance

Objectif

Les collaborations entre petites sociétés de biotechnologies et grandes compagnies pharmaceutiques sont clés pour construire une industrie du médicament innovante et efficiente. Les derniers chiffres publiés par l'agence américaine du médicament (FDA) et son homologue européen, l'agence européenne du médicament (EMA) montrent que 90% des 470 désignations orphelines obtenues en 2014, sont issues de demandes de sociétés de biotechnologies.

Les petites sociétés de biotechnologies sont un vecteur majeur de l'innovation pharmaceutique.
Faire se rencontrer industriels internationaux et entreprises de biotechnologies est donc un enjeu hautement stratégique. C'est pourquoi les Entreprises du médicament (Leem), son Comité Biotechnologies et la Banque publique d'investissement (BpiFrance) se sont associés pour organiser les premières Rencontres Internationales de Biotechnologies (RIB).

PERIMÈTRE
Cette première édition des Rencontres Internationales de biotechnologies couvre la thématique choisie en 2015

DES MALADIES RARES A LA MÉDECINE PERSONNALISÉE

incluant
- **les thérapies classiques, géniques et cellulaires participant au développement de thérapies dans le champ des maladies rares et/ou des "orphan drugs" ainsi que toutes les technologies associées (omiques …)**
- **le développement de modèles animaux pour le screening de médicaments et de biomarqueurs prédictifs de l'efficacité et de la toxicité du traitement**
- **le repositionnement de thérapies de pathologies classiques pouvant avoir un impact sur les mécanismes de pathologies rares.**

SÉLECTION

La sélection s'est effectuée en deux étapes successives : la première menée par les pôles de compétitivité Santé, la deuxième par un Comité de sélection indépendant composé d'industriels, d'investisseurs et de chercheurs.

Objective

Collaborations between small biotech companies and "big pharma" are today a vital key lever to stimulate in order to build up an innovative and efficient pharmaceutical industry. The latest official figures issued both by the American Food and Drug Administration (FDA) and the European Medicine Agency (EMA) show that 90 % of the 470 orphan product designations granted in 2014, come from biotech companies.

Those small companies represent a major vector of tomorrow's innovation.
It is therefore highly strategic to facilitate and stimulate interactions between biotech and industrial partners. In view of that innovation trend, the French Union of pharmaceutical industries (Leem), its Biotech Committee and the French public investment bank (BpiFrance) have joined together to launch the first International Biotech Meeting, between French biotech and pharmaceutical companies.

SCOPE
This edition of the International Biotech Meeting covers, through presentations of selected French biotech companies, the following scope:

FROM RARE DISEASES TO PERSONALIZED MEDICINE

- **from innovative therapies, including gene therapy, cellular therapy to drug repositioning in rare diseases / orphan drugs**
- **to development of animal models for drug screening**
- **and to new global technologies for the identification of individual biomarkers for diagnosis and prediction of treatment efficiency and toxicity**

SELECTION

The first International Biotech Meeting is grounded on a two-step selection process, conducted in the first phase by French health bioclusters and in the second phase, by an independent scientific selection committee made up of industrials, investors and academics.

LES RENCONTRES INTERNATIONALES DE BIOTECHNOLOGIES 2015

L'état des lieux du tissu des biotechnologies santé en france

Le tissu français des entreprises de biotechnologies santé est important et entreprenant : une récente étude menée par le Comité Biotechnologies du Leem montre en effet que fin 2013, la France concentre sur son territoire 457 sociétés de biotechnologies (contre 388 en 2011) employant 33 000 personnes, la plaçant ainsi au deuxième rang européen derrière la Grande-Bretagne (1 073 entreprises et 26 000 personnes employées).

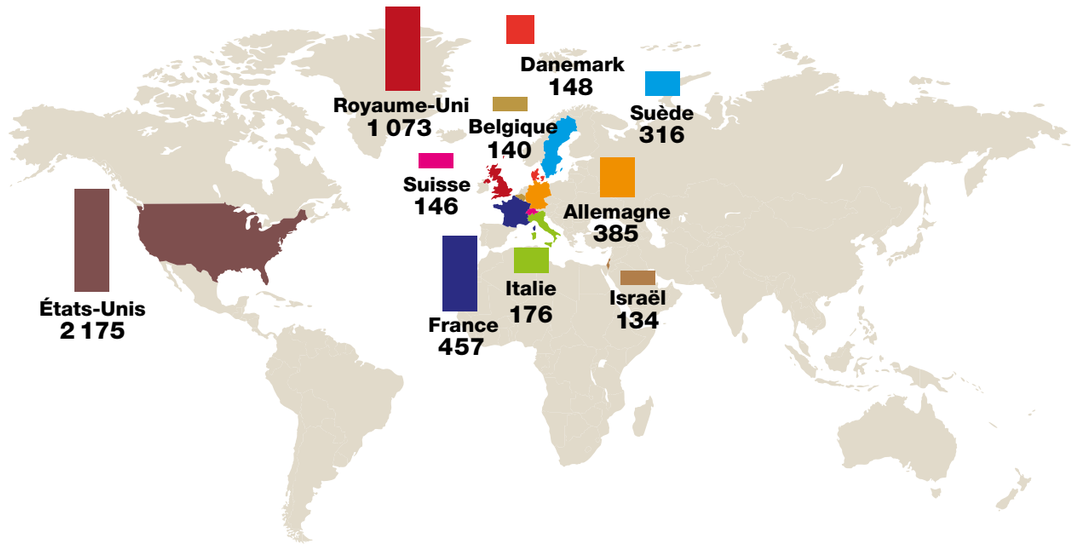
Pour en savoir plus :
www.leem.org/article/l-etat-des-lieux-du-tissu-des-biotechnologies-sante-en-france

The health biotechnology sector landscape

The French health biotechnology sector is important and flourishing : a recent study conducted by Leem Biotechnology Committee shows that it has grown to 457 companies in 2013, compared to 388 in 2011, employing 33 000 people. As far as health biotechnology is concerned, France ranks second in Europe, behind Great-Britain which concentrates 1073 companies and 26 000 employees.

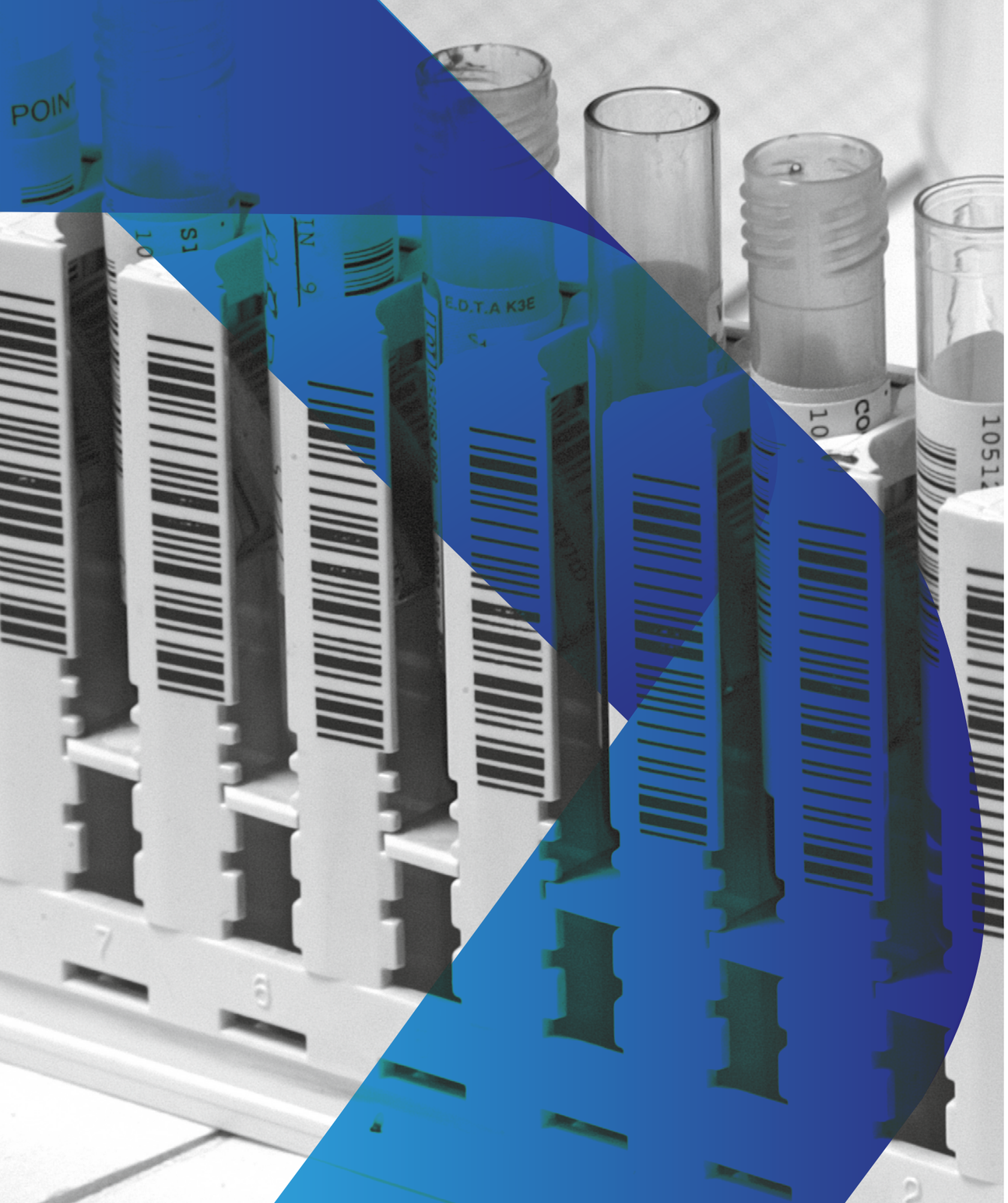
To find out more:
www.leem.org/article/l-etat-des-lieux-du-tissu-des-biotechnologies-sante-en-france

Nombre d'entreprises de biotechnologies
Number of biotechnology companies



LES RENCONTRES INTERNATIONALES DE BIOTECHNOLOGIES 2015

Sommaire / Table of contents



LES RENCONTRES INTERNATIONALES DE *BIOTECHNOLOGIES* 2015

Programme / Program

9 - 9:15 Ouverture / Opening

9:15 - 9:50 Première session / First session
- GENETHON
- HEMARINA
- LYSOGENE

9:50 - 10:30 Deuxième session / Second session
- AAVLife
- METAFORA
- NEUROCHLORE

10:30 - 11 Pause café / Coffee break

11 - 11:40 Troisième session / Third session
- PROGELIFE
- THERACHON
- SENSORION

11:40 - 12:20 Quatrième session / Fourth session
- ANAGENESIS
- ALIZE Pharma
- INFLECTIS Biosciences

12:30 - 14 Déjeuner / Lunch break

14 - 18 Rencontres B to B / B to B meetings

LES RENCONTRES INTERNATIONALES DE BIOTECHNOLOGIES 2015

Le comité de sélection
Selection committee

Les sociétés de biotechnologies ont été sélectionnées à la fois sur leur thématique de recherche, l'excellence de leur projet et leur volonté de nouer des alliances avec des entreprises pharmaceutiques.

Un comité de sélection mixte et indépendant a évalué les sociétés candidates sur la base de leurs dossiers et de leurs présentations orales.

Biotech companies have been selected on the basis of their research area, their scientific excellence and their will to forge collaborations with big pharmaceutical companies.

A joint independent committee has proceeded to their evaluation, based both on their written and oral presentations.

Zeina ANTOUN
Directeur de la recherche clinique. GSK

Zeina Antoun holds an MD degree from the American University of Beyrouth and has an American Board certification in Internal Medicine, and a University Diploma in Infectious Diseases.

Between 1994 and 1998, she worked at Bichat, Claude-Bernard Hospital as a full-time clinician in Infectious Diseases and HIV.

In 1998, she joined GlaxoSmithKline France, Clinical research unit in Virology, and was then appointed in 2004 as Director, Clinical research in Virology, Therapeutic Vaccines, Oncology and Respiratory diseases. In 2007, Zeina Antoun created a new department dedicated to Early phase research, New domains and Academic alliances, creating many links between GSK R&D groups and French Public research teams.

Zeina Antoun is a member of several external working groups at LEEM, ARIIS, Investment funds and other boards. She is also co-author on many publications.

Cécile BROSSET
Directrice du développement. BpiFrance

After 5 years at Bain & Company, a top strategy consulting firm, Cecile joins FSI (French sovereign fund), now having merged with Oseo and CDCE to build Bpifrance, the French public investment bank.

In 2013 she becomes Director of Development for Innovation at Bpifrance. She has actively worked at defining new strategic axes ("NOVA") to foster innovation and entrepreneurship in France, and accelerate growth to create future leaders. She has been an active member of the French Tech team since its origins and keeps mobilizing the French ecosystem of entrepreneurs and investors to help its current transformation.

Since 2015, Cécile is leading a new activity, Bpifrance Le Hub, aiming at catalyzing connections between large corporations, SMEs and start-ups, and accelerate business partnerships and acquisitions. Bpifrance Le Hub covers 3 different offers: an intermediation service helping major companies source and acquire start-ups (Hub Corporate), a new location to gather and debate about innovation inside Bpifrance Parisian offices (Hub Network), and an acceleration program to help late stage start-ups of all sectors commercialize and grow in France and abroad (Hub Start-up)

Pascale AUGÉ
Présidente du directoire d'Inserm transfert

Before joining Inserm Transfert, Pascale had led Institut Pasteur's Technology Transfer and Entrepreneurship office since 2011. She has more than 15 years' experience in the fields of technological innovation in life science and health technology, research transfer and entrepreneurship, and the biotechnology start-up industry.

She headed business development at Neurotech, Entomed and AB Science then operational strategy in the sciences and health technology, and ran Ernst & Young activities in France. She also chaired the evaluation committee reviewing "high-market-potential products, technologies or services" at France's Agence Nationale de la Recherche from 2008 to 2011.

Marie-Pierre CHEVALIER
Directrice des alliances strategiques

As Strategic Alliances Director at Pfizer France since 2009, she is responsible for identifying potential R&D partners within the French R&D landscape of excellence. The objective is to develop sustainable Research Partnerships in France with Academic Institutions or Biotech companies for projects fitting well with Pfizer strategic research needs.

She joined Pfizer in 2003 as Business Development manager, leading the Oncology portfolio management. Then, she moved as Medical Communication Team Leader in Oncology. In 2007, she took the position of the Medical Communication Director, delivering a Advisory Board strategies and activities. Prior to joining Pfizer, she started her pharmaceutical career at Pharmacia in the Oncology Business Unit and served in various positions from clinical research to marketing responsibilities.

She graduated as Doctor in Medicine and held a master degree in Management at the University Pierre & Marie Curie, Paris.

She is currently member of the Strategic Committee of InnoBio fund (Venture Capital), member of the Executive Committee at ARIIS (National Alliance for Research and Innovation in Health Industries) and member of the Board of the LEEM Biotech.

Pr Pierre CORVOL

Professeur émérite au Collège de France

Pierre Corvol, MD, is Professor emeritus at the College de France and Honorary Administrator of the College de France. He was Chief of the Hypertension Unit of hopital Broussais in Paris (1986 – 1999) and Scientific Director of the Inserm research unit Vascular pathology and renal endocrinology (1982 – 2006). His research is focused on the study of the hormonal regulation of blood pressure. He showed the crucial role of the renin angiotensin aldosterone system in the control of the cardiac and renal function. His research team contributed to a great extent to the developpment of drugs currently used in the treatment of high blood pressure and cardiovascular diseases. He initiated the first studies on the genetic basis of human hypertension. He has recently worked on angiogenesis and the remodeling of the arterial vascular wall in cardiovascular pathologies.
He is a member of the french Academy Sciences, of the Academy of Medicine and of the American Academy of Arts and Sciences. He was awarder many prizes, among them the Ciba Award for Hypertension Research (1985) and the Grand Prix Inserm (2006).

Main publications (among 732) : renin and angiotensin converting enzyme cloning (Nature, 1982 ; PNAS, 1988) ; molecular genetics of human hypertension (Cell, 1992) ; fonctional consequences of inactivating mutations of the renin angiotensin system in humans (Human Mol. Genetics, 2011 et 2013).

Pr Marc HUMBERT

Centre de Référence de l'Hypertension Pulmonaire Sévère, Hôpital Bicêtre (APHP)

Marc Humbert, MD, PhD, is Professor of Respiratory Medicine at the South Paris University in Le Kremlin-Bicêtre, France. In addition to his academic responsibilities, he is a consultant and specialist at the National Reference Centre for Pulmonary Hypertension at the Department of Respiratory and Intensive Care Medicine, Hôpitaux Universitaires Paris-Sud, Assistance Publique Hôpitaux de Paris, France. Along with membership to several scientific councils and institutes, Marc Humbert is the past Chairman of the Research Committee of the Assistance Publique Hôpitaux de Paris and current Director of the INSERM Unit "Pulmonary Hypertension: Pathophysiology and Innovative Therapies" and of the "Thorax Innovation" University Hospital Department. Since January 2013, he is the Chief Editor of the European Respiratory Journal. He has published widely in the fields of pulmonary hypertension and pulmonary inflammation in the most prestigious medical journals and has received several distinctions including the Cournand Lecture Award from the European Respiratory Society (2006) and the Descartes-Huygens Award from the Royal Netherlands Academy of Arts and Sciences (2009). Thomson Reuters listed Marc Humbert as one of the world's most influential scientific minds in the field of Clinical Medicine (2014). Marc Humbert is co-chairing the 2015 ESC/ERS guidelines on Pulmonary Hypertension.

Christian DELEUZE

Président Genzyme France

Christian Deleuze is currently the President of Genzyme France and Genzyme Polyclonals, part of the Sanofi Group. He joined the company in 2010 and leads the company's strategic planning in line with the global Genzyme mission, ensuring patients are provided with treatments, driven by cutting-edge science and a commitment to treating unmet medical needs in Rare Diseases, (Gaucher, MPS1, Fabry and Pompe disease) and since 2014, in the Multiple Sclerosis therapeutic area.

He obtained his medical degree from, Lariboisière Saint-Louis in Paris and graduated from the ESSEC Business & Management School In the early 90's. He began his professional pharmaceutical career at Bayer Pharma, and from there went on to hold different positions at Searl, Pharmacia and Pfizer where he was Marketing Director and responsible for the launch of Celebrex, in charge of strategy and tactics for branding and global positioning.

In 2003 he created the French subsidiary of Sankyo Pharma, which became Daiichi Sankyo France. In his role as President and founder, from 2003 to 2010, he positioned France as the European leader for the Japanese group, with 500 employees.

Today Christian is also President of the Rare Disease Committee at the LEEM (the French pharma syndicate), where he sits on the board and is a member of both the Administration Committee and the Biotechnology Committee. The LEEM Rare disease group unites the rare disease community; pharmaceutical companies, association representatives and health institutions dedicated to the orphan disease cause.

Chahra LOUAFI

Directrice d'investissements. BpiFrance

Chahra Louafi is an investment director and head of the biotherapies and rare diseases fund of Bpifrance (formerly CDC Entreprises). Before joining CDC Entreprises in 2001, she was in charge of project development and company creation for a private incubator that specialised in biotechnologies.

She is Vice-Chairman of the supervisory board of Inserm Transfert Initiative and a member of the supervisory board of Cap Décisif Management. She also sits on the boards of directors of DBV Technologies, Sensorion, Eyevensys, MedDay, Pixium Vision, Lysogene, AAVLIE and Mnemosyne.

Pr José-Alain SAHEL

Directeur de l'Institut de la Vision

José-Alain Sahel is Professor of Ophthalmology at Pierre and Marie Curie University Medical School, Paris, France and Cumberlege Professor of Biomedical Sciences at the Institute of Ophthalmology-University College London, UK. He is Chairman of Departments of Ophthalmology at the Quinze-Vingts National Eye Hospital and at the Rothschild Ophthalmology Foundation. He coordinates the Paris-based Ophthalmology Clinical Investigation Center, overseeing more than 50 clinical trials, some of them within the most advanced areas of biomedical technologies worldwide, such as retinal implants and gene therapy. Prof. Sahel heads the National Reference Center for Retinal Dystrophies and chairs a network of more than 90 European clinical trial centres on retinal diseases.

José Sahel is founder and director of the Vision Institute in Paris, a site for translational research on treatments for currently untreatable inherited and age-related ocular diseases that comprises 18 principal investigators and more than 250 staff members, and functions in synergy with the Quinze-Vingts National Eye Hospital. The primary focus of Sahel's fundamental and clinical research is the understanding of the mechanisms associated with retinal degeneration, together with the conception, developing and evaluation of innovative treatments for retinal diseases (e.g. stem cells, gene therapy, pharmacology, and artificial retina). Sahel and his collaborators discovered the protein Rod-derived Cone Viability Factor (RdCVF), a molecule secreted in normal retina that protects cone photoreceptors. Besides research on developmental biology, functional genomics, physiology and therapeutics, his laboratory conducts research on the development of high resolution in vivo cellular imaging, relevant biomarkers and disease models in an environment adductive to industrial partnerships.

José Sahel was coordinator of the European Framework Program-6 largest integrated project on retinal functional genomics, neuroprotection, ageing and now therapy 'EVI-Genoret'. He sits on the Executive SAB of Foundation Fighting Blindness. Sahel coordinates large scale European Research programs including the European Research Council (ERC) Synergy project HELMHOLTZ. He published over 300 peer-reviewed articles and co-authored more than 20 patents. He sits on several editorial boards, including Science Translational Medicine and The Journal of Clinical Investigation. He has been the recipient of numerous awards, including Foundation Fighting Blindness Trustee Award, Alcon Research Institute Award for Excellence in Vision Research, Grand Prix NRJ-Neurosciences-Institut de France, CNRS Medal of Innovation, Jules Gonin lecture, the Prize of the of the Retina Research Foundation...

José Sahel was elected to the European Academy of Ophthalmology (2006), the Academia Ophthalmologia Internationalis (2007), the Academy of Sciences-Institut de France (2007) and the German National Academy of Sciences Leopoldina (2014). He is Honorary Member of the Deutsche Ophthalmologische Gesellschaft, Knight of Ordre National de la Légion d'Honneur and Honoris Causa Doctorate of the University of Geneva. He was a founder and chief scientific advisor of Fovea, which became the ophthalmological division of Sanofi-Aventis, and is a scientific co-founder of GenSight Biologics and Pixium Vision.

Annick SCHWEBIG

Présidente d'Actelion France et du Comité Biotechnologies du Leem

Annick Schwebig joined Actelion in 2000. Her primary task, as General Manager of Actelion Pharmaceuticals France, was to set up and develop the French affiliate. Prior to that, Annick Schwebig held various positions of increasing importance within Bristol-Myers Squibb where she was Vice-President for Research & Development in Europe from 1993 to 2000. Her other responsibilities include:

- Within LEEM (Les Entreprises du Médicament) :
  - Administrator
  - Biotech Committee President
  - ARIIS Secretary (Alliance for Research and Innovation in Health Industries)
- Vice-President of the non-governmental organization "Equilibres & Populations"
- Member of the Paris Ile de France Chamber of Commerce and Industry
- Member of experts committee « funds large venture » within BPI France (a Public Investment Bank)
- Cellectis Administrator
- Board of Trustees Member of the ESSEC Business School
- Vice-President of the Inserm strategic council

Rafaèle TORDJMAN

Managing Partner. Sofinnova Partners

Rafaèle Tordjman, MD, PhD, is a Managing Partner. She joined Sofinnova Partners in 2001. Prior to this, she worked as a research scientist at the Institut National de la Recherche Médicale (INSERM) in Cochin Hospital, Paris. Before joining INSERM, she was a medical doctor specializing in clinical haematology and internal medicine. She obtained her PhD, with high honours, in haematopoiesis and angiogenesis from the University Paris VII followed by a post doctoral fellowship in immunology.

Rafaèle obtained her medical degree and her specialisation in Haematology and Internal Medicine as a five-year fellow in Paris University Hospitals. She also participated in the "Young Manager Program" at INSEAD (France, in 2002). She has invested in and is on the board of Ascendis, DBV technologies – which went public on Euronext Paris, Flexion Therapeutics – which went public on Euronext ParisNasdaq, Nucana Biomed, MedDay and ObsEva. She was also on the board of Corevalve, Endoart before it was successfully sold to Allergan Inc, of Preglem before the latter was successfully sold to Gedeon Richter and of HBI Ltd before being acquired by Meda.

Les sociétés de biotechnologies

Biotech companies

Company name: **AAVLIFE**
CEO: **Amber SALZMAN**
Phone: **+33 (0)6 79 53 24 46/+1 610 659 1098**
Mail: **amber@aavlife.com**
Company location: **Paris, France**
Area of expertise: **Gene therapy**

Company description

AAVlife is developing a gene therapy approach (AAVrh10 vector) for the treatment of the cadiomyopathy associated to Friedreich Ataxia. The program is currently completing large animal studies with the objective to start the clinical development mid-2016 and to obtain the human POC at the end of 2017.

Know-how and technological competences

AAVlife benefits of the expertise and the know-how of its scientific founders. Especially from Dr Hélène Puccio who developed a transgenic mouse model able to mimic the cardiomyopathy associated to Friedreich Ataxia. Thanks to this animal model, we were able to demonstrate the efficacy of our gene therapy approach. These POC were published in Nature Medicine in May 2014.

Ongoing partnerships

AAVlife collaborates with IGBMC and the research team of Dr Hélène Puccio for the preclinical development of our approach.
AAVlife also collaborates with Cornell University and Pr Ron Crystal (scientific founder) for the development of the vector and the optimization of our approach.
AAVlife signed a license with RegenX for Development of Treatments for Friedreich's Ataxia Using NAV® Vectors.
AAVLife signed a license with Inserm to gain exclusive access to the IP for treating Friedreich's Ataxia cardioyopathy with AAV vectors.

Further partnerships sought

AAVlife is looking for a partner for the codevelopment of the gene therapy product for the treatment of the cardiomyopathy associated to Friedreich Ataxia.

Company name: **Advicenne**
CEO: **Luc-André GRANIER**
Phone: **+33 (0)6 19 20 29 50**
Mail: **lag@advicenne.com**
Company location: **Nîmes, France**
Area of expertise: **Neuropaediatry and Nephropaediatry**

Company description

Advicenne is a pharmaceutical company that develops and commercialises drugs for patients with neurological disorders, such as epilepsy, and renal orphan diseases. The company is launching products in France and will also commercialise some in nearby countries.
Our products and treatments are safe, effective and adapted for paediatric use, with a focus on correct dosage and ease of administration.

Know-how and technological competences

Advicenne offers authentic therapeutic alternatives; leveraging input from neuropaediatricians and nephro- paediatrician who help the company in identifying the major unmet medical needs in the 2 therapeutic areas. Our drugs are designed to take into account the specific need of children. We develop small-size and tasteless forms for paediatrics, offering doses adapted to each child's weight. The company is formed of a group of highly qualified persons with all expertise skills ranging from research to commercialisation

Ongoing partnerships

We are actively working with a dozen EU reference centres and key opinion leaders for the orphan and paediatric conditions whose unmet needs are being investigated and resolved with our innovative and flexible bottom-up medical approach – from clinical need to medical solution. We are the SME partner and coordinator of several national and European collaborative research programs. We work closely already with numerous partners that are implicated in all phases of drug development.

Further partnerships sought

The company has already raised a total of close to 20 million euros, half from the public sector and the other half from private sector. We are looking for strategic partnerships on two distinct areas :
- Co-development opportunities in order to speed up our mid-stage pipeline opportunities, preferably partners having expertise in pharmaceutical development of innovative drug formulations.
- Pharmaceutical partner in order to secure the market access and launch our late stage pipeline drug candidates.

Company name: **Alizé Pharma**
CEO: **Thierry ABRIBAT**
Phone: **+33 (0)4 72 18 94 28**
Mail: **tabribat@alz-pharma.com**
Company location: **Lyon, France**
Area of expertise: **Metabolic Diseases and Rare Diseases**

Company description

Alizé Pharma is a group of companies specialized in the development of innovative biopharmaceutical drugs, proteins and peptides for the treatment of metabolic diseases and rare diseases. Since its creation in 2007 the group has raised 15.1 M€ from private and institutional investors and has acquired and implemented 3 programs. As of 2015, 2 programs are at the clinical stage and a first industrial partnership has been signed.

Know-how and technological competences

Our core know-how is translational medicine and early clinical development. The Alizé Pharma companies are managed and operated by a team of drug development experts and a board of directors with a breadth of international experience. Our business strategy is to acquire early-stage programs, advance them to the clinical stage and partner them with the pharmaceutical industry.

Ongoing partnerships

Our pegcrisantaspase program (ASPAREC® / JZP416, a new pegylated recombinant L-asparaginase) is partnered with Jazz Pharmaceuticals (Nasdaq: JAZZ) and is in Phase II/III clinical development for the treatment of acute lymphoblastic leukemia. Other collaborations (in-licenses, research collaborations) are ongoing with Erasmus Medical Center (Dr AJ van der Lely), University of Turin (Dr Ezio Ghigo), University of North Carolina at Chapel Hill (Dr David Clemmons).

Further partnerships sought

Alizé Pharma SAS is open for partnership discussions on its AZP-531 product, a stabilized analog of unacylated ghrelin, a physiological gastrointestinal peptide. This program is in Phase II clinical development for the treatment of Prader-Willi syndrome and in Phase Ib for type 2 diabetes.
Enclosed please find more information on the AZP-531 program with a particular focus on the Prader-Willi syndrome indication.

Company name: **Anagenesis Biotechnologies**
CEO: **Jean-Yves BONNEFOY**
Phone: **+33 (0)6 20 88 50 92**
Mail : **jybonnefoy@anagenesis-biotech.com**
Company location: **Strasbourg, France**
Area of expertise: **Healthcare**

Company description

Anagenesis Biotechnologies is a preclinical-stage stem cell-based company focused on developing novel treatments for genetic and chronic muscle degenerative diseases with unmet medical needs. Our goal is to help create a world where everyone can move, every single day.

Know-how and technological competences

Anagenesis Biotechnologies owns the rights to a unique proprietary technology, which is the result of 15 years of research in the laboratory of Pr Olivier Pourquié, a world expert in the field of musculo-skeletal development and stem cells. This technology allows to produce, on a industrial scale, illimited amount of muscular cells from stem cells. It constitues a unique tool for biomedical research and it gives rise to hopes of seeing a whole new class of treatments emerge for muscular diseases, including Duchenne muscular dystrophy (DMD). These discoveries have been recognized as one of the 25 major discoveries of the 20th century in Developmental Biology, by the scientific magazine Nature.

Ongoing partnerships

Anagenesis Biotechnologies has been supported by AFM Téléthon since the beginning of the project : it is a shareholder of the company and has awarded more than 4M€ over the past 6 years in grants to the company and the public research lab from which the technology originated. Anagenesis initiated a collaborative project with Q-States Biosciences. Together, the two companies have developed a project based on a new high throughput screening test to identify treatment candidates. The project has been awarded by MLSC and won the 1st prize of the 2014 Ubistart contest (UbiFrance, International Galien Foundation). Our company also has supports of several local funders and partners such as BPI France, Région Alsace, the competitive cluster Alsace BioValley and the startups incubator SEMIA.

Further partnerships sought

The aim of Anagenesis Biotechnologies is to market within 7 years a treatment for DMD patients and achieve partnerships with others biotechs or pharmaceutical groups, at the end of phases I or II of clinical trials (clinical proof of concept). The company is currently looking to raise 5M€ to 10M€ to support the preclinical development of its two complementary programs (cell therapy and small molecules) for Duchenne Muscular Dystrophy, in order to enter clinical trials in 2018.

Company name: **GENETHON**
CEO: **Frederic REVAH**
Phone: **+33 (0)1 69 47 12 85**
Mail: **frevah@genethon.fr**
Company location: **Evry, France**
Area of expertise: **Gene therapy**

Company description

Généthon, created by the AFM-Telethon, has the mission to make innovative gene therapy treatments available to patients affected with rare genetic diseases. Having played a pioneering role in deciphering the human genome, Généthon is today, with more than 200 scientists, physicians, engineers and regulatory affairs specialists, one of the leading organizations for the development of gene therapies. Généthon has also built one of the largest sites worldwide for GMP production of gene therapy products, Généthon Bioprod. In 2012, Généthon was awarded the prestigious Prix Galien for Pharmaceutical Research (France). The pipeline of Généthon includes products currently in international clinical trials and at preclinical stages, for immune deficiencies, muscular dystrophies, ocular and liver diseases. These products are developed either with Généthon as sponsor, or in partnership with private companies and academic institutions.

Know-how and technological competences

Genethon is an integrated R&D center ensuring translational development from research up to clinical validation, including GMP biomanufacturing.
Genethon develops highly innovative biotherapies for rare and ultra-rare diseases. The technologies, methods and tools, including the unique expertise developed in the fields of Bioprocess, Bioproduction, Regulatory can also open avenues for the treatment of frequent genetic and non-genetic disorders.

Ongoing partnerships

Genethon has ongoing collaborations with a network of more than 50 partners either academic, clinical or industrial, with the objective of accelerating the development of its products, or supporting the development of the products sponsored by its partners. In addition Genethon contributes or leads several French and EU R&D consortiums including the “ADNA program” (strategic industrial collaboration partly funded by bpifrance and coordinated by the Mérieux Alliance) designed to advance development of personalized medicine and the PGT Consortium (financed in the frame of “Investissements d’Avenir”), which develops pre-industrial scale bioproduction processes.

Further partnerships sought

Genethon is opened to strategic partnerships with pharma/biotech companies for co development of gene therapy products for rare diseases from research to commercialization, as well as to collaborations on technology development, or GMP production of gene therapy vectors.

Company name: **HEMARINA SA**
CEO: **Franck ZAL**
Phone: **+33 (0)2 98 88 04 86**
Mail: **franck.zal@hemarina.com**
Company location: **Morlaix, France**
Area of expertise: **Biotechnology**

Company description

Hemarina SA is a privately held biotechnology company founded in Morlaix (Finistère) developing innovative solutions for therapeutic and industrial applications, based on extracellular hemoglobins issued from marine annelids and working as oxygen carriersHEMARINA now employs 35 people in 3 sites in France and the USA (Morlaix, Noirmoutier and Boston) and spends about 90 % of its workforce in its effort in Research and Development.

Know-how and technological competences

- HEMO2Life®: a breakthrough innovation for organ preservation. This oxygen carrier ameliorates organ preservation by carrying oxygen to the transplant organ,
- HEMOXYcarrier®: an universal therapeutic oxygen carrier that allows oxygenation recovery without the adverse side effects (primarily vasoconstriction) observed with first-generation hemoglobin-based-oxygen carriers,
- HEMOXCell®: a unique product for cell culture and recombinant protein production. This product not only improves cell growth and viability but also increases bioproduction yields,
- HEMO2Ling®: an oxygenating dressing. It is a therapeutic solution for hypoxic and chronic

Ongoing partnerships

As regards to HEMO2Life® development, the major collaborations engaged are :
- Inserm U1082, Poitiers, France: - Thuillier et al. American Journal of Transplantation, 2011
- Mallet et al., Artif Organs., 2014
- Department of Bioengineering, San Diego, USA: Tsai et al., Current Drug Discovery Technologies, 2012
- Inserm U1078, Brest, France: Le Gall et al., Journal of Biotechnology, 2014
- Clinic of Surgery, Bonn, Germany & University Medical Center, Groningen, Netherlands – COPE Consortium

Further partnerships sought

The technology developed by HEMARINA SA offers solutions to treat pathologies related to a lack in oxygen. The present industrial partnership call would be for HEMARINA SA an opportunity to engage discussions with larger industrial partners about the opening of its technology to the treatment of rare pathologies. Then, these potential new alliances would bring visibility and maturity to HEMARINA SA.

HORAMA
THERAPIES FOR RETINOPATHIES

Company name: **HORAMA SAS**
CEO: **Denis CAYET**
Phone: **+33 (0)9 53 73 68 15**
Mail: **d.cayet@horama.fr**
Company location: **Main: Paris - Secondary: Nantes, France**
Area of expertise: **Gene Therapy / Ophthalmology**

Company description

HORAMA is a biotechnology company aimed at the development of gene therapy medicinal products for rare, degenerative inherited retinal ophthalmologic pathologies. From a demonstrated preclinical proof-of-concept, HORAMA will ensure the development of its portfolio's recombinant adeno-associated viral vectors (rAAV) through the regulatory pharmaceutical, preclinical and clinical steps to bring therapeutic solutions to the patients.

Know-how and technological competences

Thanks to its scientific team (see "collaborations" below), employees and strong relationships with experienced subcontractors, HORAMA masters all the steps for the pharmaceutical (including GMP production), preclinical (including GLP studies) and clinical development of rAAV for use in ophthalmological pathologies. HORAMA's team has already proven successful in conducting a rAAV phase 1/2 clinical trial in Leber congenital amaurosis.

Ongoing partnerships

- In Nantes : INSERM UMR1089 in Atlantic Gene Therapy, « gene therapy for retinal and neuromuscular diseases », directed by Dr Philippe Moullier, including its Process Development group; Nantes University Hospital ; EFS-ABG (GMP facility)
- In Montpellier : INSERM U 1051 in Institute for Neurosciences of Montpellier, «Genetics and therapy of retinal blindness and optic neuropathies» directed by Prof. Christian Hamel ; Montpellier University Hospital

Further partnerships sought

HORAMA is willing to collaborate on product developments : we seek partners for preclinical and clinical development steps.
HORAMA is also looking for funding opportunities end 2015.

InFlectis BioScience
Innovative therapeutics to treat protein misfolding diseases

Company name: **InFlectis BioScience**
CEO: **Pierre MINIOU**
Phone: **+33 (0)6 07 08 60 98**
Mail: **pierreminiou@inflectisbioscience.com**
Company location: **Nantes, France**
Area of expertise: **Pharma Industry**

Company description

InFlectis BioScience is a France-based drug discovery company whose goal is to discover and develop drugs to treat degenerative diseases having their etiology in the accumulation of misfolded proteins.

Know-how and technological competences

- IFB-088 FIRST-IN-CLASS drug candidate targeting PPP1R15A phosphatase regulatory subunit to treat orphan neurodegenerative diseases having their etiology in misfolded protein accumulation;
- Second generation of New Medical Entities targeting PPP1R15A phosphatase regulatory subunit to treat non-orphan diseases having their etiology in misfolded protein accumulation;
- Proprietary cell-based HTS technology to identify NCEs able to modulate protein conformation

Ongoing partnerships

MRC (UK): Exclusive license to exploit MRC's property rights on patent PCT/EP2014/050422.
Valneva (FR): Exclusive worldwide license to exploit Valneva's property rights on patents covering the 3D-Screen technology.

Several research and service agreements with CROs to perform in cellulo and in vivo evaluation of proprietary PPP1R15A inhibitors.

Further partnerships sought

The company strategy is to out-license clinical-stage drug development program to large pharmaceutical companies able to run late stage clinical development, manufacturing, marketing & sales.
Due to the many diseases having their etiology in protein misfolding, InFlectis BioScience is open to consider earlier R&D partnerships for IFB-088 series and other chemical families.

Company name: **: LYSOGENE**
CEO: **Karen AIACH**
Phone: **+33 (0)1 41 43 03 90**
Mail: **karen.aiach@lysogene.com**
Company location: **Neuilly-sur-Seine, France**
Area of expertise: **Gene therapy**

Company description

Lysogene is a clinical stage biotechnology company. It develops gene therapy products aiming at treating orphan diseases with CNS involvement and high unmet medical needs. Its current pipeline includes LYS-SAF301 for Sanfilippo Syndrome A (MPSIIIA) and LYS-GM101 for GM1-gangliosidosis. LYSOGENE retains global rights for both products. Lysogene was created in 2009 by Karen Aiach in response to her daughter's being diagnosed and Olivier Danos, a worldwide expert in gene therapy.

Know-how and technological competences

The company has gained unique skills in the following fields:
- Translational and clinical research in brain gene therapy
- Preclinical studies using adeno-associated virus (AAV)-based technology
- Clinical trial in a pediatric neurodegenerative lysosomal storage disorder
- Orphan / Pediatric / ATMP (advanced therapeutic medicinal products) regulatory pathways

Ongoing partnerships

The company has several collaborators and partners, amongst which:
- Assistance Publique – Hôpitaux de Paris (France)
- Inserm (France)
- University College of London (UK)
- University of Manchester (UK)
- University of Massachusetts Medical School & Auburn University (US)

Further partnerships sought

The company will look at opportunities supporting its efforts towards:
- The development of its portfolio via the acquisition or in-licensing of gene therapy assets targeting diseases with CNS / brain involvement and/or involving new gene delivery technologies
- The development of a sustainable, scalable and commercial-stage vector manufacturing platform.

Company name: **METAFORA biosystems**
CEO: **Vincent PETIT**
Phone: **+33 (0)1 60 87 89 25**
Mail: **vincent.petit@metafora-biosystems.com**
Company location: **Evry, France**
Area of expertise: **Diagnostics**

Company description

METAFORA biosystems is a French biotech company, which turns a disruptive technology into innovative assays that allow "reading" the metabolic status of cells.
In less than three years the company has commercialized products for the Lifesciences market and has set up a worldwide distribution network. Leveraging its experience in metabolic biomarkers, METAFORA's next growth lever is to achieve rapid commercialization of new products for the In-Vitro Diagnostics (IVD) market. Its versatile platform based on blood cells analysis, named METAblood® SCAN, allows for the early diagnosis of rare diseases, and promises to be a powerful technology for the monitoring of chronic illnesses. Its two first tests under development are a screening test for the Glut1 Deficiency Syndrome by 2016 (an orphan metabolic disease), and a disruptive blood test for immune response monitoring by 2019. Both tests will be companion diagnostics to first detect, then treat patients (rare disease), and adapt therapies to each patient.
The scientific and industrial vision is to develop the company into a company focused on personalized, predictive and preventive medicine through metabolic monitoring of blood cells.

Know-how and technological competences

METAFORA's motto is: "Fuel feeds function, nutrient transporters fuel cell metabolism". Its technology is based on quantitative profiling of nutrient transporters at the cell surface using proprietary ligands, combined to dedicated algorithms. The overall technology detects metabolic reprogramming undergone by cells when they adapt their metabolism. A multidiagnostics platform, named METAblood® SCAN, is currently under development, which will generate a first screening test for an orphan metabolic disease by 2016, and a disruptive blood test for immune response monitoring by 2019.

Ongoing partnerships

To develop the METAblood SCAN® technology for diagnostics use, METAFORA has built a strong ecosystem of leading partners and clinicians working on both rare and mass diseases. Research agreements are setup with the CNRS, INSERM, AP-HP (la Pitié-Salpêtrière and Robert Debré hospitals), IMAGINE institute and PASTEUR Institute, notably.

Further partnerships sought

Other metabolic diseases resulting from genetic mutations might also be addressed by the technology to achieve early diagnoses, and allow for an early, adequate management of the disease. To name a few: hypophosphatemia, Fahr's disease etc. On the other hand, mass diseases, especially chronic diseases in which inflammation is part of the pathophysiology, or cancers, can benefit from METAFORA's technology. A multidiagnostics platform is currently under development.
Before its full validation for diagnostic use, the technology is available for translational research and clinical trials of drug candidates. METAFORA is open to partnerships with Pharmas and Biotechs which could be interested in applying METAblood SCAN® to their developments, and to co-develop companion diagnostics.
On the other hand, the company is also looking for CRO partnerings to work on industrial deployment of the technology.

Metabolism is a wide, but underexploited field opening great avenues for many diseases diagnostics and monitoring, and should participate to a great extent to the development of tomorrow's medicine.

Company name: **NEURADIS**
CSO: **Anselme PERRIER**
Phone: **+33 (0)6 81 04 03 20**
Mail : **aperrier@istem.fr**
Area of expertise: **Neurobiology**

Company description

Neuradis is a Biopharma aimed at discovering and developing new chemical entities (NCE) for Huntington's disease (HD) and neurodegenerative diseases.

Know-how and technological competences

The company is a spin-off of of I-Stem, the Institute for Stem cell Therapy and Exploration of Monogenic diseases. Neuradis expertise and assets include 1) "disease in a dish" models based on human pluripotent Stem Cells (hPSCs) 2) a technology platform to identify new drugs. 3) three chemical series of molecules for Huntington's disease. The company has the expertise to develop these innovative molecules for therapy of HD up to a phase I/II proof of concept clinical trial.

Ongoing partnerships

Neuradis is currently exploring the possibility of establishing external collaborations. As I-Stem is internationally recognized as a leader in pluripotent stem cells research and development on several rare diseases. I-Stem has multiple partners either from the academic, pharmaceutical, or biotech worlds. Neuradis will benefit from this unique network to establish collaborations thanks to the AFM patients and clinicians communities.

Further partnerships sought

Company is looking for partnership with large pharmaceutical or biotech companies for collaborative development of its new molecules in the neurodegenerative diseases field.

Company name: **Neurochlore**
CEO: **Yehezkel BEN ARI**
Phone: **+33 (0)6 20 66 80 00**
Mail: **yehezkel.ben.ari@inserm.fr**
Company location: **Marseille, France**
Area of expertise: **Neurobiology – drug development – repositioning**

Company description

Neurochlore is a biopharmaceutical company founded by Prof Ben-ARI in 2011 and focused on the development of a off patent drug, Bumetanide, in autism spectrum disorder (ASD) and Fragile X syndrome. The company has started a phase II dose ranging study in France recruiting 90 children and adolescents with autism spectrum disorder (ASD). The development plan has been validated by EMA through a pediatric investigation plan (PIP) procedure in March 2013.
According to recent epidemiological surveys, about 1 in 100 children has been identified with ASD. ASDs are reported to occur in all racial, ethnic and socioeconomic groups. Autistic children have difficulties with social interaction, displaying problems with verbal and nonverbal communication, exhibiting repetitive behaviors and having narrow, obsessive interests. no fda or EMA approved drugs for autism. The global ASD market is expected to grow because of the high increase in prevalence and the rapid increase in public awareness about the conditions. There is a high unmet need which is largely driven by the availability of only a few approved drugs like Abilify and Risperidone (atypical antipsychotics) that are moderately successful in terms of safety and efficacy profile and which do not address the core symptom of the disease.
Bumetanide has the potential to improve social engagement, which represents a new paradigm in the treatment of ASD and fragile X syndrome. The Neurochlore team is truly excited about the prospect of helping patients and their families achieve an improved quality of life.

Know-how and technological competences

Neurochlore R&D activities are managed by a highly focused and competent team.
The research activities are managed by Prof Ben-Ari in Marseille. The team has shown that bumetanide blocks the NKCC1 cation-chloride co-transporter, and thus decreases internal chloride concentration in neurons. In turn, this concentration change makes the action of GABA more hyperpolarizing (inhibitory), which may be beneficial for treatment of ASD. The thematics on Neurochlore focus on brain developmental sequences including the excitatory /inhibitory shift of GABA actions. Three post-doctoral researchers and one technician are involved in vivo, in vitro physiology, behavior and motor activities measurements in relevant animal models.
Clinical activities are managed by D. Ravel and subcontracted to experienced service providers. Neurologists and child psychiatrists reinforce the team.
Neurochlore has developed two new formulations adapted to children and adolescents.
The European Patent Office has recently granted a patent for the use of Bumetanide and other inhibitors of chloride influx in Gabaergic neurones to treat autism.

Ongoing partnerships

Collaboration with Hilgo Bruining MD, PhD, department of Psychiatry, Brain Center Rudolf Magnus Utrecht: Paradoxical benzodiazepine response : a rationale for bumetanide in neurodevelopmental disorder. A phase II clinical study with Bumetanide in 90 children with ASD will be undertaken in 2015.

Further partnerships sought

Investors to finance:
- A phase pivotal III study in autistic children and adolescents (120 patients) . The design of the study is validated by European health authorities (2016-2018)
- A phase IIB study in 60 fragile X syndrome patients (2016-2017)

Company name: **NEURONAX SAS**
CEO: **Stéphane GOBRON**
Phone: **+33 (4)73 33 90 24 / +33 (6) 62 47 51 39**
Mail : **sgobron@neuronax.com**
Company location: **Clermont-Ferrand, France**
Area of expertise: **Nervous System Diseases**

Company description

NEURONAX is a biotech company focusing on the development of a family of neuroprotective and neuroregenerative candidate-drugs in order to treat damage of the nervous system and to contribute to improve neurological function, and then quality of life of patients. Moreover, our technological platform based on various cell-based assays allows the enlargement of the proprietary portfolio and is also proposed outside in the form of contract of service or contract of research.

Know-how and technological competences

Our unique, proprietary and patented peptidic compounds and derivatives have shown vitro and vivo a multifaceted activity protecting nervous cells (neurons and oligodendrocytes) especially from oxidative stress and apoptosis, as well as promoting cell extension. They are first-in class candidates which may address various unmet medical needs, including but not limited to SCI, TBI, stroke, multiple sclerosis, Lou Gehrig disease... Firstly focused on spinal cord injuries, our lead candidate, NX210, has been granted Orphan Drug Designation by the EMA for the treatment of acute SCI and an IND application is in progress. Phase I/II clinical trial in this indication is expected to this year.

Ongoing partnerships

NEURONAX is exploring the potential of its proprietary technology in collaborations with leading scientists:
- IGCNC – EA 7282 / UMR CNRS 6284 ISIT (Clermont-Ferrand)
- EA 3842 - Homéostasie Cellulaire et Pathologies (Limoges)

Further partnerships sought

Various experimental results obtained with our family of compounds argue for various indications such as stroke, Alzheimer's, Parkinson's and Lou Gehrig diseases, as well as myelin disorders, hearing or vision loss. In order to explore these potential applications, NEURONAX is continuously expending its network of collaborations and is seeking partnerships to further develop its technologies or its applications, and to accelerate clinical development.

BIOTECHS

Company name: **ProGeLife**
CEO: **Christophe HUBERT**
Phone: **+33 (0)6 27 28 44 44**
Mail : **c.hubert@progelife.com**
Company location: **Marseille, France**
Area of expertise: **Life Sciences**

Company description

Founded in June 2014, ProGeLife's ambition is to develop patented innovative therapeutic solutions to treat progeroid rare diseases where progerin is involved.
Pierre Cau (partners of ProGeLife), in genetics and cell biology. Well-known specialists of genetic progeroid rare diseases, they discovered the mutation responsible for progeria that leads to progerin, a protein produced by everybody in the course of physiological aging and now set as one of the main biomarkers of aging.

Know-how and technological competences

ProGeLife has demonstrated in-vitro and in-vivo (animal) proofs of concept for two patented and innovative families of products with orphan designations:
- An NADPH oxidase 1 peptide inhibitor for the treatment Xeroderma pigmentosum Type C (XPC)
- A combination of aminobiphosphonate and statin in Progeria, with a Phase II clinical trial (2008-2013) A third family of innovative compounds, proteasome inhibitors, with additional opportunities and applications, has been patented in January 2015.

Ongoing partnerships

ProGeLife has collaborations with INSERM unit UMR_S910 (Marseille) for rare diseases of aging, INSERM unit UMR_S1035 (Bordeaux) for a rare DNA repair disease (Xeroderma pigmentosum) and with the Universities of Aix-Marseille and of Bordeaux to exploit patents through licensing contracts.
ProGeLife is currently in discussions with financial investors to secure its development.

Further partnerships sought

ProGeLife's goal is to partner to bring these innovations to the patients. ProGeLife is looking for partnership with pharmaceutical companies for the co-development (Phases II) and the commercialization in the main markets of its therapeutic innovations in selected rare diseases:
- Progeria and related progeroid disorders, rare diseases of aging
- DNA repair diseases such as Xeroderma pigmentosum Type C (XPC)
- RASopathies, rare diseases caused by mutations in genes encoding RAS proteins especially Costello and Noonan syndromes

Company name: **SENSORION SA (Alternext Paris : ALSEN)**
CEO: **Laurent NGUYEN**
Phone: **+33 (0)4 67 20 77 30**
Mail : **laurent.nguyen@sensorion-pharma.com**
Company location: **Montpellier, France**
Area of expertise: **Life sciences**

Company description

Biotech specialised in the treatment of pathologies of the inner ear such as acute vertigo, tinnitus and hearing loss (general population-adult & pediatric, orphan diseases).
Backed by its pharmaceutical R&D experience and a comprehensive technology platform, Sensorion is developing three small molecule drug candidates for treating the symptoms of vertigo or tinnitus, for preventing complications associated with progressive lesions in the inner ear and for preventing the toxicity of chemotherapy in the inner ear.

Know-how and technological competences

- Spun off from Inserm (the French institute of health and medical research) in 2009, Sensorion has matured since into a biotech operating under pharma standards.
- Strong scientific and academic background in inner ear disorders
- Comprehensive technology platform dedicated to inner ear disorders
- know-how in research, clinical development & regulatory affairs, business development & licensing/acquisition, marketing & sales

Ongoing partnerships

- Licensing agreement with Palau Pharma (Spain) for developping a first-in-class H4R antagonist in inner ear disorders
- Collaborative project with Griffin Discoveries (NL) to develop new neuromodulators for the treatment of inner ear disorders under a Eureka/Eurostar grant

Further partnerships sought

Opportunités recherchées (5 lignes maximum)
- collaboration with pharmas or biotechs to develop new drug treatment for inner ear disorders using Sensorion's platform and know-how
- licensing-in, build-up and acquisition of assets to strengthen our product pipeline and/or technology platform
- join collaborative project in line with our business strategy to fuel non-dilutive funding efforts
- mid-term: licensing-out opportunities

BIOTECHS

Company name: **TherAchon**
CEO: **Gianni GROMO**
Phone: **+33 (0)6 29 65 28 46**
Mail : **gianni@versantventures.com**
Company location: **Nice, France**
Area of expertise: **Achondroplasia**

Company description

TherAchon develops a novel therapeutic approach for the treatment of achondroplasia, a pediatric rare disease affecting bone growth. Achondroplasia is the most frequent genetic form of dwarfism and is linked to a mutation in the FGFR3 receptor (G380R mutation). The therapeutic strategy is based on the use of soluble protein decoys restoring normal bone growth. The company was created in May 2014 by Elvire Gouze and financed in July 2014 by Versant Ventures and Inserm Transfert Initiative.

Know-how and technological competences

TherAchon benefits of the expertise and the know-how of its scientific founder, Elvire Gouze, in the field of achondroplasia. Dr Elvire Gouze demonstrated the in-vivo efficacy of a soluble FGFR3 in the treatment of achondroplasia in a highly relevant animal model (Fgfr3(ach/+) mice). These results were published in Sept-13 in Science Translational Medicine. TherAchon has a recombinant protein production platform as well as a purification facility, including an FPLC. The team screens decoys based on ligand binding, in vitro and in vivo efficacy. Technologies also include screening on cell lines or primary cells, histology and anatomopathology platform, skeleton analyses and bone radiography.

Ongoing partnerships

TherAchon collaborates with the team of Dr Elvire Gouze at Inserm for the lead selection process ongoing with the company. The objective is to select the lead at the end of 2015.
We have also signed an exclusive and worldwide license with Inserm Transfert covering soluble fibroblast growth factor receptor 3 (FGFR3) polypeptide for use in the prevention or treatment of skeletal growth retardation disorders.

Further partnerships sought

TherAchon is looking for partners for the co-development of this novel protein approach for the treatment of achondroplasia. These include, but are not limited to, the development of more sensitive titer assays, CMC, …

Les entreprises du medicament

Pharmaceutical companies

Company name: **Actelion Pharmaceuticals Ltd**
Company size: **2,500 employees**
Number of countries: **31 operative affiliates**
Annual sales 2014: **CHF 1.95 Billion**
R&D Staff: **~1,000**
R&D investment: **19% of product sales**

COMPANY DESCRIPTION

Actelion Ltd. is a leading biopharmaceutical company focused on the discovery, development and commercialization of innovative drugs for diseases with significant unmet medical needs.

Founded in late 1997, with now over 2,400 dedicated professionals covering all key markets around the world including Europe, the US, Japan, China, Russia and Mexico, Actelion has its corporate headquarters in Allschwil / Basel, Switzerland.

HISTORY OF COMMITMENT TO RARE DISEASES

We are leaders in the science and medicine of pulmonary arterial hypertension (PAH), with over 15 years of experience. Our understanding of the complex pathways and molecular mechanisms of this disease has enabled the development of tailored medicines that can make a real difference to patient outcomes.

We are set to continue our leadership in the field of PAH into the mid-2020s, thanks to our assets Veletri®, Opsumit®, and potentially selexipag, currently under review with Health Authorities, together with our partners Nippon Shinyaku.

Our expertise in human biology, especially our knowledge of specific families of molecular targets, such as G-Protein Coupled Receptors, led us to Opsumit and it is this knowledge that will take us to new areas.

PRODUCT PORTFOLIO IN THE AREA

Actelion is a leader in the field of pulmonary arterial hypertension (PAH). Our portfolio of PAH treatments covers the spectrum of disease, from WHO Functional Class (FC) II through to FC IV, with oral, inhaled and intravenous medications. Although not available in all countries, Actelion has treatments approved by health authorities for a number of specialist diseases including Type 1 Gaucher disease, Niemann-Pick type C disease, Digital Ulcers in patients suffering from systemic sclerosis, and mycosis fungoides type cutaneous T-cell lymphoma.

PROSPECTIVE IN THE AREA : THEMATIC PRIORITIES

Our strategy for long-term value creation, set out in 2012, is being delivered around three key objectives – sustaining and growing our PAH franchise, building additional specialty franchises, and optimizing profitability. In the coming years we will diversify portfolio – through R&D and/or business development, to find additional meaningfully differentiated products to benefit patients.

Martine CLOZEL
M.D.

Clinical Education
- "Internat des CHU" (residency): Major of the competitive examination, Nancy, 1978
- Recipient of the award honor "Lauréat de l'Université de Nancy" for 5 consecutive years.
- M.D. Thesis: Nancy University, France, 1980 Topic: Pharmacology of Methotrexate in Pediatrics. With honors
- Masters of Pharmacology, 1980
- Specialty in Pediatrics, 1983
- Assistant Professor in Neonatal Medicine and Intensive Care, 1983-1984

Research Career
- Research Fellow: McGill University, Montreal, Canada; 1980-1982
- Price-winner of the Eli-Lilly Fellowship Program: UC San Francisco (Cardiovascular Research Institute); 1984-1985. Post-doctoral position
- Researcher, F. Hoffmann-La Roche Ltd; 1987-1997
- Scientific Expert, F. Hoffmann-La Roche Ltd; 1994-1997.
- Roche "Research and Development Prize"; 1997
- Co-founder Actelion, 1997
- Senior Vice President, Co-Head of Drug Discovery, Head of Pharmacology and Preclinical Development 1999.
- Chief Scientific Officer, 2009

Current position
Chief Scientific Officer, Co-Head of Drug Discovery.

Recent Honors
- Who's Who citations
- Chevalier dans l'Ordre de la Légion d'Honneur (2008)
- Prix Hermès de l'Innovation (2011)
- Member of the Editorial Board on Hypertension, Journal of Cardiovascular Pharmacology, Science Translational Medicine
- Reviewer on multiple journals including high-ranking journals (Am J Physiol, FEBS Letters, Nature, Circulation, Hypertension, etc…)

Company name: **AMGEN**
Company size: **2,500 employees**
Number of countries: **31 operative affiliates**
Annual sales 2014: **CHF 1.95 Billion**
R&D Staff: **~1,000**
R&D investment: **19% of product sales**
Headquarters: **Thousand Oaks (California, USA)**

COMPANY DESCRIPTION

Amgen is committed to unlocking the potential of biology for patients suffering from serious illnesses by discovering, developing, manufacturing and delivering innovative human therapeutics. This approach begins by using tools like advanced human genetics to unravel the complexities of disease and understand the fundamentals of human biology.
Amgen focuses on areas of high unmet medical need and leverages its biologics manufacturing expertise to strive for solutions that improve health outcomes and dramatically improve people's lives. A biotechnology pioneer since 1980, Amgen has grown to be one of the world's leading independent biotechnology companies, has reached millions of patients around the world and is developing a pipeline of medicines with breakaway potential.

HISTORY OF COMMITMENT TO RARE DISEASES

Amgen has a long history of commitment to rare diseases in different therapeutic areas.

Haematopoiesis : Idiopathic Thrombocytopenic Purpura (ITP)
ITP is a rare disease, an autoimmune disorder affecting an estimated 50,000 people in the EU, which can lead to serious bleeding events that can be potentially life threatening.
Amgen has discovered romiplostim (Nplate), a peptibody which has been commercialized since 2008. Nplate is a thrombopoietin (TPO) mimetic, an engineered therapeutic fusion protein with attributes of both peptides and antibodies, but is distinct from each. Romiplostim works similarly to TPO, a natural protein in the body. Romiplostim stimulates the TPO receptor, which is necessary for growth and maturation of bone marrow cells that produce platelets. Romiplostim is indicated for treating adult patients with chronic ITP.

Onco-Hematology (ALL)
Amgen, with Micromet acquisition, has developed the first of the BiTE® antibodies to treat a rare disease : Philadelphia-negative (Ph-) relapsed/refractory B-precursor acute lymphoblastic leukemia (ALL). In the U.S., it is estimated that more than 6,000 cases of ALL will be diagnosed in 2014, and in the European Union, more than 7,000 cases of ALL are diagnosed each year.

Blinatumomab (Blincyto) is an investigational BiTE® antibody designed to direct the body's cell-destroying T cells against target cells expressing CD19, a protein found on the surface of B-cell derived leukemias and lymphomas. Blinatumomab, has received both orphan drug designation and breakthrough therapy designation from the FDA for the treatment of adults with Philadelphia-negative (Ph-) relapsed/refractory B-precursor acute lymphoblastic leukemia (ALL), a rapidly progressing cancer of the blood and bone marrow. Blinatumomab is also being investigated for its potential to treat pediatric relapsed/refractory ALL, relapsed/refractory Philadelphia positive (Ph+) B-precursor ALL, minimal residual disease positive (MRD+) B-precursor ALL, relapsed/refractory non-Hodgkin's lymphoma (NHL), including relapsed/refractory diffuse large B-cell lymphoma (DLBCL).

Hyperlipidemia : Homozygous familial hypercholesterolemia
Amgen has recently developed a novel biologic agent against cholesterol: evolocumab (Repatha). Repatha is an investigational fully human monoclonal antibody that inhibits proprotein convertase subtilisin/kexin type 9 (PCSK9), a protein that reduces the liver's ability to remove low-density lipoprotein cholesterol (LDL-C), or "bad" cholesterol, from the blood. Committed to rare diseases, Amgen has done a specific study (Tesla trial) for the use of Repatha in homozygous familial hypercholesterolemia (HoFH). HoFH is caused by genetic defects inherited from both parents that affect the function of the low-density lipoprotein (LDL) receptor, the receptor responsible for removing LDL-C cholesterol (bad cholesterol) from the body. HoFH is characterized by extremely high levels of LDL cholesterol (LDL-C) that increase cardiovascular risk in these patients, many of whom are affected at an early age. The FDA advisory committee has recommended this indication in its review of Repatha on June 9, 2015.

PRODUCT PORTFOLIO IN THE AREA

Romiplostim (Nplate)
Blinatumomab (Blincyto)
Evolocumab (Repatha)

PROSPECTIVE IN THE AREA : THEMATIC PRIORITIES

Hematology / Oncology
Inflammation
Bone Health
Neuroscience
Cardiovascular
Metabolic disorders
Nephrology

Norbert PRENZEL
Director Business Development, Amgen.

Dr. Norbert Prenzel is a Director in Amgen's worldwide Business Development Organization. He is responsible for Amgen's business development interactions with life science investors, academic institutions, technology transfer organizations as well as with Biotech and Pharma companies based in Europe.

Major focus of Amgen's business development and licensing activities are related to the following therapeutic areas: Oncology, Bone diseases, CNS disorders, Inflammation, Nephrology, Metabolic and Cardiovascular disorders.

Norbert joined Amgen through the acquisition of Micromet Inc., where he acted as Director Business Development overseeing BD and Alliance Management activities.

Prior to joining Micromet, Norbert worked as BD and Licensing Consultant for various European Biotech companies. Prior to that and following his PhD thesis, which he did in Axel Ullrich's department at the Max-Planck Institute for Biochemistry, he co-founded the start-up company U3 Pharma AG, where he served as Program Director and Head of Business Development until the acquisition of U3 Pharma by Daiichi Sankyo in 2008.

Norbert was awarded the Junior Research Award of the Max Planck Society for his doctoral thesis on signal transduction and cancer. He also holds a MBA degree from WHU/Kellogg School of Management.

Company name: **ANTABIO SAS**
Company size: **10-15 employees**
Number of countries: **French company with UK subsidiary**
Annual sales 2014: **NA**
R&D Staff: **8 Ph.D.**
R&D investment: **€5 million/year**
Partnerships: **6 academic collaborations**

COMPANY DESCRIPTION

Antabio SAS is a privately-held biopharmaceutical company with a presence in France and the UK, dedicated to the discovery of first-in-class compounds for treating severe infections that are resistant to antibiotic treatment. Two of Antabio's discovery programmes have received Wellcome Trust Seeding Drug Discovery Awards to support, respectively, the development of [i] a novel, safe and efficacious inhibitor of bacterial metallo ß-lactamases to be combined with a carbapenem for the treatment of drug-resistant nosocomial infections and [ii] a first-in-class inhibitor of Pseudomonas biofilms to be co-administered with standard-of-care antibiotics for the long-term management of chronic respiratory infections.

In addition, Antabio has established an industrial discovery engine supported by Bpifrance (the French Public Investment bank), and dedicated to the discovery, evaluation and development of new combination and/or adjunctive antibiotic therapies.
www.antabio.com

HISTORY OF COMMITMENT TO RARE DISEASES

Antabio has built a fast-moving pipeline of candidate drugs targeting key resistance and virulence mechanisms used by pathogenic bacteria to cause life-threatening infections, such as those affecting Cystic Fibrosis (CF) sufferers.

In 2014, the company has been awarded the "Concours Mondial de l'Innovation 2030" Worldwide Innovation Challenge for its first discovery program PQSI, focused on the treatment of chronic infections in CF patients.

In 2015, the company received a €4.0m Wellcome Trust Seeding Drug Discovery Award to fund a second line of research PBI focused on the development of a small molecule inhibitor of PA biofilms to be used in combination with standard-of-care antibiotics for treatment of chronic infections in CF patients.

PRODUCT PORTFOLIO IN THE AREA

The company is developing two independent programmes PQSI and PBI, currently at the lead optimization stage, whereby small molecule compounds targeting clinically-relevant bacterial biofilm determinants will be combined with antibiotics for the treatment of chronic infections in CF patients.

PROSPECTIVE IN THE AREA : THEMATIC PRIORITIES

Antabio intends to bring its CF programmes to the IND-filing (PBI) and preclinical (PQSI) stages by 2018. The company envisages reaching proof of concept in man for its CF programmes before out-licensing to a suitable partner.

However, where there is a clear strategic and complementary fit, Antabio will consider earlier partnerships with academic and industrial organizations to accelerate the progression of its candidate drugs to the clinic.

Marc LEMONNIER
Président d'Antabio

Marc is a life sciences entrepreneur with a deep knowledge of pathogenic bacterial infections and their biology. A molecular microbiologist by training, Marc is the author of numerous peer-reviewed articles in the antibacterial field. As the founding CEO of the company, Marc structured the scientific, commercial and corporate development strategy of Antabio' business and led the successful financing strategy, attracting high single-digit million Euro funding since the company's foundation.

Marc has designed, coordinated and managed private-public and industrial partnerships at the national and international level first as an academic in the early days of his career and since then as part of Antabio's active business partnering strategy.

Company name: **BAYER**
Company size: **113 200 employees**
Number of countries: **283 companies and subsidiaries**
Annual sales 2014: **€ 40,2 milliards**
R&D Staff: **7 700**
R&D investment: **€ 3,2 mlliards**
Partnerships: **6 academic collaborations**
Haedquaters: **Berlin, Germany**

COMPANY DESCRIPTION

Bayer a choisi d'axer sa stratégie sur la contribution aux enjeux sociétaux majeurs mondiaux centrés sur les sciences de la vie. Bayer est un groupe mondial, siégeant en Allemagne.

Bayer France : 3 100 collaborateurs au 31. 12. 13.
Bayer CropScience France : Chiffre d'affaires BCS France en 2013 : 593 millions d'€.
Bayer HealthCare France : Chiffre d'affaires BHC France en 2013 : 739 millions d'€.

PRODUCT PORTFOLIO IN THE AREA

YAZ® / Jasmin® / Jasminelle® (Santé de la Femme)
Kogenate® (Médecine de Spécialité)
Nexavar®, Stivarga® (Médecine de Spécialité)
Betaferon® (Médecine de Spécialité)
Mirena® (Santé de la Femme)
Xarelto® (Médicine Générale)
Eylea® (Ophtalmologie)
Levitra (Médecine Générale)
Pritor® (Médecine Générale)

R&D FOCUS PORTFOLIO AND PROSPECTIVE IN THIS AREA

Budget de R&D 2013 :
3,2 Milliards €.

PLACES OF WORLDWIDE INVESTIGATIONS

Berlin / Wuppertal / Cologne / Monheim
Berkeley / San Francisco / Pékin

Eric GAUTHIER
Head of Specialties Medecine (Oncology; CTEPH, Hematology, Neurology, Radio & Imagery) and Medical Governance since 2007

MEMBERSHIPS
1996 – 1997
Treasurer of Association for Meta-Analysis in Cancerology (AMAC) chaired by Professor Pascal Piedbois, Créteil, France.

1995 – 2000
Member of Meta-Analysis Group In Cancer (MAGIC) chaired by Professor Norman Wolmark Pittsburgh, USA.

1996 – 2000
Treasurer (participation in the negotiation of the contracts) then Responsible for communication of AERO chaired by Professor Pascal Piedbois, Créteil, France.

Since 2004
Member of director board of the European Association of Research in Oncology (AERO).

Contact
Eric GAUTHIER
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Tel. 1 : 0328163570
Mobile : 0627221945
Mail : eric.gauthier@bayer.com

Company name: **Bristol-Myers Squibb**
Company size: **25 000 employees**
Number of countries: **30**
Annual sales 2014: **15.9 billions $**
R&D Staff: **7 300 staff worldwide**
R&D investment: **4.5 billions $**
Haedquaters: **New York City**

COMPANY DESCRIPTION

Our Mission
To discover, develop and deliver innovative medicines that help patients prevail over serious diseases.

Our Commitment
To our patients and customers, employees, global communities, shareholders, environment and other stakeholders, we promise to act on our belief that the priceless ingredient of every product is the integrity of its maker. We operate with effective governance and high standards of ethical behavior. We seek transparency and dialogue with our stakeholders to improve our understanding of their needs. We take our commitment to economic, social and environmental sustainability seriously, and extend this expectation to our partners and suppliers

HISTORY OF COMMITMENT

Sprycel (Dasatinib)
Yervoy (Ipilimumab)
Opdivo (Nivolumab)
Daklinza (Daclatasvir)
Eliquis (Apixaban)
Nulojix (Belatacept)
Orencia (Abatacept)

R&D FOCUS PORTFOLIO AND PROSPECTIVE IN THIS AREA

ImmunoOncology
ImmunoScience
Virology
Cardio Vascular
Fibrotic Diseases
Genetically Defined Diseases
Metabolics

PLACES OF WORLDWIDE INVESTIGATIONS

Several major centers with network of facilities worldwide (R&D and plants)

Isabelle DUPRAT LOMON
Disease Area Head ImmunoScience

Training and academic background
1987
Medical Doctor (Paris University)

1986
Degree in Statistics - Epidemiology and Research (Paris University)

1995
Degree in Pharmaco-Epidemiology (Paris University)

Industry background
Present Disease Area Head ImmunoSciences - BMS France

2014 - 2009
Director Health Economics and Outcomes Research - Europe in CV Metabolics & Virology - BMS Europe

2009 - 2005
Director Europe Pricing and Reimbursement Europe - BMS Europe

2005 - 1995
Health Economics ad Outcomes Research - Bayer Pharma France and Europe
1995 - 1987
Clinical Project Leader Phase II to IV in Bayer Pharma and Upsa

Areas of research interest
ImmunoSciences
Outcomes Research
Epidemiology

Contact
Address BMS France
3 rue Joseph Monier
92500 Rueil Malmaison
Tel. 1 + 33 (0) 1 58 83 68 96
Mobile + 33 (0) 6 07 59 87 60

Company name: **Clevexel Pharma**
Company size: **53**
R&D Staff: **30%**

COMPANY DESCRIPTION

Created in 2013 as a spin-off of CEPHALON Laboratory (TEVA Group) CleveXel Pharma is a pharmaceutical company with an innovative business model based on maximizing the marketing potential of disruptive molecules in exchange for a stake in the partner's equity or for the gradual purchase of a license. CleveXel Pharma selects promising molecules from the proof of concept in animals and catalyzes their development to the proof of concept in humans stage at which it becomes marketable by the pharmaceutical industry.

PEOPLE

Skilled employees (15 years of service on average) offer a wide array of skills in the CNS with Modiodal, Provigil and Nuvigil. Leadership of a complementary management team with unique pharmaceutical background

STRATEGIC THERAPEUTIC DOMAINS

To date CleveXel Pharma has a diversified portfolio of 10 projects covering the fields of oncology and central nervous system.

BUSINESS MODEL

Selection of high potential projects at the end of the discovery phase through a selective process of due diligence. Investment “in-kind” by providing means of development against equity participation. Development from early preclinical stage to proof of clinical efficacy drugability & marketability assessment partnership with clinical centers. Project out-licensing to middle and big pharmaceutical companies. Revenue sharing with partners with upfront milestones and royalties from the out-licensee.

R&D EXPERTISE

Highly specialized 53 staff offering a wide array of skills in drug development and clinical research with all the associated technical and QA/QC support.

HISTORY OF COMMITMENT

Active Member of ARIIS Committee & of the RIR Program since their inception.

R&D FOCUS PORTFOLIO AND PROSPECTIVE IN THIS AREA

2 Actives R&D PROGRAMS :
CVXL-0056 : ~Myelodyplastic Syndromes (MDS)
CVXL-0101 : Acute Myeloid Leukemia and Non-Hodgkin lymphomas

Laurent BENEL
Life Sciences CSO

Training and academic background
Doctorat (Ph D) in Pharmaceutical Sciences from Université Paris Sud & Ecole Pratique des Hautes Etudes Cellular and molecular biologist by training with a strong background in cell culture and cellular pharmacology. Complementary training in project management, team management, metrology, computer system validation, ISO-9001, GAMP, GLP.

Industry background
2013 - Present
Co-founder & Senior Director, Life Sciences CLEVEXEL Pharma Expertise in the field of CNS & ONCOLOGY : Lifes Sciences/Clinical Research/Pharmaceutical Development/Quality.

2010 - 2013
Associate Director, Validation and Metrology CEPHALON, France.

2001 - 2010
In charge of the completion of renovation project for the Development Center (in absence of site director). Associate Director, Validation, Logistics and Support Department Senior Manager, Validation and GxP Support Department Head of Oncology Research Department CEPHALON, France.

1995 - 2001
Head of Inflammation Biology Department Laboratoire LAFON, France.

2001 - 1992
Senior Researcher, Pharmacokinetics & Metabolism Department Contract researcher, Cellular Toxicology unit (implanted at Faculty of Pharmacy in Paris) Laboratoire LAFON.

Christian BLOY
President

Training and academic background
6 YEARS OF FONDAMENTAL RESEARCH IN HEMATOLOGY AND CELLULAR BIOLOGY

Collaboration with English and American laboratories. 21 YEARS OF PRE-CLINICAL DEVELOPMENT and MANUFACTURING TECHNICAL SUPPORT. Elaboration and following up of quality control and manufacturing optimization development plans. Elaboration and following up of pre-clinical development plans.

Creation and follow up of an experts' group. Management of a Development center ...

1991: Post doctoral training course in the P. Agre's laboratory (John Hopkins Hospital, Baltimore, USA).

Peter Agre, Nobel Price of Chemistry in 2003.

1990: Doctorat (Ph.D.), Immunology, University of Paris VI, France. 1986: Diplôme d'Etude et de Recherche en Biologie humaine (DERBH), Immunohematology, University of Paris VI, France.

1985: C.E.S. Immunology, University of Paris VI, France.

1984: Master degree in Biochemistry and Immunology University of Paris VI, France.

Industry background
2013 - Present
CLEVEXEL Pharma CEO & Founder Expertise in the field of CNS & ONCOLOGY : Lifes Sciences/Clinical Research/Pharmaceutical Development/Quality.

2013 - 2010
CEPHALON EUROPE / TEVA Site Manager & Senior Director Europe Drug Development / Member of European executive committee.

2002 - 2010
GENZYME CORPORATE : Senior Director, Research & Development / Member of senior sciences committee : Immune Mediated Diseases / Transplant. GENZYME POLYCLONALS S.A.S : Senior Director of Technical Services / Member of senior executive committee. GENZYME POLYCLONALS S.A.S : Director of Research and Development / Member of executive committee.

1998 - 2002
FLAMEL TECHNOLOGIES Direction of Pre-Clinical Development / Member of management committee.

1992 - 1998
CASSENNE LABORATORY (HOECHST MARION ROUSSEL group) Pre-Clinical Department Manager. CASSENNE LABORATORY Head of Pre-Clinical Vascular Development : Development of pharmaceutical molecules to support the medical marketing department (publications, action mechanisms) & to define new therapeutic directions.

1986 - 1992
INSTITUT NATIONAL DE TRANSFUSION SANGUINE Engineer, Head of project in J.P./ Cartron's Laboratory.

Adrien DECQUE
Scientific Coordinator

Training and academic background
2010 - 2014
PhD Candidate - Institut Pasteur. Nuclear Organization and Oncogenesis unit, headed by Pr Anne Dejean. Thesis subject : SUMOylation in innate immunity and oncogenesis.

2007 - 2010
Ecole Normale Supérieure de Lyon. Normalien. Specialty : Biology.

2005 - 2010
PharmD - Institut des Sciences Pharmaceutiques et Biologiques. Université Claude Bernard Lyon 1.

Industry background
2014 - Present
CLEVEXEL Pharma - Scientific Coordinator & Sourcing, Lifes Sciences.

genzyme
A SANOFI COMPANY

Company name: **Genzyme**
Company size: **8 000 employees**
Number of countries: **40**
Annual sales 2014: **2604M€**
R&D investment: **$ 8,393 Billions**

COMPANY DESCRIPTION

Genzyme has pioneered the development and delivery of transformative therapies for patients affected by rare and debilitating diseases for over 30 years. We accomplish our goals through world-class research, collaboration with the global patient community, and with the compassion and commitment of our employees. With a focus on rare diseases and multiple sclerosis, we are dedicated to making a positive impact on the lives of the patients and families we serve. Genzyme's portfolio of transformative therapies, which are marketed in countries around the world, represent groundbreaking and life-saving advances in medicine. As a Sanofi company, Genzyme benefits from the reach and resources of the world's largest pharmaceutical companies with a shared commitment to improving the lives of patients.

HISTORY IN THE AERA

As a pioneer of the biotechnology movement in the early 1980s, Genzyme has always been driven by cuttingedge science and a commitment to treating unmet medical needs. Genzyme has long been known for our expertise in the class of rare genetic diseases known as lysosomal storage disorders (LSDs). We have also expanded – through both in-house development and strategic acquisitions and partnerships – to other disease areas such as thyroid cancer and multiple sclerosis.

PRODUCT PORTFOLIO IN THE AREA

Products Marketed (Worldwide): Aldurazyme®, Cerdelga®, Cerezyme®, Cholestagel®, Elaprase®, Fabrazyme®,

PROSPECTIVE IN THE AREA : THEMATIC PRIORITIES

Creating a solid foundation for Genzyme's future growth, our R&D efforts include novel therapeutic candidates as well as new indications for our existing products. Explore our pipeline to see highlights of our most promising research.

Patisiran**
Familial amyloid polyneuropathy /RNAi/ Phase III

Revusiran**
Familial amyloid cardiomyopathy/RNAi/Phase III

GZ402671
Fabry disease, substrate reduction/ small molecule/Phase II

GZ402671
Gaucher disease Type 3, substrate reduction /small molecule/Phase I
Olipudase (acid sphingomyelinase)
Niemann-Pick disease type B/ protein-based therapy/Phase I

NeoGAA GZ402666
Pompe disease, 2nd generation enzyme /protein-based therapy/Phase I

AAV2-hAADC*
Parkinson's disease /gene therapy/Phase I

Items marked with a ** are being developed in collaboration with Alnylam and * are being developed in collaboration with Voyager Therapeutics.

In addition to the candidates in clinical development, we are conducting earlier stage research in a range of therapeutic areas including genetic diseases such as cystic fibrosis, spinal muscular atrophy, and Leber's congenital amaurosis type 1.

Christian DELEUZE
President Genzyme France and Genzyme Polyclonals

Christian Deleuze is currently the President of Genzyme France and Genzyme Polyclonals, part of the Sanofi Group. He joined the company in 2010 and leads the company's strategic planning in line with the global Genzyme mission, ensuring patients are provided with treatments, driven by cutting-edge science and a commitment to treating unmet medical needs in Rare Diseases, (Gaucher, MPS1, Fabry and Pompe disease) and since 2014, in the Multiple Sclerosis therapeutic area.

He obtained his medical degree from, Lariboisière Saint-Louis in Paris and graduated from the ESSEC Business & Management School In the early 90's. He began his professional pharmaceutical career at Bayer Pharma, and from there went on to hold different positions at Searl, Pharmacia and Pfizer where he was Marketing Director and responsible for the launch of Celebrex, in charge of strategy and tactics for branding and global positioning.

In 2003 he created the French subsidiary of Sankyo Pharma, which became Daiichi Sankyo France. In his role as President and founder, from 2003 to 2010, he positioned France as the European leader for the Japanese group, with 500 employees.

Today Christian is also President of the Rare Disease Committee at the LEEM (the French pharma syndicate), where he sits on the board and is a member of both the Administration Committee and the Biotechnology Committee. The LEEM Rare disease group unites the rare disease community; pharmaceutical companies, association representatives and health institutions dedicated to the orphan disease cause.

Genzyme exists in France since 1994 and counts over 400 employees in total. The headquarters are situated in Saint Germain en Laye (78), with teams throughout the French regions including activities in French overseas territories and in Lyon at the Polyclonal bioproducion site. The plant is in the Gerland district in Lyon, the world renowned biotech cluster. The site produces polyclonal antibodies preventing organ rejection in transplanted patients. This product is exclusively produced by the Sanofi group at Genzyme in Lyon and is available in 68 countries worldwide.

Seng H. CHENG
Biosketch - PhD

Seng Cheng is Head of Research and Early Development of the Rare Diseases Division at Genzyme, a Sanofi Company. He received his BSc and PhD degrees in Biochemistry from the University of London, UK and trained as a postdoctoral fellow at the National Institute for Medical Research in London in the field of tumour biology. He worked as a Staff Scientist at Integrated Genetics Inc., and later joined Genzyme Corporation to work on several discovery projects including the structure and function of the cystic fibrosis transmembrane conductance regulator. As Group Vice President of Genetic Diseases Science at Genzyme, he also managed the development of novel gene delivery systems as well as translational research in genetic diseases, a number of which transitioned to clinical testing. Dr Cheng's current areas of focus include inherited metabolic, muscle, lung and neurodegenerative diseases. He has co-authored 249 research articles and reviews, and is a named co-inventor on 58 issued patents in the area of biotechnology. In his current position, he is responsible for directing the translational research and early clinical development activities in rare genetic diseases.

Company name: **GlaxoSmithKline**
Company size: **96 500 employees**
Number of countries: **115**
Annual sales 2014: **32,8 billion euros**
R&D Staff: **14 000**
R&D investment: **4 billion euros**

COMPANY DESCRIPTION

We are a science-led global healthcare company that researches and develops a broad range of innovative medicines and brands. Our products are used by milions of people around the world, helping them to do more, feel better and live longer.
We have three primary areas of business in pharmaceuticals, vaccines and consumer healthcare.Research is vitally important to the success of our business. We are one of the few healthcare companies researching medicines and vaccines for the World Health Organisation's three priority diseases - HIV/AIDS, tuberculosis and malaria.

HISTORY OF COMMITMENT

GSK's Rare Diseases Unit (RDU) was established in 2010 to develop and commercialize GSK's existing and future portfolio of medicines indicated for the treatment of rare diseases, a grouping of more than 7000 diseases usually defined by a prevalence <= 5/10 000 in the US and in EU.
The mission of the RDU is to be a leading provider of breakthrough solutions to patients with serious unmet medical needs, a hallmark of patients suffering from rare diseases. Increasing scientific and genetic understanding of human biology has made the area of rare disease treatments a fertile area for drug discovery and development. The creation of the RDU brings together a dedicated team that can access the GSK's resources while operating in a more personalised way with the rare disease community through seeking their insights. GSK is utilizing its broad expertise in development of medicines, significant scientific and regulatory experience, manufacturing capability and global reach to create and deliver new medicines to treat rare diseases.
Rare diseases occur across a very broad spectrum of illness with diseases often having little or nothing in common with each other; as a consequence new medicines can emanate from any of GSK's Discovery Performance Units (DPUs) or Therapy Areas (TAs) or from external collaborations.

PRODUCT PORTFOLIO IN THE AREA

The RDU currently has a core commercial portfolio of Pulmonary Arterial Hypertension (PAH) medicines, a pipeline of late stage medicines in gene therapy and amyloidosis, as well as a rich and diverse early stage pipeline spread across many rare diseases indications. In 2015, as a result of a partnership with San Raffaele Telethon Institute for Gene Therapy, GSK became the first pharma company to file a marketing application for a stem cell ex vivo gene therapy anywhere in the world for ADA-SCID - a rare and devastating disease in which children have a severely deficient immune system because of a mutation in one gene.

R&D FOCUS PORTFOLIO AND PROSPECTIVE IN THIS AREA

As part of efforts to help as many patients as possible with rare diseases, the RDU will continue working with the internal discovery and early development units and also review and examine external opportunities for collaboration.

PLACES OF WORLDWIDE INVESTIGATIONS

Over the past three years, we have collaborated with several institutions all over the world

Andrea SPEZZI

Vice President and Medicine Development leader GSK. Rare Diseases Unit

Dr. Andrea Spezzi has more than 12 years of experience in the global pharmaceutical industry, leading compounds and people to develop from early discovery through clinical development and registration/commercialization, medicines that address unmet medical needs worldwide.

Currently Andrea serves as Vice President and Medicine Development Leader in GSK Rare Diseases Unit, leading a successful portfolio in Pulmonary Arterial Hypertension, advancing therapies in the field of lysosomal storage diseases, particularly those with CNS compromise, and providing medical and clinical development expertise for the Gene Therapy portfolio in the area of immune deficiencies. Previously, she held different global positions of increased responsibilities in Takeda Pharmaceuticals base in the London office and a year in the Chicago, USA, where she had responsibilities for the Cardiovascular and Metabolic Therapeutic area. Before that, she worked in London at Hammersmith Medicines Research Unit as Research Physician, focusing in clinical pharmacology, translational medicine and phase I/II studies in multiple therapeutic areas. Before moving to the pharmaceutical industry, Andrea practiced Paediatrics in England, Spain and Argentina.

Andrea graduated as Physician from the Faculty of Medicine, University of Buenos Aires (UBA), she is Bachelor of Medicine and Surgery in Madrid, Spain; she is Specialist in Paediatrics and completed a post graduate training in Paediatric Immunology at the University Children Hospital Pedro de Elizalde in Buenos Aires, Argentina. She is a Specialist in Pharmaceutical Medicine from the University of Basel and was awarded with the title of Fellow of the Royal College of Pharmaceutical Physicians in London, UK.

Soizic COURCIER

Medical & Regulatory Affairs Director

Soizic Courcier is Physician (University Bichat-Beaujon, Paris), graduate in Business Strategy (ESCP) and Public Health, Market Access and Health Security (CNAM).

She joined the pharmaceutical industry for over 20 years. She has held positions of increasing responsibilities in clinical development and medical affairs in different companies, at international and national level.

Soizic Courcier holds the position of Medical and Regulatory Affairs Director since 2007 within GlaxoSmithKline (GSK).

She is involved in many initiatives including in the LEEM where she chairs as President of the Clinical Research Attractiveness group.

She is also member of the Executive Committee of ARIIS (Association for Research in Health Industries) as well as Treasurer.

Zeina ANTOUN

Director, Clinical Research, Academic Alliances and NPI

Zeina Antoun holds an MD degree from the American University of Beyrouth and has an American Board certification in Internal Medicine, and a University Diploma in Infectious Diseases. Between 1994 and 1998, she worked at Bichat, Claude-Bernard Hospital as a full-time clinician in Infectious Diseases and HIV.

In 1998, she joined GlaxoSmithKline France, Clinical research unit in Virology, and was then appointed in 2004 as Director, Clinical research in Virology, Therapeutic Vaccines, Oncology and Respiratory diseases. In 2007, Zeina Antoun created a new department dedicated to Early phase research, New domains and Academic alliances, creating many links between GSK R&D groups and French Public research teams.

Zeina Antoun is a member of several external working groups at LEEM, ARIIS, Investment funds and other boards. She is also co-author on many publications.

Company name: **IPSEN**
Company size: **4 500 employees**
R&D staff: **900 employees**
Annual sales 2014: **€ 1.2 billion**
www.ipsen.com

COMPANY DESCRIPTION

Ipsen is a global specialty-driven pharmaceutical company committed to the treatment of targeted debilitating diseases. Its development strategy is supported by three franchises: neurology, endocrinology and urology-oncology. Ipsen also has a significant presence in primary care. Ipsen's R&D is focused on its innovative and differentiated technological platforms, peptides and toxins. In 2014, R&D expenditure totaled close to €187 million, representing close to 15% of Group sales.

The Group has an active policy of partnerships. Therefore, the Group has strategically established its R&D sites in geographical proximity with highly regarded university research centres, enabling the teams to access the best science & innovation:

i) The Research and Development Centre at Les Ulis (France) is close to Paris-Saclay campus. The scientists are focused on drug discovery of novel medicines in the fields of neurology and oncology.

ii) The Research and Development Center newly established in Cambridge (Massachusetts, United States) reinforces its leadership in the field of peptides and its open-innovation strategy with proximity of world-class academic centers and biotechs.

iii) The Research Center in Abingdon (Oxford, UK) _ Ipsen BioInnovation (previously Syntaxin) _ is focused on the discovery of new modified recombinant botulinum toxins, mainly for neurology indications.

HISTORY OF COMMITMENT

Ipsen's mission, "innovation for patient care", highlights the Group's determination to focus on patients and unmet medical needs and to provide innovative therapeutic solutions. Focusing on patients is critical to determine unmet medical needs and identify both the pathophysiological mechanisms and biological targets that should be developed.

Rare diseases is an area where patients definitively need new and differentiated treatments. Ipsen is highly engaged in the delivery of treatments for the management of Neuroendocrine tumors (NET) which represent 1% of digestive tumors. Outside oncology sphere, Ipsen focuses its efforts in neurology on helping people suffering from neurodegenerative conditions responsible for movement disorders. In this particular field, Ipsen develops programs in Parkisonism and Huntington's disease.

PRODUCT PORTFOLIO IN THE AREA OF RARE DISEASES

Somatuline: the first therapy approved by the FDA (2014) in the United States for the treatment of patients with locally advanced or metastatic gastroenteropancreatic neuroendocrinetumors (GEP-NETs). Somatuline®'s approval was based on demonstration of improved progression-free survival (PFS) in CLARINET® study that enrolled 204 patients with unresectable, well- or moderately-differentiated, locally advanced or metastatic, non-functioning GEP-NETs.

Increlex: a formulation for twice daily injection of human recombinant IGF-1 used for the long-term treatment of growth failure in children and adolescents with severe primary insulin-like growth factor 1 deficiency (primary IGFD).

Dysport®: a botulinum neurotoxin type A complex, used notably to treat spasticity of upper limbs following a stroke or spinal cord injury.

R&D FOCUS PORTFOLIO AND PROSPECTIVE IN THIS AREA

BN82451B (Phase IIa) – this molecule is currently in clinical development in Huntington's disease and has completed a phase I clinical pharmacology trial. A Phase IIa clinical proof of concept of BN 82451B in Huntington Disease patients is currently underway in Germany. This molecule is simultaneously able to exert multiple pharmacological activities and is designed to protect the mitochondria.

LRRK2 kinase inhibitors (Pre-clinical phase) – Ipsen has entered in 2012 into a research collaboration with Oncodesign to discover and develop innovative LRRK2 kinase inhibitors as potential therapeutic agents against genetic forms of Parkinson's Disease.

PLACES OF WORLDWIDE INVESTIGATIONS

USA / UK / France

Christophe THURIEAU

Sr VP Global Scientific Affairs and Strategic Sourcing & President Ipsen Innovation

Training and academic background
1987-1989
Pre and post doctoral fellow, Harvard Medical School, Boston (USA).

1986-1988
Ph.D., University Pierre and Marie Curie, Paris , France.

Industry background
2010-Present
Sr VP Global Scientific Affairs and Strategic Sourcing & President Ipsen Innovation.

2007-2009
VP Translational Research and President Ipsen Innovation.

2002-2007
VP Research, Ipsen group.

2000-2001
Research Director, Ipsen group.
1997-2000
Senior Director New Discovery Technologies, Ipsen group.

1989-1996
Laboratory Head, Servier laboratories.

Areas of research interest
Oncology, Neurology, Endocrinology, Biology and chemistry technology platforms.

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Pierre-Etienne CHABRIER

Vice President Scientific Affairs Neurology at IPSEN

Training and academic background
Docteur "es Sciences"
Post-doctoral fellow at the Imperial College in London (UK)
Training in chemistry and pharmacology in CNRS and Pasteur Institute (France).

Industry background
2010-Present
Vice President Scientific Affairs Neurology / IPSEN

1982
Pierre-Etienne joined IPSEN in 1982 to set up a Molecular Pharmacology unit before becoming Head of Biology research.
Leading various research programs in neurology, oncology, inflammation and cardiovascular system, he was notably involved in the study of endothelial vasoactive substances and their role in cerebrovascular disorders.
In the field of neurology, he has developed pharmacological strategies for the treatment of neurodegenerative diseases and neuromuscular disorders with small molecules and proteins.
Areas of research interest
Identify and manage external collaborations from research to early clinical development in neuromuscular disorders.

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Sylvia JULIEN

Senior Project Leader External Partnerships, Scientific Affairs, IPSEN

Training and academic background
2006-2007
POST-DOCTORAL SCIENTIST,
Max-Planck-Institute of Biochemistry, Martinsried, Germany.

2000-2006
DOCTORAL SCIENTIST & MASTER STUDENT,
Institut Curie, Orsay, France.

1997-2001
Ecole Normale Supérieure de Cachan, Biochemical and Biological Engineering Department.

Industry background
2013-Present
Senior Project Leader External Partnerships, Scientific Affairs department/ IPSEN.

2010-2013
Project Leader External Partnerships, Scientific Affairs department/ IPSEN.

2008-2009
Post-doctoral fellow – CReMEC Consortium / IPSEN.

Areas of research interest
Identify, set-up and manage research / pre-clinical strategic alliances and pre-competitive consortia with academic institutes, biotechs, pharmaceutical industries and software editors in the following areas: uro-oncology, endocrinology, neurology using peptided and toxins as therapeutic agents.

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Company name: **Janssen**
Company size: **118,000 employees**
Number of countries: **60 countries**
Annual sales 2014: **886M€**
R&D investment: **7,5 Milliards de dollars**
Headquarters: **Issy les Moulineaux, France**

COMPANY DESCRIPTION

Janssen: A Reputation for Innovation

Janssen is one of the world's leading research-based pharmaceutical companies, with operations throughout the world.

The company is committed to delivering great medicines and has introduced a range of innovative treatments that can make an important difference to the lives of patients with serious health conditions such as schizophrenia, epilepsy, prostatic cancer, multiple myeloma, lymphoma, HIV/AIDS, hepatitis C, Castelman disease.

Our Innovation
We currently have more than 100 ongoing collaborations and alliances globally. These strategic relationships take many forms, but our goal is always the same: to advance scientific research to deliver innovative, differentiated solutions that provide value to patients, physicians and health care systems around the world.

PRODUCT PORTFOLIO IN THE AREA

In orphan diseases, we have siltuximab for Castelman disease and ibrutinib for chronic lymphocytic leukemia and non-Hodgkin lymphomas.

R&D FOCUS PORTFOLIO AND PROSPECTIVE IN THIS AREA

Tumor Strongholds

To execute our overarching strategy, we have created internal Tumor Strongholds (TSHs) whose function is to:
- Focus on particular tumor types where the need for treatment options is greatest;
- Bring the most compelling science to bear to develop transformational therapeutic solutions.

Tumor Strongholds are a highly effective model for drug development, enabling a focused strategy that informs prioritization, optimizes investment and ensures the best chance of success.

Within each stronghold, our discovery and clinical development areas (including biomarkers and translational medicine) and our commercial capabilities are fully integrated and strategically aligned – from the start and through every stage of drug development and commercialization – ensuring that the resources, insight and expertise needed to deliver new advances are fully dedicated and optimized.

Our priority TSHs focus on hematologic malignancies and prostate cancer. Additional areas of focus include lung cancers, colorectal cancer and breast cancer. We also are keenly interested in identifying new targets for drug development in pre-malignancies, and in technologies that can accelerate target identification, drug screening, predictive biomarkers and drug development.

The Importance of Biomarkers
Biomarkers are an increasingly important component of our drug development activities, whether we are testing compounds early in development or investigating strategies to intercept cancers at their earliest stages and identify patients at imminent risk of cancer progression.

In addition, we see enormous potential in transforming predictive biomarkers into companion diagnostic tests that can then be used in the clinic and help guide targeted, individualized use of our therapies. As a result, a growing number of our pipeline products have a companion diagnostic in parallel development.

Evolving Tumor Microenvironment Strategy
Our evolving strategy focuses on innovative approaches to drug development based on a deep understanding of the critical relationships between cancer cells and the tumor microenvironment. For the first time, paradigmshifting therapeutic targets are now within reach as we learn more about the extent to which tumor cells interact with normal cells, secreted proteins, small molecules and blood vessels in their immediate surroundings, and the critically important role this plays in cancer initiation, progression, metastasis and drug resistance.

Alain MIARA
Medical Strategy and Partnerships

Training and academic background
Medical Doctor and Clinical Pharmacology Master 2 by training

Industry background
Working in Janssen for 13 years

Areas of research interest
Janssen is involved in 5 major therapeutic areas:
Cardiovascular and Metabolism
- Type 2 Diabetes

Immunology
- Rheumatoid Arthritis
- Inflammatory Bowel Disease
- Psoriasis
- Pulmonary Infectious

Diseases and Vaccines
- Hepatitis
- Respiratory Infections Neuroscience
- Mood Disorders
- Alzheimer's Disease

Oncology
- Prostate Cancer
- Lung Cancer
- Hematologic Malignancies

Contact
Address 1, Camille Desmoulin Street
92057 Issy les Moulineaux

Company name: **Lilly**
Company size: **70 000 employees**
Number of countries: **125 countries**
Annual sales 2014: **19,6 Billions $**
R&D Staff: **8 042 employees**
R&D investment: **4,7 Billions $**
Headquarters: **Indianapolis (Indiana, U.S.A.)**

COMPANY DESCRIPTION

Lilly units caring with discovery to make life better for people around the world.

HISTORY OF COMMITMENT

We've been the pioneers behind major breakthroughs against some of the world's most devastating diseases, but our work is never finished. Discovering new medicines means never being satisfied with what we've achieved. It demands the determination to overcome failure, push beyond convention, and find ways to come through no matter the odds. It means uniting our expertise with the creativity of research partners around the world to keep finding ways to make life better.

R&D FOCUS PORTFOLIO AND PROSPECTIVE IN THIS AREA

We currently have the richest mid-to-late stage pipeline in our history, representing a variety of therapeutic areas mainly including cancer, diabetes, neurosciences and autoimmunity We're focused on developing a complementary mix of small and large molecules across our pipeline in order to address the diverse needs of the patients we serve.
Personalized medicine, also known as "tailored therapeutics", promises to deliver greater precision, higher value and improved outcomes for indiviudal patients. Tailoring is being built into every single drug development program, using a variety of approaches, to identify differences across patient populations. This lies at the heart of Lilly's vision for biopharmaceutical innovation.

PLACES OF WORLDWIDE INVESTIGATIONS

United States, Canada, China, England, Japan, Singapore, Spain and a joint venture facility in Australia

Trafford CLARKE
Managing Director, Lilly Research Centre, Eli Lilly and Company Ltd

Trafford Clarke obtained a BSc in Chemistry from the University of Liverpool after which he completed a PhD in Organic Chemistry in Prof. Steven Ley's research group at Imperial College, London. He then spent two years as a post-doctoral fellow at Cornell University in the USA in Prof.

Bruce Ganem's research group. Trafford joined Eli Lilly and Company in 1986 as a medicinal chemist since when he has worked extensively across the LRL R&D organization both in Europe and the USA.

In 2003 he served as Senior Director in the Pharmaceutical Project Management organization with responsibility for all LRL assets from candidate selection to end-Phase II.

From 2004-7, he was the site-head for the R&D facility in Mont-Saint Guibert (MSG) in Belgium after which he returned to Corporate Centre as Executive Director, LRL Six Sigma. Effective March 2013, he was appointed as site-head of the Erl Wood Research facility in the UK

clarke_trafford@lilly.com

Company name: **MSD**
Company size: **70 000 employees**
Number of countries: **more than 140 countries**
Annual sales 2014: **$42,2 billion**
R&D Staff: **14 100 employees**
R&D investment: **$6.5 billion**
Partnerships: **private and academic partnerships**

COMPANY DESCRIPTION

MSD is a global leader in delivering innovative health solutions through its medicines, vaccines, biologic therapies, and consumer and animal health products. MSD also demonstrate its commitment to increasing access to healthcare through far-reaching policies, programs and partnerships. MSD is known as Merck in the United States and Canada.
MSD product offering categories include heart and respiratory health, infectious diseases, diabetes, cancer, inflammatory diseases and women's health.
MSD focus research on conditions that affect millions of people around the world - diseases like Alzheimer's, diabetes and cancer

PRODUCT PORTFOLIO IN THE AREA

Our main therapeutic areas :

- Diabetes : Januvia® (sitagliptin) and Janumet® (sitagliptin/metformin HCl),
- Inflammatory/immunology : Simponi® (golimumab), Remicade®(infliximab)
- Oncology : Emend® (aprepitant), Temodar® (temozolomide), Sylatron® (peginterferon alfa-2b), Zolinza® (vorinostat) and Keytruda® (pembrolizumab) for the treatment of advanced melanoma in patients whose disease has progressed after other therapies
- Cardiovascular
- Infectious diseases
- Respiratory

MSD is interested in the concept of rare diseases as models for more common diseases

For more information
http://www.merck.com/product/home.html

PROSPECTIVE IN THE AREA : THEMATIC PRIORITIES

We focus our research on several key therapeutic areas, including:

ONCOLOGY
Pembrolizumab MK-3475 :
Merck's anti-PD-1 therapy, is being investigated for use in more than 30 tumor types of cancers, as monotherapy and in combination. KEYTRUDA is a humanized monoclonal antibody that blocks the interaction between PD- (Programed Cell Death-1 protein) and its ligands, PD-L1 and PD-L2.

Phase III and Under Review
Bladder Cancer (Pembrolizumab)
Head and Neck Cancer (Pembrolizumab)
Non-Small Cell Lung Cancer (Pembrolizumab)
Melanoma (Pembrolizumab) (Europe)

Phase II
Solid tumors : **MK-2206** is an orally available allosteric small-molecule inhibitor of AKT
Gastric Cancer (Pembrolizumab) MK-3475

NEURROSCIENCES
Phase III and Under Review
Alzheimer's Disease : MK-8931 is an orally available investigational inhibitor of beta-site amyloid precursor protein cleaving enzyme 1 (BACE) that is being evaluated in clinical trials for the treatment of Alzheimer's disease.
Phase II
Alzheimer's Disease : MK-7622

DIABETES AND ENDOCRINOLOGY
Phase III and Under Review
Ertugliflozin (MK-8835) is an investigational oral sodium glucose cotransporter (SGLT2) inhibitor being evaluated for the treatment of type 2 diabetes.
Collaboration : Ertugliflozin is being developed in collaboration with Pfizer, Inc.

Omarigliptin (MK-3102) is an investigational oral, once-weekly DPP-4 inhibitor being evaluated for the treatment of type 2 diabetes.

MK-1293 is an investigational insulin glargine candidate being evaluated for the treatment of patients with type 1 and type 2 diabetes.

For more information on the entire MSD research programs
www.merck.com/research/pipeline/home.html

Kumaril (Kumar) BHARGAVA
Executive Director at Merck Research Labs

Training and academic background
Ph.D., MBA, R.Ph. Kumar holds a Bachelors degree in Pharmacy from Birla Institute of Technology & Science, Pilani, India a Ph.D. in Pharmacy from The Philadelphia College of Pharmacy & Science and an MBA from the University of Chicago. He is a licensed pharmacist in the State of Pennsylvania.

Industry background
Prior to merck he was a Chief Executive Officer at Sarentis Therapeutics, a specialty company with focus in Opthalmology. Prior to Sarentis Dr. Bhargava was a Senior Director in External Research & Development Innovation group at Pfizer where he played a key role in acquiring multiple technologies and companies in Rare and Orphan Diseases and establishing collaborations with biotech firms and academia.

Prior to Pfizer Kumar was a Product Director for Procrit™, four billion dollar franchise for Johnson & Johnson. In this role he lead the efforts in finding new ways to treat anemia associated with end stage renal disease. He has more than fifteen years of experience in Drug and Business Development gained at Pfizer, J&J and at Abbott Labs.

Areas of research interest
finding opportunities in Orphan disease area, Regenerative medicine and diseases that has clear genetic linkage

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Manfred HORST
Director Licensing & External Research Europe for MSD

Training and academic background
MD, PhD, MBA. Dr. Manfred Horst studied medicine in Munich, Montpellier and London and specialised in Allergic Diseases at the University Clinic in Montpellier, where he also completed his PhD.

Industry background
After earning his MBA at INSEAD, Fontainebleau, Manfred spent 7 years with Ciba-Geigy and Novartis, Basel, two years as General Manager of a startup in the health insurance business, and up until now 13 years with Merck & Co./MSD. Since 2004, Manfred has been in his current function as Director Licensing & External Research Europe for MSD, where he scouts for Licensing opportunities and manages alliances in a number of European countries

Areas of research interest
Immunology and atopic/allergic diseases

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Company name: **Pfizer**
Company size: **78 300 employees (2014)**
Annual sales 2014: **$ 49,6 Billions**
R&D investment: **$ 8,393 Billions**
Partnerships: **more than 200 new R&D collaborations in 2014**

COMPANY DESCRIPTION

Pfizer Inc.: Working together for a healthier world®
At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products. Our global portfolio includes medicines and vaccines as well as many of the world's best-known consumer health care products.

Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world.

For more than 150 years, Pfizer has worked to make a difference for all who rely on us. To learn more, please visit us at www.pfizer.com.

HISTORY OF COMMITMENT TO RARE DISEASES

Pfizer's Rare Disease Research Unit, led by Chief Scientific Officer Kevin Lee, is adopting an innovative and collaborative approach to the development of new medicines for patients with rare diseases. We have a track record of creating innovative strategic partnerships with academic institutions, patient advocacy groups, and commercial enterprises to accelerate the development of novel therapeutics across the entire spectrum of rare diseases. Our commitment to academic collaboration is highlighted by the recent signing of the Rare Disease Consortium agreement with six of the leading Universities in the UK, providing a vehicle to work collaboratively with leading physician scientists on drug discovery projects.

We are looking to capitalize on recent scientific advances linking diseases to specific genetic defects. As 70% of rare diseases are monogenic in origin, we believe this is an area where scientific knowledge is enabling significant advances in drug development.

Our expertise in large molecule therapeutics, small molecule protein chaperones, and transcriptional modulators has resulted in a broad pipeline of potentially transformative medicines across multiple disease areas.

PRODUCT PORTFOLIO IN THE AREA

We have built a strong non-cancer rare diseases portfolio of 12 medicines approved worldwide that treat rare diseases in the areas of hematology, neuroscience, inherited metabolic disorders and pulmonology. In the near term we are helping patients manage their disease and improve their quality of life. We also have longer-term work that is exploring how to correct certain rare diseases by studying the underlying causes of the disease. More details on our website.
www.pfizer.com/research/science_and_technology/product_pipeline

PROSPECTIVE IN THE AREA : THEMATIC PRIORITIES

Pfizer WRD is interested in establishing alliances to develop therapeutics, expand disease biology understanding, and identify biomarkers that impact:

Hematology (non-malignant)

- **Hemophilia**
  - *Coagulation factors with extended duration of activity and/or improved delivery*
  - *Oral agents to treat haemophilia*
  - *Immune tolerance*
  - *Novel approaches (including gene therapy) to treat hemophilia patients*
- **Other rare hematologic (non-malignant) indications**
  - *Sickle cell anemia, & beta-Thalassemia follow on with focus on disease modifying and/or therapies that significantly change disease pathology*
  - *Hemostasis (systemic and topical)*
  - *Opportunistic approaches in the field of*
  - *hematology that promise well differentiated novel medicines*

Neuromuscular Diseases

- **Duchenne / Becker muscular dystrophy and other muscular dystrophies, disease-modifying therapies preferred**
- **Spinal Muscular Atrophy**
- **Friedreich's ataxia: Upregulate frataxin expression, inhibition of degradation or frataxin pathway bypass**
- **Amyotrophic lateral sclerosis: Protein misfolding approaches and other disease-modifying approaches**

Pulmonary Diseases

- **Cystic Fibrosis (in conjunction with the CF Foundation)**
- **Pulmonary arterial hypertension and idiopathic pulmonary fibrosis**

Disease modifying approaches for other diseases such as transthyretin amyloidosis, myasthenia gravis, Huntington's disease

- **General mechanisms of interest**
  - *Pharmacologic chaperones and other modifiers of protein trafficking, misfolding, or degradation that could apply to multiple diseases (e.g., a small molecule approach that could apply across multiple lysosomal storage disorders)*
  - *Targeting technologies/platforms (e.g., muscle and CNS targeting)*
  - *Modifiers of gene transcription via epigenetic approaches*
  - *Nucleic acid/gene therapy approaches to therapy*
  - *Antibody-drug conjugates*
  - *Oral small molecule and biologics approaches*

Bert BRUCE

Vice President
Rare Disease Commercial Development
Pfizer, Inc.

Bert Bruce, MBA is Vice President for Rare Disease Commercial Development at Pfizer, Inc. His responsibilities span drug development, business development, commercial preparedness and lifecycle management for development and commercialized Rare Disease medicines. He is also accountable for the development and execution of enterprise strategies in Rare Disease.

Bert has a diverse skill set and broad pharmaceutical background, with leadership experiences across multiple therapeutics areas and disciplines and Johnson & Johnson, Wyeth and Pfizer. He has been an integral leader in complex programs including the Alzheimer's Immunotherapy Program and the acquisition team that brought tafamidis (Vyndaqel®) into the Pfizer Rare Disease Portfolio.

Bert earned a B.S. from Eastern College and an M.B.A. from the University of Pennsylvania's Wharton School of Business. He is well recognized for his strategic vision, collaborative leadership, influencing skills, and development of high-performing teams; his professional passions include improving the participation of minorities in clinical trials and leadership development.

Bert currently serves as an Executive Council Member of the National Biotechnology and Pharmaceutical Association, an Executive Board Member of the Get Together Group, a professional life-science networking organization, and as a member of the Pfizer Global Blacks Council.

Brenda COOPERSTONE

M.D., Vice President, Medicines Development Group, Pfizer Inc.

Dr. Brenda Cooperstone received her M.D. from McGill University, completed her residency in pediatrics at the Montreal Children's Hospital, her clinical fellowship in pediatric nephrology at Children's Hospital of Philadelphia and a research fellowship at the University of Pennsylvania, Renal Electrolyte Division.

She is certified by the American Board of Pediatrics and Pediatric Nephrology and the Royal College of Physicians and Surgeons of Canada, Pediatrics. After completion of her training and laboratory work, Dr. Cooperstone was on staff at the Scottish Rite Children's Hospital, a member institution of Children's Healthcare of Atlanta. In 1999, Dr. Cooperstone joined the pharmaceutical industry in Medical Affairs at Wyeth Pharmaceuticals.

She joined Pfizer in 2009 as Vice President, Medicines Development Group in the Specialty Care Business Unit at Pfizer with oversight across a wide range of therapeutic areas. Currently, Dr. Cooperstone is Vice President, Global Medicines Development, with responsibility for the Rare Disease portfolio in the Global Innovative Products Business Unit.

Dr. Gene LIAU

He received his Ph.D. in Biochemistry at Vanderbilt University and his B.S. in Biology from University of North Carolina at Chapel Hill. He held the position of Tenured Professor at George Washington University Medical Center and Senior Scientist, Jerome H. Holland Laboratory, American Red Cross prior to joining the pharmaceutical industry. Dr. Liau is currently Executive Director and leads the External R&D effort for the Rare Disease Unit within Pfizer.

He is focused on developing external alliance strategies, leads Due Diligence teams, champion external collaborations within Pfizer and most recently spearheaded the Gene Therapy collaboration with Spark Therapeutics. Previously, Gene was at Novartis for twelve years, initially building a Research Unit focused on using gene therapy for cardiovascular and metabolic applications and eventually leading a cardiovascular/metabolic division at the Novartis Institutes for BioMedical Research (NIBR) in Cambridge, MA where he was involved in the development of various drugs including aliskiren, LCZ696 and elinogrel.

Prior to joining Pfizer, Gene also helped Shire build an innovative rare disease portfolio. Dr. Liau brings over fifteen years of pharmaceutical drug discovery experience and has authored over fifty peer-reviewed publications. He has a proven track record in developing innovative drug discovery programs, building & leading successful global multi-disciplinary teams, successful in-licensing of external opportunities, and managing large collaborations with academic institutions and biotechnology companies.

Kevin Lee
SVP, CSO and Head of the Rare Disease Research Unit
Pfizer Inc

Kevin Lee is SVP of Research and CSO for the Rare Disease Research Unit at Pfizer. In his current role Kevin has helped define Pfizer's strategy in Rare Diseases and has been instrumental in the initiation and implementation of several notable collaborations recent examples of which include those with the Cystic Fibrosis foundation; Solid Ventures; Care4Rare, the Canadian clinical genetics network and the UK Rare disease consortium involving the GMEC cluster of universities.

Prior to joining Pfizer, Kevin conceptualized and led epigenetics research at GSK and was responsible for the creation of the EpiNova DPU as well as leading the formation of multiple strategic commercial and academic partnerships for the company. Kevin studied pharmaceutical sciences at Nottingham University followed by a PhD in pharmacology at Cambridge. He undertook postdoctoral training as a Wellcome Trust International Prize Fellow before joining the Parke Davis Research unit in Cambridge.

Before joining GSK, Kevin lectured at Warwick Medical School, founded Cambridge Biotechnology (acquired by Biovitrum) and Neurosolutions. Kevin is an author on over 100 peer reviewed scientific publications, has an MBA from Warwick Business School and has been awarded an honorary Chair in Molecular Pharmacology from the University of Warwick

Kevin W. Williams
M.D., J.D., M.P.H.

Kevin Williams joined Pfizer in January 2004 as a Director, Regional Medical & Research Specialists on the HIV RMRS team. In July 2005, Kevin moved into the RMRS Team Leader position leading both a combined ID/HIV RMRS team and, subsequently, a separate HIV RMRS team. After 3½ years in the RMRS Team Leader role, he became the Group Leader, Customer Medical Specialists, U.S. Medical Affairs in the Specialty Care Business Unit in January 2009. In this capacity, he supervised the RMRS across all disease areas in the SCBU, the Outcomes Research RMRS, and the Academic Medicine team. Beginning in 2010, Kevin served as a U.S. Medical Affairs Group Leader in the SCBU with responsibility across a number of disease areas ranging from Inflammation to Transplant to GI. For the past couple of years, Kevin has led the U.S. Medical Affairs efforts in Rare Diseases. As a U.S. Medical Affairs Group Leader in SCBU, he supervised both HQ- and field-based colleagues supporting inline and pipeline compounds in Rare Diseases. At the beginning of 2014, Kevin moved into a new role as the Vice President/Disease Area Lead for Rare Disease in the GIP Global Medical Affairs organization. In this position, he supervises a group of global colleagues providing medical leadership and strategic support for inline and pipeline assets in Endocrinology, Hematology, TTR-Amyloidosis, Gaucher's Disease, and other rare diseases. Kevin received his medical degree from the UCLA School of Medicine and is board-certified in Internal Medicine. Following a two-year fellowship in Health Services Research at UCLA and a brief academic career as an Instructor of Medicine at the UCLA School of Medicine, he spent eight years in private practice caring for HIV positive patients while maintaining an academic appointment at the UCLA School of Medicine as an Assistant Clinical Professor of Medicine. In addition to his medical degree, Kevin has a Masters in Public Health from the UCLA School of Public Health and a JD degree from Harvard Law School.

Michael Skynner PhD
Head of Rare Disease Alliances - Pfizer Rare Disease Research Unit

Michael joined Pfizer in 2013 and is spearheading its rare disease efforts in Europe and heads the Rare Disease Consortium agreement with GMEC. Previously, he founded and headed up a Discovery Performance Unit (DPU) at GSK. This unit developed clinical programmes targeting inflammatory kinases with an emphasis on externalisation and partnership, and successfully progressed molecules from target to phase II.

He co-founded a biotech start-up company, Cambridge Biotechnology Ltd, which developed small molecule drugs into the clinic before exiting via a successful trade sale to Swedish Orphan Biovitrum.

Michael has over 15 years pharmaceutical and 5 years academic experience and obtained his PhD in Biochemistry at Imperial College in 1994.

Pierre Fabre

Company name: **Pierre Fabre**
Company size: **10 000 employees**
Number of countries: **42 subsidiaries**
Annual sales 2014: **€ 2,1 Billion**
R&D Staff: **1500 employees**
R&D investment: **€ 216 million**
Headqueters: **Boulogne, France**

COMPANY DESCRIPTION

The Pierre Fabre Group, the second-largest independent laboratory in France, employs around 10,000 people and achieved sales of 2,1 billion € in 2014. Pharmaceutical laboratory currently held in majority by the Pierre Fabre Foundation, recognized as an organisation of public utility.

Headquartered in the South-West of France, Pierre Fabre has currently branches in 44 countries and distribution agreements in over 130 countries.
Fully integrated company (R&D, Commercial Operations, Manufacturing, Distribution), Pierre Fabre nurtures three complementary activities: Pharmaceuticals (Rx), Healthcare (OTC) and Dermo-Cosmetics. It achieves more than 50% of its business outside France.

The total 2014 R&D budget en 2014 was 210 million € in its three major therapeutic areas:
* Oncology,
* Central Nervous System,
* Dermatology.
Europe's leading cosmetologics laboratory (Avene: leading brand in Europe, China and Japan).
Pioneer in the research and production of plant actives ingredients through the Pierre Fabre Botanical Exertise.

HISTORY OF COMMITMENT

Pharmaceutical laboratory founded in 1961 by Pierre Fabre, French pharmacist in Castres (Tarn).
Our mission: caring for the human being as a whole.
We imagine and develop innovative solutions for enhancing people's well-being from health to beauty care.
We do this with health professionals, our trusted partners worldwide; by drawing perpetual inspiration from nature to plants; and by placing pharmaceutical ethics at the very heart of our operations.
Moreover, Pierre Fabre Group demonstrates a strong experience in developing.

R&D FOCUS PORTFOLIO AND PROSPECTIVE IN THIS AREA

Oncology
Dermatology
Woman Health
Neuropsychiatry

Nicolas GUILBAU
Director, External Research in Oncology

Training and academic background
1990
PAUL SABATIER UNIVERSITY (Toulouse, France),Ph.D. in Cellular Pharmacology.
1991
LABORATORY OF FUNDAMENTAL PHARMACOLOGY and TOXICOLOGY
Post-Doctoral research Fellow in 'Preclinical Evaluation Group of antitumor drugs'

Industry background
INSTITUTE OF RESEARCH PIERRE FABRE (I.R.P.F.)
Toulouse, France

2013 – Present
Director, External Research in Oncology, CRDPF

2006 - 2013
Head of Experimental Oncology Research Center, CRDPF
ONCODESIGN BIOTECHNOLOGY Dijon, France

2002 – 2006
Chief Scientific Officer
INSTITUTE OF RESEARCH SERVIER (IDRS)
Suresnes, France

1997 - 2001
Head of In Vivo Group in Oncology Department
1991 - 1997 Research Scientist, then Senior Scientist in Oncology Dpt (IdRS)

Profile
- 25 years of R & D experience in oncology-based biotechnology / pharmaceutical companies
- Commitment in dozens of research programs in oncology, 4 of them having reached clinical
- Career marked by management of scientific staff and projects:
- Overall responsibility and supervision of groups of more than 50 research scientists and technicians
- Extensive experience in initiating and designing Academic / Biotech / Pharma collaborations and partnerships

- Development and/or supervision of technological platforms: Pharmacology in vitro and in vivo, Animal Imaging, Medicinal Chemistry
- Co-author of 53 articles published in scientific journals, reviewschapters and more than 80 scientific communications presented in international meetings.
- Co-inventor of 12 patents and patent Co-inventor

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Christine CHAUMONT
R&D Project Director

Training and academic background
1999
Degree of Pharmacy (University Paris XI) and PhD in Galenical Research (University Paris VI and CNRS ESA7033)
1994 - 1999
Intern in the public hospitals of Paris: Necker (Medical genetics Pr Munnich, Pharmacy Pr Singlas), Paul Brousse (Pharmacy Dr Fredj), Pitié Salpêtrière (Biochemistry Pr Delattre), Charles Foix (Biochemistry and Hematology, Pr Durand)
1995 - 1999
Master of Pharmaceutical Technology and Biopharmacy (University Paris XI)
1995
Post-graduate degree in statistic applied to medicine (CESAM)

Industry background
2014 - Present
PIERRE FABRE ORPHAN DERMATOLOGY (Toulouse), Managing Director
2005 - Present
PIERRE FABRE DERMATOLOGIE (Toulouse), R&D Project Director
2005 - 2005
AB SCIENCE (Paris), Clinical Project Manager
2000 - 2004
BIOALLIANCE PHARMA (Paris), Project Director and Project Manager

Areas of research interest
Fifteen years experience in project management (from discovery through marketed products) in pharmaceutical industry or biotech.
Management of a full development of a worldwide product with orphan status for dermatology.
Management of clinical trials in orphan pathologies.

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Mail christine.chaumont@pierre-fabre.com

Company name: **Sanofi**
Company size: **110 000 employees**
Number of countries: **42 subsidiaries**
Annual sales 2014: **33,8 Mds €**
R&D Staff: **15 000 employees**
R&D investment: **4,8 Mds €**
Net income: **6,847 Mds €**
Headqueters: **Paris, France**

COMPANY DESCRIPTION

Sanofi, a global and diversified healthcare leader, discovers, develops and distributes therapeutic solutions focused on patients' needs.
Sanofi has core strengths in the field of healthcare with seven growth platforms: diabetes solutions, human vaccines, innovative drugs, consumer healthcare, emerging markets, animal health and the new Genzyme.
Sanofi is listed in Paris (EURONEXT: SAN) and in New York (NYSE: SNY).

PRODUCT PORTFOLIO IN THE AREA

Gaucher's disease- Cerezyme, Cerdelga®,
Pompe's disease- Myozyme®/Lumizyme® (alglucosidase alpha)
Familial hypercholesterolemia- Kynamro, Cholestagel®
Fabry's disease- Fabryzyme (agalsidase bêta)
Mucopolysaccharidose type I- Aldurazyme® (laronidase)
mucopolysaccharidose type II (Hunter's syndrome)-Elaprase®

R&D FOCUS PORTFOLIO AND PROSPECTIVE IN THIS AREA

Hypercholesterolemia- alirocumab (PCSK9 inhibitor).

François BESNARD
External Innovation – Direction Strategy
Science policy & External Innovation

Training and academic background
PhD degrees in Neurobiology and Molecular Biology from Louis Pasteur University (1988)

Industry background
After his PhD, he completed a post doc at the NIH (Bethesda) as a research fellow and then visiting scientist on transcriptional regulation of an astrocyte specific gene GFAP.

He has more than 20 years of experience in pharmaceutical research in a wide range of diseases, including neurodegenerative diseases, obesity and oncology. He published more than 50 papers in peer-reviewed journals.
During his career in industry, since 1992, François held several managerial positions of increasing responsibility in Synthelabo and Sanofi.

Research organisations, where he alternated roles in Biotechnologies : Molecular biology, functional genomics and molecular genomics support to medicinal chemistry programs and in the development of a biopharmaceutical portfolio.

Since 2010 his activity his focused on identification, evaluation and negotiation of external opportunities ; seeking collaborations and compounds in licensing for neurodegenerative diseases.

Areas of research interest
Neurobiology: ion channel ; neurodegenerative diseases ; Alzheimer ; Parkinson.

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Company name: **Shire**
Company size: **Over 5000 Globally**
Number of countries: **68 countries**
Annual sales 2014: **5.8bn**
R&D investment: **$840m Non GAAP R&D**
Headqueters: **5 Riverwalk Citywest Business Campus Dublin 24 Ireland**

COMPANY DESCRIPTION

Shire enables people with life-altering conditions to lead better lives.

Our strategy is to focus on developing and marketing innovative specialty medicines to meet significant unmet patient needs.

We focus on providing treatments in Neuroscience, Rare Diseases, Gastrointestinal, and Internal Medicine and we are developing treatments for symptomatic conditions treated by specialist physicians in other targeted therapeutic areas, such as Opthalmics.

HISTORY OF COMMITMENT

Shire was founded in 1986 in the UK. Since then, Shire has grown revenues from $68 million in 1997 to well over $4.9 billion in 2013, and Non GAAP operating income from $0.5 million in 1997 to over $1.8 billion in 2013. Shire's market capitalisation as of Dec 3, 2014 is $42 billion (£26 billion).

There's a simple purpose that sits at the heart of our business: to enable people with life-altering conditions to lead better lives. This means we focus on developing treatments for conditions where the impact of our medicines can make an immediate and tangible difference for patients. We provide treatments in Neuroscience, Rare Diseases, Gastrointestinal, and Internal Medicine.

This might be a therapy to treat an extremely rare and life-threatening disease like Hunter syndrome or Fabry disease; or a medicine for a specialist condition like ADHD or ulcerative colitis which if not treated effectively, can dramatically affect the lives of the patient and their whole family.

PRODUCT PORTFOLIO IN THE AREA

Shire aims to excel in innovation to meet patients' significant unmet needs. In 2014 we continued to strengthen our pipeline through focused R&D and business development.

R&D FOCUS PORTFOLIO AND PROSPECTIVE IN THIS AREA

The Company has grown both organically and through acquisition, completing a series of major transactions that have brought therapeutic, geographic and pipeline growth and diversification.

The Company will continue to conduct its own research and development, focused on rare diseases, as well as evaluate companies, products and pipeline opportunities that offer a strategic fit and have the potential to deliver value to all of the Company's stakeholders: patients, physicians, policy makers, payers, investors and employees.

The Company will execute on its purpose through its strategy and business model which includes four strategic drivers: Growth, Innovation, efficiency & people, through deep understanding of patients' needs, the Company is able to: serve patients with high unmet needs in selected commercially attractive specialty therapeutic areas; drive optimum performance of its marketed products – to serve patients today; build its pipeline of innovative specialist treatments through both R&D and Corporate Development activities – to enable the Company to serve patients in the future.

During 2014, Shire focused its R&D efforts, investing $840 million to drive pipeline innovation. Pipeline highlights included: Pipeline enhanced through business development with new rare disease investments in Lumena (GI), Fibrotech (Renal), ArmaGen (Hunter syndrome) and BIKAM (Ophthalmics).

Fast track and orphan drug designation granted for SHP607 for the prevention of retinopathy of prematurity ("ROP"). Fast track designation granted for SHP609 for the treatment of Hunter syndrome with Central Nervous System ("CNS") symptoms. Top-line results announced from OPUS2 & SONATA trials for Lifitegrast (SHP606)..

Omar FRANCONE
Head of Discovery Biology and Translational Research Shire Lexington, USA

Training and academic background
Dr Francone received his BA in Biochemistry from the National University of the Littoral, Santa Fe, Argentina, and his PhD from the University of Paris VII, Paris, France. He completed his postdoctoral training at the Cardiovascular Research Institute at the University of California, San Francisco, CA, USA.

Dr Francone is the author of over 60 peer-reviewed scientific publications in this field, including peer-reviewed articles, book chapters and conference papers.

Industry background
Omar Francone has more than 20 years of scientific and leadership experience in drug discovery and development. He is currently Head of Discovery Biology and Translational Research at Shire where he is leading a team of scientists focused on developing novel therapeutics for the treatment of genetic diseases. Prior to joining Shire, Dr Francone held roles of increasing strategic, scientific and leadership responsibilities at Pfizer. While at Pfizer, he led multiple drug discovery programs and delivered several compounds to clinical development, spanning multiple molecular mechanisms, in the area of metabolic disease.

Areas of Research Interest
There's a simple purpose that sits at the heart of our business: to enable people with life-altering conditions to lead better lives. This means we focus on developing treatments for conditions where the impact of our medicines can make an immediate and tangible difference for patients.

We provide treatments in Neuroscience, Rare Diseases, Gastrointestinal, and Internal Medicine. This might be a therapy to treat an extremely rare and life-threatening disease like Hunter syndrome or Fabry disease; or a medicine for a specialist condition like ADHD or ulcerative colitis which if not treated effectively, can dramatically affect the lives of the patient and their whole family.

Our growth strategy is to focus on developing and marketing innovative specialty medicines for symptomatic conditions to meet significant unmet patient needs. Our two strategic priorities are to drive optimum performance of our existing products, enabling access to these medicines for patients today; and building our pipeline through research and development (R&D) and business development (BD), delivering access in the future for patients.

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