

#### **Event report: Cellectis R&D/ Analyst Day**

## **Genome Engineering & CART Cells:** At the forefront of immuno-oncology

September 16, 2014 — Paris (France) — Cellectis (Alternext: ALCLS.PA), a leader in the development of adoptive immunotherapies based on engineered allogeneic CART cells (UCART) has hosted an R&D/ Analyst Day on September 11 in New York City. This meeting was intended to explain shareholders and investors Cellectis' research and development. More than 100 people attended that public event at which Cellectis' scientists provided an overview of the Company's therapeutic innovations with feature presentations by key opinion leaders: Pr. Laurence Cooper, MD Anderson Cancer Center; Dr. Jaume Pons Senior Vice President and CSO of Rinat, the biotech unit of Pfizer and Eric Falcand, Director, Alliance Management & US Licenses at Servier. The meeting started at 8:00 a.m. and ended after a Q&A session at noon.

#### **Summary of the event**

After a welcoming remarks by Dr. André Choulika, Ph.D., Chairman and CEO of Cellectis, the meeting opened with a keynote address by Pr. Laurence Cooper, MD, Ph.D., University of Texas, MD Anderson Cancer Center, who discussed the clinical and economic benefits of immuno-oncology area and demonstrated the apparent benefits of an allogeneic approach to undertake off-the-shelf T-cell therapy for patients.

- Dr. Philippe Duchateau, Ph.D., Chief Scientific Officer, then presented the Company's skills and know-hows, Dr. Julien Valton, Ph.D., Innovation Project Leader, explained how Cellectis has fully matured TALEN<sup>TM</sup> technology that is considered as one of the most precise, efficient and the safest genome editing method today.
- Dr. Laurent Poirot, Ph.D., Head of Early Discovery, highlighted how Cellectis has developed unmatched capabilities for engineering T cells to fit the therapeutics needs of multiple cancer indications and how traits can be built into T cells to overcome tumor immune evasion.
- Dr. Jaume Pons, Ph.D., Senior Vice President and Chief Scientific Officer of Rinat, the biotech unit of Pfizer, described Cellectis and Rinat/Pfizer's partnership as a scientific and cultural match aiming to produce 'Off-the-Shelf' therapies.
- Dr. Alexandre Juillerat, Ph.D., Project Leader, detailed Cellectis' CAR architecture, the multichain CAR (mcCAR), able to exhibit an anti-tumor activity that is indistinguishable from the single chain CAR in vitro and in vivo. He demonstrated that Cellectis mcCAR scaffold extends the engineering possibilities and constitutes a step forward for targeting solid tumors.
- Dr. Julianne Smith, Ph.D., Vice President CART Development, presented Cellectis' first two "off the shelf" T-cell products: UCART19 (partnered with

Servier) and UCART123, from its proprietary platform of adoptive cancer immunotherapy, deployable on a large scale for different oncology indications.

Eric Falcand, Director, Alliance Management & US Licenses at Servier, described Cellectis/Servier's collaboration as a win-win partnership allowing Cellectis to structure itself in various domains and successfully develop products and Servier to access to innovative technologies in line with its strategy to answer unmet medical needs in oncology.

Stéphan Reynier, Eng., MSc., Chief Regulatory and Compliance Officer, detailed Cellectis' CART cell manufacturing platform and explained the Company's CMC (Chemistry, Manufacturing, Controls) and Regulatory strategies for CART cell adoptive immunotherapy.

Dr. Mathieu Simon, MD, Executive Vice President, closed the meeting by giving future perspectives for Cellectis' allogeneic technology. He explained why Cellectis poised to be a major player in immuno-oncology according to its "best in class" science, its hybrid development model, its own product portfolio and its deployment in the USA.

A summary of the presentations given during the R&D Day are available on our website: http://bit.ly/1qcfla9

#### **Biographies**

#### André Choulika, Ph.D., Chairman and Chief Executive Officer

André Choulika is the Chairman, CEO, and founder of Cellectis. Dr Choulika is a pioneer in the analysis and use of meganucleases to modify complex genomes. After receiving his PhD in molecular virology from the University of Paris VI (Pierre et Marie Curie), he completed a research fellowship in the Harvard Medical School Department of Genetics. Later, while working in the Division of Molecular Medicine at Boston Children's Hospital, he developed the first approaches to meganuclease-based human gene therapy.

Dr Choulika also has management training from the HEC (Challenge+).

Guest speaker

# Laurence James Neil Cooper, MD, Ph.D., Professor, Department of Immunology, The University of Texas MD Anderson Cancer Center, Houston, TX

Dr. Laurence J.N. Cooper obtained his M.D. and Ph.D. degrees at Case Western Reserve University in Cleveland and then training in Pediatric Oncology and Bone Marrow Transplantation (BMT) at the Fred Hutchinson cancer Research Center in Seattle. He joined M.D. Anderson Cancer Center in 2006 and currently leads the Pediatric Cell Therapy service (formally named the BMT program). In addition to caring for children, adolescents and young adults undergoing autologous and allogeneic hematopoietic stem-cell transplantation (HSCT), he runs a laboratory translating immunology into clinical practice. His program has multiple investigator-initiated trials that infuse T cells and NK cells to target malignancies. The adoptive transfer of lymphocytes represents the future of HSCT as he and other investigators enhance the potency of the immune system to eliminate residual cancers.

To find out more about M.D. Anderson Cancer Center: <a href="www.mdanderson.org">www.mdanderson.org</a>

#### Philippe Duchateau, Ph.D., Chief Scientific Officer

Philippe Duchateau received his PhD in biochemistry and molecular biology from the University of Lille and the Institut Pasteur. He joined Cellectis in 2001 after nine years at the Cardiovascular Research Institute of the University of California, San Francisco (United States). He previously headed Cellectis' Research department, starting in 2004.

#### Julien Valton, Ph.D., Innovation Project leader

Dr. Julien Valton obtained his Ph.D. degree at Université Joseph Fourier in Grenoble where he was trained as enzymologist. He then joined the Yale School of Medicine to apply his knowledge to therapeutic research, by investigating the mechanism of inhibition of receptor tyrosine kinases involved in cancer development. In 2009, he moved a step further into the field of applied science by joining the R&D Department of Cellectis in Paris, where he actively participated to set, improve and use meganucleases and TALEN™ for targeted gene therapy and genome engineering purposes. He is currently involved in the development of next generations of CAR T cells to improve their safety and efficacy.

#### Laurent Poirot, Ph.D., Head of Early Discovery

Laurent Poirot studied at Ecole Polytechnique (Palaiseau, France) with majors in Physics and Biology. He obtained his PhD in immunology in the laboratory of professors D. Mathis and C. Benoist (Strasbourg, France and Harvard Medical School, Boston, MA) where he studied the molecular and cellular basis of type I diabetes pathogenesis. As a postdoctoral fellow at the Genomics Institute of the Novartis Research Foundation, he developed high throughput approaches to identify new gene functions in the immune system. He joined Cellectis in 2009 and after working on gene editing of hematopoietic stem cells, he optimized TALEN<sup>TM</sup> induced gene inactivation in human T cells. He now leads the research group in charge of early therapeutic discovery at Cellectis.

#### Guest speaker

### Jaume Pons, Ph.D., Senior Vice President and Chief Scientific Officer of Rinat, the biotech unit of Pfizer

Jaume Pons, Ph.D., is Senior Vice President and Chief Scientific Officer of Rinat, the biotech unit of Pfizer in South San Francisco, California. In this role, he is responsible for portfolio delivery from idea to clinical development, up to human proof of concept. Dr. Pons also serves as Chief Technology Officer, focused on antibody technologies, and is a member of the Pfizer Worldwide Research and Development leadership team.

Previously, Dr. Pons created and led Rinat's protein engineering group, and is an inventor of several antibodies that are now in late-stage clinical development, including RN624 (tanezumab) and RN316 (bococizumab). Under his direction, Rinat continues to advance Pfizer's growing capabilities in generating therapeutic antibodies, and has progressed eight antibodies into the clinic.

Dr. Pons earned his Ph.D. in molecular and cell biology at the Institute on Fundamental Biology, Barcelona, Spain, and his B.S. in biochemistry from Autonoma University of Barcelona. He conducted his postdoctoral studies in antibody engineering at the University of California, Berkeley.

To find out more about Pfizer: www.pfizer.com

#### Alexandre Juillerat, Ph.D., Project Leader

Alexandre Juillerat Ph.D. is a gradute in chemistry of the University of Lausanne, Switzerland. After receiving his PhD in protein engineering from the École polytechnique fédérale de Lausanne (EPFL, Switzerland) he joined the laboratory of Structural Immunology at the Institut Pasteur in Paris, France. He then joined Cellectis in 2010 as project leader for the R&D department.

#### Julianne Smith Ph.D., Vice President, CART Development

Julianne Smith Ph.D. joined Cellectis in 2002 and has been involved in the therapeutic division since its inception. She is currently VP of CART development and leads the research team working on CAR design and initial process development for UCART19 as well as new leads in both liquid and solid tumors. Dr. Smith has a strong background in DNA repair and recombination with a Ph.D.

in Genetics and Development from Columbia University and a B.A. in Biology from Johns Hopkins University.

#### Guest speaker

#### Eric Falcand, Director, Alliance Management & US Licenses at Servier

Dr Eric M. Falcand has been working within the Pharmaceutical Industry for more than 25 years. Doctor in Veterinary Medicine and holder of a DESS in Pharmacy and an MBA from EM Lyon, he quickly held marketing and sales management positions in various countries for Delagrange, then Synthelabo (now Sanofi). He joined Servier to become General Manager in Russia and then CEO in the UK for many years. In charge of US partnerships and Licenses at Head Office in Paris, then of out-licensing activities within the Business Development team, he contributed to craft important deals with key players of the Industry. In addition, he was asked to build the Alliance Management Group in 2012 with the objective to create this new function with a team of Alliance Managers. In that capacity, he got closely involved with the Cellectis deal. His strong management experiences in various cultural environments and his in-depth understanding of the Servier culture have contributed to consolidate the company's recent focus on external partnerships.

To find out more about Servier: www.servier.com

#### Stéphan Reynier, Eng., MSc., Chief Regulatory and Compliance Officer

Stéphan Reynier joined Cellectis in 2011 in charge of Regulatory Affairs and Compliance after some years of experience as Director at Voisin Consulting, a consulting firm specialized in supporting small and medium size enterprises in the development and the placing on the market of Cell and Gene Innovative Medicines. Prior work experiences involved Clinical Development and Medical Affairs at Gilead Sciences and Parexel Int.

#### Mathieu Simon, MD, Executive Vice President

After graduating from medical school at the University of Paris in 1982, Dr Mathieu Simon embarked upon an illustrious international career in the pharmaceutical sector. After serving as Director of Marketing and Sales at Wyeth France, he became Group Vice President of Marketing and Clinical Affairs for Wyeth Pharmaceuticals in the United States, and later led several of the Wyeth Group's biggest regional subsidiaries in the Benelux countries, Italy, Greece, and the Balkans. In 2010, Dr Simon was named Senior Vice President of Pharma Global Operations at Pierre Fabre Médicament. He joined the Cellectis Group in 2012.

#### **About Cellectis**

Cellectis is a biopharmaceutical company focused on oncology. The company's mission is to develop a novel generation of therapy based on engineered T-cells to treat cancer. Cellectis capitalizes on its 14 years of expertise in genome engineering, based on TALEN™, meganucleases and the state-of-the-art electroporation technology Pulse Agile, to create a new generation of cancer immunotherapy for treating leukemias and solid tumors. Cellectis adoptive cancer immunotherapy for chronic and acute leukemias is based on the first allogeneic T-cell chimeric antigen receptor (CAR) technology. CAR technologies are designed to target surface antigens expressed on cells. These treatments reduce toxicities associated with current chemotherapeutics and have the potential for curative therapy. The Cellectis Group is focused on life sciences and uses leading genome engineering technologies to build innovative products in various fields and markets. Cellectis is listed on the NYSE Alternext market (ticker: ALCLS). To find out more about us, visit our website: www.cellectis.com.

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