Precision Medicine & New Technologies: Transforming Clinical Research
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Precision Medicine & New Technologies: Transforming Clinical Research

“Tonight, I’m launching a new Precision Medicine Initiative to bring us closer to curing diseases like cancer and diabetes — and to give all of us access to the personalized information we need to keep ourselves and our families healthier.”

President Barack Obama, State of the Union Address, January 20, 2015

While Precision Medicine has been heralded as the foundation for the next decades’ progress in Global Health, we must address several challenges before the vision can be realized and the real benefits delivered to patients.

• How can we harness newly available technologies to improve the endpoints used for evaluating clinical efficacy? Can this help us manage the rising cost for new therapies?
• How do we fully integrate new approaches in Big Data to health care delivery and how do we balance patient privacy with the need for data sharing and continued research?
• What will be the cost of Precision Medicine for the different actors of the health industry and for the patients?
• How will we face the anticipated high costs of new treatments? How will we ensure the continued research and development required to ameliorate rare diseases therapies? Who will pay the price of such development?

Organizers

Office of Science and Technology of the Embassy of France in the United States: with offices throughout the United States, the Office for Science and Technology’s mission is to promote bilateral partnerships in science, technology and innovation. It has been organizing the FAID conference in Boston since 2002.

GLOBAL CARE Initiative is a unique consortium of five non-profit French research Carnot Institutes, all of them international research and innovation leaders in the human health field. The consortium operates as a one-stop-shop for life science companies and research organizations that seek R&D collaborations from basic research to the late clinical phases. The consortium has extensive experience of partnerships with major pharmaceutical, biotech, diagnostic and medtech companies.
Organizers

The CALYM* Carnot Institute brings together 13 entities specialized in lymphoma research, the most frequent blood cancer: 11 high-level public research labs plus LYSA and LYSARC, two non-profit academic research organizations. Its objective is to accelerate innovation and its transfer in the lymphoma field through a reinforced public-private partnership and a unique R&D offering from identification of new cellular targets to international phase III registration trials.

The Carnot Institute ICM** is a private Neuroscience research Institute, the largest of its kind, dedicated to developing new healthcare solutions for major brain diseases. Based in the fantastic environment of Paris Pitie-Salpetriere hospital, with its 25 research teams, +500 researchers and clinicians and 80 clinical trials ongoing, ICM actively develops and validates new preclinical and clinical tools for optimized drug discovery. Home for +20 start-up companies, 5 of which at clinical stage, ICM also primes integrated programs at the crossroads of biotech, medtech and digital healthcare.

The Carnot Institute Curie Cancer is built on the expertise of Institut Curie (with 3400 people), a leading comprehensive cancer center in Europe and a world-renowned hospital. Curie Cancer offers to industrial partners the possibility to set-up research collaborations from early discovery (cognitive research) to most advanced research (clinical research).

The Carnot Institute of Institut Pasteur promotes ambitious academic-industrial partnerships with biotechnology and healthcare industries. Through a better understanding of the fundamental mechanisms of host/pathogen interactions, the institute wishes to foster the development of innovative products in collaboration with industrial partners in 3 areas: therapeutic, diagnostic and vaccine.

The «Voir & Entendre» Carnot Institute represents one of the most important international centers in neurosensory research as it brings together the ‘Institut de la Vision’, a research unit on Genetics and Physiology of Hearing, and the Clinical Investigation Center of the ‘Quinze-Vingts’ National Eye Hospital. This institute is designed as a place of exchange gathering on the same site patients, clinicians, researchers and industrials in order to accelerate innovation in terms of health products and high technology, while delivering solutions to unmet needs of people impaired by pathologies and handicaps affecting visual and hearing systems.

* Consortium for the acceleration of innovation and its transfer to the field of Lymphoma
** Institut du Cerveau et de la Moelle Epiniere - «Brain and Spine Institute»
Wilson Sonsini Goodrich & Rosati
Wilson Sonsini Goodrich & Rosati has developed a broad expertise in the field of life sciences. The firm’s life sciences practice includes representation of more than 500 companies in biotechnology, biopharmaceuticals, drug discovery technology, genomics, medical devices, diagnostics, and health care services.
www.wsgr.com

With the kind support of

Association Instituts Carnots
The “Carnot” certification is granted to public research structures—Carnot institutes—that concurrently conduct upstream research and an ambitious partnership research policy to the benefit of the industrial world.
The Carnot institutes network gathers 34 major French research structures dedicated to fostering companies’ innovation in various fields, from the automotive and nuclear industries to green energies, telecom and computer science. Among these 34 Carnot institutes, five are devoted to the human health sector: CALYM, Curie Cancer, ICM, Pasteur MI, and Voir et Entendre.
The Carnot institutes generate 50% of the total revenue of academic-industry partnerships in France and are supported by the French Government to develop a quality-guided approach for a innovative applied research with companies.
www.instituts-carnot.eu/en
The Massachusetts Life Sciences Center
The Massachusetts Life Sciences Center (MLSC) is a quasi-public agency of the Commonwealth of Massachusetts tasked with implementing the Massachusetts Life Sciences Act, a 10-year, $1-billion initiative that was signed into law in June of 2008. The MLSC’s mission is to create jobs in the life sciences – biotechnology, pharmaceuticals, medical devices, diagnostics and bioinformatics — and support vital scientific research that will improve the human condition.
www.masslifesciences.com

ANR
Agence Nationale de la Recherche (ANR) provides funding for project-based research in all fields of science - for both basic and applied research - to public research organisations and universities, as well as to private companies (including SMEs). Employing a method based on competitive peer reviews compliant with international standards, ANR provides the scientific community with instruments and programmes promoting creativity and openness, and stimulate new ideas and partnerships, particularly between academia and industry. Its activity also contributes to enhancing the competitiveness and the influence of French research in Europe and across the world.
www.agence-nationale-recherche.fr/en

Lyonbiopole
Lyonbiopole is the one-stop shop for healthcare innovation in Rhône-Alpes. It aims to support the emergence and development of innovative technologies, products and services for a more personalized medicine to the patient’s benefit.
www.lyonbiopole.com

Medicen
The Medicen Paris Region global competitiveness cluster aims to position the Paris Region as a European industrial leader in diagnostic and therapeutic innovation and leading-edge health technologies, thus enabling it to become one of the global centres for translational medicine.
www.medicen.org
Program

8:00 am **REGISTRATION & BREAKFAST**

9:00 am **Welcome Address**
- His Excellency Gérard Araud, Ambassador of France to the United States

9:15 am **Panorama of Precision Medicine in Massachusetts**
- Travis A. McCready, JD, President & CEO, Massachusetts Life Sciences Center

9:30 am **Carnot Institutes: Research & Innovation for Industry and RTOs**
- Pascal Deschaseaux, MD, MBA, General Manager, CALYM & VP, Association des Instituts Carnot

Carnot Institutes are a major French State-funded multi-disciplinary research network, building economic development through technologies and innovation. A Carnot Institute is a leading research organization, which places partnering research, i.e. research led with and for companies, at the heart of its strategy. The 34 Carnot Institutes represent together 15% of the French public laboratory workforce (27,000 research professionals) and more than 55% of France’s public research directly funded by the industry. The Carnot institutes collaborate with other Research and Technological Organizations (RTOs) internationally.

GLOBAL CARE Initiative, the co-organizer of the FAID 2016, is the consortium of the 5 human health-centered Carnot Institutes. It comprises the Carnot Institutes of Institut Pasteur (infectious diseases), of Institut Curie (cancers) and of ICM (CNS diseases), Voir & Entendre (vision and audition diseases and rehabilitation) and CALYM (lymphoma).

**SESSION I - BIG DATA IN CLINICAL TRIALS: FROM INTEGRATED PATIENTS PROFILES TO COMPLEX BIOMARKERS**

9:45 am **A Multidimensional Contribution to Personalized Medicine in Lymphoma**
- Gilles Salles, MD, PhD, Chairman, CALYM

Lymphoma represents a heterogeneous group of malignant diseases originating from blood cells called lymphocytes. Although about half of lymphoma patients can be cured, there is a considerable variability in the success rate of therapy. Prognostic indexes developed in the 90’s were based on several clinical parameters and validated on cohorts of thousands of patients, thanks to the international collaboration of lymphoma experts. In the last 15 years, new biological tools and large scale genomic approaches allowed deciphering the intrinsic biological diversity of lymphoma tumors. Many biomarkers were identified and new stratifications models derived using a combination of those with clinical parameters emerged. Longitudinal follow-up of blood derived biomarkers were also able to predict the efficacy of therapies applied to patients. The inherited host genetic profile was also found to influence patients’ outcome. Meanwhile, the efficacy of therapy could be monitored with new imaging techniques. The integration of all these data represents today a real challenge addressed through different research programs conducted by LYSA, The Lymphoma Study Association. They will contribute to the development of personalized medicine and may help to design in silico models aiming to predict the clinical efficacy of a given drug, hence contributing to a faster drug development.

- Michel Meignan, MD, PhD, Professor of Nuclear Medicine, Henri Mondor University Hospital

The imaging activities of the LYSA started in 2007 when we launched with the EORTC a cooperative trial based on the results of a centralized review of PET/CT performed under treatment to manage therapy. We built a system of online centralized review using Workstations settled in the main LYSA centers linked through a virtual private network. This
allowed the results of a centralized review performed by 6 experts to be obtained within 72 hours. Imaging performed under treatment (interim PET) being more and more used to guide therapy in our subsequent trials and in many trials worldwide we moved towards a web based solution which allows each reviewer to report the images on its personal computer without uploading them. A database was progressively acquired including now more than 7000 examinations of patients with various subtypes of lymphoma obtained before and after 2 or 4 cycles of treatment. From these data we were able to use imaging as a biomarker to predict outcome and to define new therapeutic strategy. We have defined new criteria of PET reporting (Deauville criteria) and new quantitative parameters of the response such as the ΔSUV. We now develop quantitative index from PET performed before therapy to evaluate the total metabolically active tumor burden. The idea is to stratify patients in different risk categories to tailor therapy according to the risk and to adjust the dosage of the drugs to the mass of the tumor. Encouraging results have been obtained from our group in different types of lymphoma. We have shown that the stratification given by the tumor volume can be refined with clinical or molecular data. The challenge is now to link imaging clinical and biological data in our data base to define new prognostic models and to optimize the new trials.

• Fabrice Jardin, MD, PhD, Director, INSERM U918 Research Team, Centre Henri Becquerel

Lymphomas constitute a highly heterogeneous group of hematological malignancies that has been partially deciphered in the last past decades by new molecular biology tools, including Gene Expression Profiles (GEP) and more recently Next Generation Sequencing (NGS). In most cases, the treatment is based on the combination of chemotherapy and immunotherapy (namely the worldwide standard “R-CHOP” regimen) that cures approximately 60% of the patients. In this context, LYSA (The Lymphoma Study Association) currently promotes several biological tests with the aim of tailoring therapies and favoring the emergence of the “post R-CHOP” era. For this purpose, new molecular tools that can be widely used in routine or to stratify patients enrolled in clinical trials have been developed by LYSA. This includes the first NGS study of a large cohort of diffuse large B-cell lymphoma (the main lymphoma subtype) patients using a targeted gene panel (lymphopanel) and benchtop sequencers, focusing on genes identified as important for lymphomagenesis or whose potential has been pinpointed in Whole Exome Sequencing studies. Particular attention has been paid to the inclusion of actionable targets, highlighting potential candidate patients for novel personalized therapies. The “lymphopanel” has been also used to detect actionable mutations in cell-free circulating DNA in the setting of liquid biopsy. In addition to recurrent mutations detected by targeted NGS, GEP molecular tests that are likely to represent theranostic biomarkers in a highly competitive academic and non-academic context, have been developed by the LYSA group.

• Peter Ho, MD, PhD, CMO, Epizyme Inc

The histone methyl transferase enhancer of zeste-homolog 2 (EZH2) is the catalytic subunit of the polycomb repressive complex 2 (PRC2) and responsible for methylation of lysine 27 of histone H3 (H3K27), which results in chromatin remodeling and repressed transcription when trimethylated (H3K27me3). Aberrant EZH2 activity has been implicated as an oncogenic driver in non-Hodgkin lymphoma (NHL). The SWI/SNF complex also remodels chromatin, activates transcription and acts in opposition to PRC2. Oncogenesis from mutation and/or loss of the SWI/SNF subunit INI1 in cancers such as malignant rhabdoid tumor (MRT) is sensitive to EZH2 inhibition. Tazemetostat is a potent, selective small molecule inhibitor of EZH2 that is in phase 2 clinical development in patients with NHL and advanced solid tumors (ST) that are INI1- or SMARCA4-negative. Epizyme has collaborated with LYSA on a phase 1 open-label first-in-human study to evaluate the safety, tolerability, and preliminary efficacy of tazemetostat administered orally as a monotherapy twice a day (BID). Eligible patients had either a relapsed/refractory B-cell NHL or ST. As of 7-Nov. 2015, 58 patients were enrolled to this trial. The recommended phase 2 dose was determined to be 800 mg BID. Objective responses (CRs, PRs) were observed in pts with diffuse large B-cell lymphoma, follicular lymphoma, marginal zone lymphoma, MRT, epithelioid sarcoma and MRT of ovary. Tazemetostat has demonstrated a safety profile favorable for chronic dosing and objective responses in pts with relapsed or refractory B-cell NHL including DLBCL, FL and MZL and in subjects with advanced STs consisting of MRT, ES, and MRT of ovary. Phase 2 trials in B-cell NHL and INI1- or SMARCA4-negative tumors are enrolling.

10:25 am COFFEE BREAK & NETWORKING
10:55 am **Imaging as Analysis Tool and Biomarker**  
**Pierre Tervé, VP Technic & Scientific Advisor, Keosys**

Keosys was founded in 2001 and quickly focused on PET imaging analysis devices. Keosys then extended its national exchange network and built the first Pan-European “near real-time” centralized review system for PET/CT imaging in 2007 with the Lymphoma Study Association (LYSA) for an EORTC trial. As an addition to imaging analysis products this led Keosys to design the “IMAGYS” web platform as a CTIMS (Clinical Trial Imaging Management System). This platform typically helps to handle two challenging issues of any international multi-centric imaging trial: imaging de-identification and quality check at investigator sites and consistent imaging biomarkers analysis. Specifically, for PET imaging, acquisition protocol enforcement and proper de-identification are of utmost importance in order to get usable SUV values. Patient weight and uptake time are typically two crucial parameters. It is also crucial to use validated tools to get proper information from biomarkers like ΔSUV or MRI apparent diffusion coefficient (ADC). But collecting a high volume of imaging only data is not sufficient to find new imaging biomarkers and realize value from imaging “big data”. Like all other small data, aggregation with other data, for example biobanks or clinical information, is needed to extract data and furthermore extract knowledge. Through a network of supporting partners at imaging sites and data analysis & modeling experts, Keosys’ VICTORIA imaging big data project aims at getting knowledge from data based on an ethical model.

11:05 am **Keynote Address**  
**Christopher Larkin, CTO, GE Healthcare**

GE is becoming GE Digital, and the transformation across industries has begun. The GE HealthCare presentation will feature a scan of technology strategies that GE is highlighting as the Industrial Internet for healthcare. This will include a discussion of the “digital twin” predictive analytics and asset performance solutions, as well as a look forward into Precision Medicine. GE and GE Healthcare are investing significantly in cloud-based technologies, and this presentation will outline that strategy.

11:25 am **A Virtual Laboratory for Image-based Neuroscience Discovery: Collaborative Research and Science Education in the Era of “Big Data”**  
**Charles R.G. Guttmann, MD, Director, Center for Neurological Imaging, Brigham & Women’s Hospital, Associate Professor of Radiology, Harvard Medical School**

Digital medical imaging has become ubiquitous throughout the world. Every day, millions of patients are imaged for many clinical reasons, using modalities such as magnetic resonance imaging (MRI), computed tomography (CT), positron emission tomography (PET), digital mammography and digital X-ray systems. While automated image analysis tools continue to be developed, extracting quantitative information from complex biomedical images often remains a labor-intensive task for human experts. So while our enormous and growing archives of biomedical image data play roles in the care of individual patients, this resource has been barely touched for more ambitious research and data-mining purposes. We will present a web-based “virtual laboratory” that integrates project-specific data federation, user-friendly design and execution of experiments, as well as educational modules to train and certify human experts. SPINE (Structured Planning and Implementation of New Explorations) aims to accelerate research by integrating image-derived metrics with other biomarkers, intimately meshing research and education, and fostering the development of more effective and efficient image analysis approaches.

11:35 am **Panel I - Big Data & Imaging**  
**Chair:** Charles R.G. Guttmann  
Michel Meignan / Pierre Tervé / Christopher Larkin

12:15 pm **Pitch Session**  
Quick-fire start-up pitches  
**Moderator:** Brittany McDonough, Senior Manager of Business Development, MOITI (Massachusetts Office of International Trade and Investment)
In oncology, genomics has practically entered the clinic with the emergence of molecularly targeted agents (MTAs). Some of these drugs have been approved with a companion diagnostic based on a specific genomic molecular alteration. However, MTAs have followed the same clinical development as chemotherapeutic agents and have been developed in selected tumor types. The occurrence of the same molecular alteration across various tumor types is well described. The latter raises the question of whether treatment decision in the future should be entirely based on molecular biology, independently of tumor location and histology. This approach can today be addressed in clinical trials, since major advances in high throughput technologies now make it possible to depict most druggable molecular alterations for an affordable cost in a time that is compatible with clinical practice. Studies using high-throughput technologies have been initiated with the aim of personalizing therapy in oncology. I will present the different types of next generation of clinical trials in oncology.

Immunotherapy represents one of the most significant therapeutic advances in cancer in the past 10 years; in particular, immune checkpoint blockade arguably represents a milestone in modern medicine and will likely become a major treatment option for cancer patients, with increased long-term efficacy at reduced toxicity. Other types of immunotherapies, including adoptive cell therapy, bi-specific antibodies, and vaccines are being tested in early-phase clinical trials in solid tumors. Yet, immunotherapy only works well in a fraction of treated patients. The goal of the Immunotherapy Unit of the Institut Curie is to deliver rational therapeutic approaches to increase clinical benefit by bridging knowledge and resources from the Clinical Investigation Unit, the biobank, and the state-of-the-art technological platforms in immune-monitoring, mouse modeling, experimental pathology, and genetics. I will discuss how the integration of clinical and laboratory data could be integrated to inform the design of rational immunotherapies.

Patient stratification and the choice of efficacious therapeutic strategies require the use of biomarkers that can distinguish between disease pathologies. This requires the acquisition of biospecimens from the patient, preferably in a non-invasive manner, and the identification and validation of biomarkers from cases and controls. In this talk, I will present progress on our studies to identify and validate RNA-based biomarkers from non-invasively collected human urine. These studies are aimed at distinguishing between proliferative, smooth muscle dysfunction, and fibrosis as pathobiologies contributing to lower urinary tract dysfunction (LUTD) in aging men. The identification of biomarkers that can distinguish between these pathobiologies will permit patient and therapeutic stratification to more effectively treat LUTD.
### 3:35 pm Panel II - Precision Medicine & Patient Interests
**Chair:** Jill A. Macoska
Christophe Le Tourneau / Don Pettini / Michele K. Russell-Einhor, *Senior Director, Office for Human Research Studies, Dana-Farber Cancer Institute*

### 4:15 pm COFFEE BREAK & NETWORKING

### 4:45 pm Global Network Surveillance of Antimicrobial Resistance
- **Arnaud Fontanet, MD, PhD, Director, Centre for Global Health, Institut Pasteur**

**Context:** While global antibiotic consumption continues to rise, the selection of increasingly resistant strains of bacteria and other pathogens continues to evolve. The problem of antimicrobial resistance is only exacerbated by a stall in antimicrobial innovation and discovery. Unaddressed, antimicrobial resistance has the potential to reverse over 70 years of medical advancement, with large economic and social costs.

**Project:** The Institut Pasteur is launching a Global Network for Surveillance of Antimicrobial Resistance that will draw on the existing regional surveillance activities of the Institut Pasteur International Network, particularly in areas with the largest burden of infectious diseases and potential for antimicrobial resistance development. This project will establish a global subnetwork of resource hubs that will collect microbiological samples with relevant epidemiological and clinical data, determine antimicrobial resistance profiles through phenotypic and genotypic analyses, store samples in secured biobanks, and share sequencing and accompanying data through a high performance computing facility for joint analyses on pathogens of public health concern: bacteria, HIV, P. falciparum and M. tuberculosis.

**Expected outcome:** The use of the latest ‘-omic’ technologies and pathogen diagnostics will provide a comprehensive, quantitative estimate of the emergence and spread of antimicrobial resistance around the world in order to inform and improve future public health measures.

### 5:00 pm CLOSING REMARKS
8:00 am **REGISTRATION & BREAKFAST**

**SESSION II - FROM INTEGRATED BUSINESS MODELS TO NEW FUNDING STRATEGIES FOR EARLY STAGE R&D**

8:30 am **Keynote Address - The Institut Pasteur, a New Innovation Model**  
- Christian Bréchot, MD, PhD, *President*, Institut Pasteur

The Institut Pasteur is an international research institute comprised of 33 Institutes across 26 countries. The academic institutions support public/private research programs, with industrial research and development contracts that leverage our research expertise and provide royalty payments. Additional support is obtained through government contributions, public gifts, donations and revenues from assets. To ensure its sustainability, the Institut Pasteur is establishing a new innovation model, which will enrich the Institut Pasteur international network (IPIN), and help to reinforce ties with Institut Pasteur, Paris. Grounded in its strong tradition of curiosity-driven scientific research, the integration of this new incentive scientific programs will help modernize the institutes approach to research funding and help to drive what is an ambitious program to recruit talented scientists with attractive packages, nurture individual career tracks and a strengthen administrative support for our researchers, worldwide. The implementation of this innovation model rests on the creation of a Department of Development and Grant office and the reinforcement of the European and international environment of the Research Applications and Industrial Partnerships Department.

8:50 am **Short Allocution**  
- Robert G. Urban, PhD, *Head*, Johnson & Johnson Innovation Center, Boston

9:05 am **Harrington Project**  
- Jonathan Stamler, MD, *Director*, Harrington Discovery Institute

The Harrington Project for Discovery & Development is a $250 million US and UK initiative to accelerate the development of medical breakthroughs by physician-scientists into medicines that benefit patients. It is a unique model that aligns, through mission and structure, non-profit and for-profit resources into a new system for drug development. The Harrington Project addresses a set of major challenges in medicine that have created a development gap for promising discoveries and contributed to a long-term decline in new medicine approvals. Launched in 2012, The Harrington Project has already demonstrated its innovative model from the sourcing and development of breakthrough technologies to establishing strategic partnerships with disease foundations and pharmaceutical companies, all with a focus of advancing new medicines for patient benefit.

9:20 am **From Academe to Startup: The Journey of an Entrepreneur**  
- Stephanie Marrus, MBA, *Director*, Eutrepreneurship Center, University of California, San Francisco

9:35 am **Panel III - New Business Models of Innovation**  
- Chair: Johannes Fruehauf, MD, PhD, *Founder & President*, LabCentral  
  Christian Bréchot / Robert G. Urban / Jonathan Stamler / Stephanie Marrus

10:15 am **COFFEE BREAK & NETWORKING**

10:35 am **Success Story: A Relevant Model for Academia-Industry R&D Partnerships**  
- Gilles Salles, MD, PhD, *Chairman*, CALYM

CALYM, one of the 34 France’s Carnot institutes, is the consortium for the acceleration of innovation and its transfer in the lymphoma field. It brings together 13 research entities (the LYSA and LYSARC non-profit research organizations and 11 public high level research teams) specializing in lymphoma, most frequent blood cancer. Thanks to a unique...
R&D offering, CALYM elaborates and runs many preclinical and clinical programs in collaboration with pharma, biotech, imaging and in vitro diagnostics companies to bring answers to unmet medical needs in lymphoma, from the identification of new cellular targets to international phase III registration trials. CALYM’s own research programs are organized through four pillars:

(i) validation of new lymphoma biological targets and in vitro/in vivo models
(ii) identification, validation, protection and out-licensing of lymphoma biomarkers
(iii) acceleration of translational research through the identification of early pharmacodynamic signals of activity
(iv) acceleration of development, registration, and commercialization of drug candidates through the optimization of tools, processes and platforms related to clinical research.

The consortium has also developed a unique collection of cryopreserved living cells for the screening of new lymphoma therapies. Overall, CALYM offers the industry an opportunity of one-stop-shop access to a wide range of expertise, tools and platforms for their research and development strategies in lymphoma.

• Kenichi Takeshita, MD, VP Clinical R&D, Celgene Corporation

Developing new therapies for cancer patients requires strong collaboration between academia and industry. The fundamental basis for drug development starts with discoveries based on disease pathogenesis, typically made in academia. Industry partner brings its expertise in taking new experimental therapeutic agents to the clinical trial stage. Successful development of drugs to registration and approval requires continued industry-academia collaboration with each party bringing expertise to the collaboration. Celgene and LYSA / CALYM collaborations have resulted in accomplishments in many aspects of lymphoma, from translational research projects to clinical trials and surrogate endpoints.

10:55 am Success Story: Biological Integrated Approaches for Drug Development

• Keith A. Hoffmaster, PhD, Director, Global Program Management, Translational Clinical Oncology, Novartis Institutes for Biomedical Research

• Sergio Roman-Roman, PharmD, PhD, Head of Translational Research, Institut Curie

Robust preclinical data are mandatory to optimize early clinical trials and reduce the dramatic attrition rate in late clinical protocols in the field of Oncology. Institut Curie has developed a recognized know-how in preclinical oncology taking advantage of (i) the access to human samples of our Hospitals, (ii) the multidisciplinary skills of the Research Center and our clinicians, (iii) the access to state-of-the-art technological platforms, (iv) the expertise in cancer animal models and pharmacology, and (v) the capacity to identify biomarkers for patient stratification or resistance to therapies. Institut Curie has implemented years ago a Translational Research Department gathering platforms, expertise and disease-devoted teams with the objective of helping scientists from both Academia and Industry to ensure the continuum cancer research/patient care at different steps. Very-well recognized by the quality of its basic research, Institut Curie has devoted a big effort in the last years to incentivize translational research and promote clinical research, and more specifically early clinical trials. We would like to illustrate the way Institut Curie interacts with industrial partners with a successful research program established with Novartis on a rare disease, uveal melanoma. This project has allowed in vitro and in vivo proof of concept studies extremely useful for setting up an international early clinical trial which will be coordinated by Institut Curie. Furthermore, promising drug combinations have been identified which will pave the way for new therapeutic strategies in this dismal disease.
SESSION III - RARE DISEASES: MODELS AND OPPORTUNITIES

11:15 am Short Allocutions & Panel IV - Rare Diseases: Models and Opportunities

More than six thousand different types of rare diseases and disorders have been described, with more being discovered every day. In recent years, the interest for rare diseases has grown throughout the entire drug development community, fueled by the appetite for shorter clinical development timelines, supportive IP regulation and favorable regulatory environment enjoyed in the field: As a result, in 2014, the Food and Drug Administration approved a record 17 medicines in the field, while nearly 500 others are in development. Yet there is more to add to this excitement: drugs for rare diseases often benefit from the strong financial involvement of patient-advocacy groups as well as from extensive registries of patients favoring the fast recruitment for drug studies, an area in which France has long been staying forefront. Last and not least, these drugs have substantial potential to become symptomatic treatments in other, more frequent diseases. This session will focus on the field of CNS and ophthalmic disorders, where some well-chosen rare diseases display a phenotypic richness such that they offer a unique opportunity to develop “1st in man” protocols able to deeply inform the design of future POC trials in other indications. Using the examples of Tourette syndrome, neuro-metabolic disorders and several others, panelists will discuss why and how the gate of rare CNS diseases can open large avenues of development for biotech and pharma groups.

• Yves Agid, MD, PhD, Founder & Scientific Advisor, ICM
• Gerald F. Cox, MD, PhD, VP Rare Diseases Clinical Development, Sanofi Genzyme
• Fanny Mochel, MD, PhD, Head, Metabolic Research Group, ICM
• José-Alain Sahel, MD, Founder & Director, Institut de la Vision
• Michel Vellard, PhD, VP Research, Ultragenyx

12:45 pm Pitch Session
Quick-fire start-up pitches
• Moderator: Brittany McDonough, Senior Manager of Business Development, MOITI (Massachusetts Office of International Trade and Investment)

1:00 pm LUNCH & NETWORKING

2:30 pm Moderna: Pioneering Clinical Messenger RNA Therapeutics
• Stéphane Bancel, MBA, CEO, Moderna Therapeutics

3:00 pm COFFEE & NETWORKING

SESSION IV - TRANSFORMING CLINICAL RESEARCH: NEW END-POINTS AND REGULATORY RENEWAL

3:30 pm Short Allocutions & Panel V - Transforming Clinical Research: New End-Points and Regulatory Renewal

Progress in understanding retinal biology in health and disease moved first therapies to cure blindness from bench to bedside. Spectacular technological innovations allowed hopes for successful partial vision restoration in blind people through gene and cell therapies and retinal prosthesis. The development of these emergent treatments typically involves evaluation of their efficacy in clinical trials. Assessment of the clinical benefit of these new therapies however remains challenging, particularly in the view of lack of standard therapies for blindness that could be used for comparison. Accordingly, new clinical trial endpoints and regulatory approaches for approval of these new drugs, biologics and devices are needed. Ideally, in addition to the efficacy endpoints (eg visual acuity), quantitative measurements of quality-of-life and other socioeconomic parameters should be included in the battery of clinically
meaningful endpoints. Standardized tests for daily living task performance, orientation and mobility in lighted and dark environments can detect efficacy of the treatment in sensitive and reliable manner. Breakthrough high-resolution non-invasive imaging could not only serve for selection of patients for emerging therapies trials but can also be used to optimize the ratio between potential hazards and expected functional benefits of the treatment.

• José-Alain Sahel, MD, Founder & Director, Institut de la Vision
• Yves Agid, MD, PhD, Founder & Scientific Advisor, ICM
• Daniel Chung, DO, MA, Medical Affair Ophthalmic Lead, Spark Therapeutics
• Malvina B. Eydelman, MD, Director, Division of Ophthalmic and Ear, Nose and Throat Devices, FDA
• Newton Howard, PhD, Chairman, Brain Sciences Foundation

5:00 pm Poster Awards & Concluding Address
Emerging pathogen outbreaks: Finding a balance between innovation and patient access

• Nahid Bhadelia, MD, MA, Infectious Diseases Physician, Boston University School of Medicine, Director of Infection Control and Medical Response, National Emerging Infectious Diseases Laboratories

The emergence of new infectious pathogens, particularly in resource limited settings, raises unique challenges such as bioethics of performing research during humanitarian crises, innovating in an environment where standard of care is shifting and providing access to care for the largest possible number of people. How can precision medicine aid in these challenges? Where does it fall short?

5:15 pm CONFERENCE FAREWELL & NETWORKING BUFFET
Speakers

YVES AGID
MD, PhD, Founder & Scientific Advisor, ICM
Yves Agid is Professor Emeritus of Neurology and Neurosciences, member of the Academy of Sciences, and of the National Ethical Committee. He is the founder of the ICM: “Institut du Cerveau et de la Moelle épinière” or Brain and Spine Institute, an international research center being launched in Paris. Being both a clinician and a neuroscientist, his research has been devoted to studying the mechanisms and consequences of cell death with a special reference to basal ganglia disorders including Parkinson’s disease and other neuro-psychiatric afflictions.

STÉPHANE BANCEL
MBA, CEO, Moderna Therapeutics
Stéphane joined Moderna in the summer of 2011 when it was a one employee company. He has assembled a world-class team and raised more than $1Billion between equity financing and upfront from licensing collaborations. He was previously CEO of bioMérieux, a world leader in the diagnostics industry. bioMerieux has more than 6,000 employees, a market capitalization of €2.5 billion, and sales of more than €1.3 billion. Prior to his time at bioMérieux, Stéphane was the managing director of Eli Lilly Belgium and executive director of global manufacturing strategy and supply chain at Eli Lilly.

GÉRARD ARAUD
Ambassador of France to the United States
Gérard Araud, a career diplomat, was appointed Ambassador of France to the United States in September 2014. He previously held numerous positions within the Ministry of Foreign Affairs and International Development, notably including that of Director for Strategic Affairs, Security and Disarmament (2000-2003), Ambassador of France to Israel (2003-2006), Director General for Political Affairs and Security (2006-2009), and, most recently, Permanent Representative of France to the United Nations in New York (2009-2014). Over the course of his career, Mr. Araud has developed specialized knowledge in two key areas: the Middle East and strategic & security issues. As regards the latter, he was the French negotiator on the Iranian nuclear issue from 2003 to 2006. In New York, at the Security Council, he notably contributed to the adoption of resolutions on Libya (#1970 and #1973), Côte d’Ivoire (#1975), the Democratic Republic of Congo, Mali and the Central African Republic, and participated in debates on the Syrian and Ukrainian crises. He has written numerous journal articles, including one recently published in Commentaire, on the outbreak of World War One, and another in Esprit, on the search for a new world order.
NAHID BHADELIA
MD, MA, Director, Infection Control and Medical Response, National Emerging Infectious Diseases Laboratories, Infectious Diseases Physician, Boston University School of Medicine

Dr. Nahid Bhadelia, MD, MA is an infectious diseases physician, Assistant Professor at the Boston University School of Medicine and the Director of Infection Control and Medical Response at National Emerging Infectious Diseases Laboratory (NEIDL) at Boston University (BU). Her specialization is in infection control issues related to emerging pathogens and highly communicable infectious diseases. She is the director of the medical response program for BU’s biosafety level 4 laboratories at the NEIDL, one of 6 such programs in the US. Aside from her clinical training in infectious diseases, she has a master’s degree in international affairs from the Fletcher School of Law and Diplomacy and a background in health and human security with a focus on the impact of pandemics on macro level health indicators and community security. She has previously worked on projects with United Nations International Strategy for Disaster Reduction and the Global Fund to Fight AIDS, Tuberculosis and Malaria. She has served as a front line physician providing care to Ebola patients in Sierra Leone with World Health Organization. She is a Senior Policy and Technical Advisor to Partners in Health for their Ebola response program in Sierra Leone. She has also been an instructor for the US CDC/FEMA and Taiwan CDC’s healthcare worker preparedness courses for the Ebola Response.

CHRISTIAN BRÉCHOT
MD, PhD, President, Institut Pasteur

Christian Bréchot holds MD PhD degrees. He has been trained at the Institut Pasteur, and at the Necker Faculty of Medicine. In 1989 he became full professor of Cell Biology and Hepatology, and in 1997 he was appointed head of the clinical department of liver diseases at the Necker-Enfants Malades Hospital. He has been in charge of a research unit at the Necker Faculty of Medicine, jointly supported by Inserm (the French National Institute of Health and Medical Research), Paris Descartes University, and the Institut Pasteur; he was also head of the National Reference Centre on viral hepatitis from 1998 to 2001. From 2001 to 2007, Christian Bréchot was General Director of Inserm. From 2008 to 2013, he has been appointed Vice-president in charge of Medical and Scientific affairs of the Institut-Mérieux company. He has been also involved in the Scientific Direction of the Technological Research Institute BIOASTER on infectious diseases, physician, Assistant Professor at the Boston University School of Medicine and the Director of Infection Control and Medical Response at National Emerging Infectious Diseases Laboratory (NEIDL) at Boston University (BU). Her specialization is in infection control issues related to emerging pathogens and highly communicable infectious diseases. She is the director of the medical response program for BU’s biosafety level 4 laboratories at the NEIDL, one of 6 such programs in the US. Aside from her clinical training in infectious diseases, she has a master’s degree in international affairs from the Fletcher School of Law and Diplomacy and a background in health and human security with a focus on the impact of pandemics on macro level health indicators and community security. She has previously worked on projects with United Nations International Strategy for Disaster Reduction and the Global Fund to Fight AIDS, Tuberculosis and Malaria. She has served as a front line physician providing care to Ebola patients in Sierra Leone with World Health Organization. She is a Senior Policy and Technical Advisor to Partners in Health for their Ebola response program in Sierra Leone. She has also been an instructor for the US CDC/FEMA and Taiwan CDC’s healthcare worker preparedness courses for the Ebola Response.

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Dr. Gerry Cox is Vice President of Rare Diseases Clinical Development at Sanofi Genzyme, Cambridge, MA. Since joining Genzyme in 2000, Dr. Cox has overseen the clinical development programs for several lysosomal storage disorders and was the medical lead for the regulatory approvals of two enzyme replacement therapies (Aldurazyme for MPS I in 2003, and Elaprase for MPS II in Japan in 2006) and one substrate replacement therapy (Cerdelga for Gaucher disease type 1 in 2014). His group also is developing olipudase enzyme replacement therapy for Niemann-Pick B disease (Phase 3) and a novel CNS-penetrant glucosylceramide synthase inhibitor for Gaucher disease type 3 and several other indications (Phase 2). He holds two patents related to the treatment of Niemann-Pick B disease. Dr. Cox is a practicing physician who is board-certified in clinical, biochemical, and molecular genetics, and formerly in pediatrics. Prior to Sanofi Genzyme, he was a full-time staff geneticist at Boston Children’s Hospital, where he continues to see patients part-time and train fellows in the genetics clinic. He is an instructor in pediatrics at Harvard Medical School and was a founding member of the scientific advisory boards for the Barth Syndrome Foundation and the Pediatric Cardiomyopathy Registry. Dr. Cox graduated magna cum laude from Harvard College in 1980 with a B.A. in biology. He completed the Medical Scientist Training Program at the University of California at San Diego, where he received his M.D. and a Ph.D. in biology in 1989. His PhD thesis under Dr. S.J. Singer characterized the immunocytochemical localization of two novel cytoskeletal proteins (zeugmatin and enactin) associated with...
adherens junctions and sarcomeres. He then went to Boston Children’s Hospital, where he completed an internship and residency in pediatrics, and clinical and research fellowships in genetics. As a Howard Hughes post-doctoral fellow with Dr. Louis Kunkel, Dr. Cox researched dystrophin-related and associated proteins and linked the COL9A3 gene to autosomal dominant multiple epiphyseal dysplasia with mild myopathy. Dr. Cox has authored more than 70 peer-reviewed publications, reviews, and book chapters. His clinical interests include inborn errors of metabolism and genetic causes of cardiomyopathy and eye diseases.

PASCAL DESCHASEAUX

MD, MBA, General Manager, CALYM & VP, Association des instituts Carnot

Pascal Deschaseaux is the General Manager of the Carnot Lymphoma Institute (CALYM) and the Vice-President, International Affairs, of the Carnot Institutes Association. He is also the General Manager of the non-profit academic organization LYSARC, a global leader in the field of lymphoma clinical research operations. His professional experience comprises 5 years as a hospital physician, 18 years in the pharmaceutical industry and the last 8 years in the academic sector. He has expertise in management, clinical research, marketing and business development and has worked for several pharmaceutical companies, mostly in international or global senior positions. A former co-founder and CEO of a biotech company, he was the General Manager and Secretary General, respectively, of two French bioclusters. So far, his responsibilities have encompassed ethical (main) and OTC drugs, medical devices and services, in some of the biggest pharmaceutical markets, including oncology, diabetes and CNS.
ARNAUD FONTANET
MD, PhD, Director, Centre for Global Health, Institut Pasteur

Arnaud Fontanet is a medical epidemiologist (MD in 1988 at Paris V, specialisation in rheumatology in 1990, Paris V, and DrPH from Harvard School of Public Health in 1993) specialized in infectious diseases epidemiology. After working at the WHO Global Program on AIDS (1993-1994), he spent five years in Ethiopia and two years in the Netherlands with the Ethio-Netherlands AIDS Research Project (1994-2001). In 2002, he joined Institut Pasteur to launch the Emerging Diseases Epidemiology unit. There, his focus has been on viral hepatitis C (www.hepniel.org) and emerging infections such as SARS and MERS. Arnaud Fontanet is also Professor of Public Health at the Conservatoire National des Arts et Métiers, where he is Director and co-founder of the Pasteur-Cnam School of Public Health (http://ecole-pasteur.cnam.fr). In 2014, he was appointed as Director of the newly created Pasteur Centre for Global Health Research and Education.

CHARLES R.G. GUTTMANN
MD, Director, Center for Neurological Imaging, Brigham & Women’s Hospital, Associate Professor of Radiology, Harvard Medical School

Charles Guttmann is the founding Director of the Center for Neurological Imaging (CNI) at Brigham and Women’s Hospital and an Associate Professor of Radiology at Harvard Medical School. His team applies quantitative neuroimaging strategies to the study of neurological diseases, such as multiple sclerosis (MS) and cerebro-vascular diseases in the elderly. Dr. Guttmann has also spearheaded the development of informatics infrastructure in support of large-scale neuroimaging discovery research, including an image-centered, multi-disciplinary database and image analysis workflow management system, as well as – more recently – a virtual laboratory for collaborative neuroscience research.

JOHANNES FRUEHAUF
MD, PhD, co-Founder & President, LabCentral

Johannes Fruehauf is a founder and the president and executive director of LabCentral. He is responsible for all aspects of LabCentral’s operation. Johannes is a physician and successful biotech entrepreneur. Prior to LabCentral, he founded Cambridge Biolabs, a contract research facility serving startup and virtual companies in Kendall Square. He is also a co-founder of ViThera Pharmaceuticals, Deltix and Cequent Pharmaceuticals, and an advisor or board member to numerous life sciences companies and non-profits. Johannes earned his medical degree at the University of Frankfurt and his PhD at the University of Heidelberg (Germany).
KEITH A. HOFFMASTER
PhD, Director, Global Program Management, Translational Clinical Oncology, Novartis Institutes for Biomedical Research

Keith currently is responsible for leading early development Oncology programs through to clinical proof of concept. His role encompasses the oversight and leadership of several academic collaborations to further the understanding of biological mechanisms responsible for cancer disease progression and the emergence of drug resistance. At Novartis, Keith previously led scientific evaluation and business development activity for multiple disease areas, and held roles heading DMPK discovery efforts for the Infectious Disease and CV/Metabolism areas. Prior to Novartis, Keith spent 9 years at Pfizer, involved in drug discovery/development and in establishing and leading academic research collaborations in areas such as tissue engineering, liver biology, and drug transporters. Keith received his Ph.D. from UNC-Chapel Hill with a focus on mechanisms of hepatic transport and pharmacokinetics. He maintains an active academic role, lecturing at both MIT and Harvard University in these areas.

PETER HO
MD, PhD, CMO, Epizyme Inc.
Peter Ho, MD, PhD, has focused on cancer drug discovery and development since completing his medical training at the Dana-Farber Cancer Institute. He served as a Staff Fellow at the U.S. Food and Drug Administration and then as a Senior Investigator at the Investigational Drug Branch of the Cancer Therapy Evaluation Program of the National Cancer Institute. His pharmaceutical experience has included positions of increasing responsibility at Novartis Pharmaceuticals, DuPont Pharmaceuticals, GlaxoSmithKline, and Johnson and Johnson. Since 1995, he has been responsible for the first-time-in-human dosing of 17 anticancer agents and has overseen the development of over 50 oncology compounds in clinical phases of testing. These compounds have included small molecule inhibitors of intracellular signal transduction cascades, direct cytotoxic agents, hormonal antagonists, epigenetic modulators, radiopharmaceuticals, cytokines, monoclonal antibodies, monoclonal antibody conjugates and oncology supportive care agents. This work has contributed to five NCE approvals to date: Gleevec, Arranon, Tykerb, Promacta and Votrient. He currently is the Chief Medical Officer at Epizyme.
NEWTON HOWARD
PhD, Chairman, Brain Sciences Foundation
Professor Newton Howard is the Chairman of the Brain Sciences Foundation and currently serves as Associate Professor of Computational Neuroscience and Functional Neurosurgery at the University of Oxford, where he Directs the Oxford Computational Neuroscience Lab. He is also the Director of the Synthetic Intelligence Laboratory at MIT, where he had served as the Founder and Director of the MIT Mind Machine Project from 2008 to 2012. Dr Howard is an active member of several research laboratories worldwide, including the Descartes Institute, the Brain Physics Group and INSERM, in Paris. Prof Howard has been involved in a wide range of research, spanning academic, military and commercial domains. His work is presently focused on understanding how the human brain produces consciousness, thought and language, with the objective of developing advanced diagnostic and therapeutic tools for effective treatment of neurological disorders such as Alzheimer’s Disease, Parkinson’s Disease, PTSD, depression and epilepsy.

FABRICE JARDIN
MD, PhD, Director, INSERM U918 Research Team, Centre Henri Becquerel
Fabrice Jardin is a Professor of Hematology at Rouen University, France, and a physician in the department of clinical hematology at the Henri Becquerel Comprehensive Anticancer Center, Rouen, France. He is a member of the LYSA (The Lymphoma Study Association) scientific committee and the Head of the U918 INSERM research team entitled “Genetics and Clinic of Lymphoid Malignancies”. His fields of research include biomarkers analysis, genomic analysis (CGH, NGS) and transfer technologies -more specifically those regarding diffuse large B-cell lymphomas. He is the principal investigator (PI) or co-PI of several clinical trials and translational research projects for the LYSA group and the author of over 160 scientific papers and of two patents for molecular tests.

CHRISTOPHER LARKIN
CTO, GE Healthcare
Chris Larkin is a native of Washington DC. Coming from a Navy family, he lived in many places. He attended Virginia Tech, VCU and received his Master Degree in Engineering from University of Massachusetts. His graduate work concentrated in artificial intelligence and
Professor Macoska is the Alton J. Brann Endowed Chair and Distinguished Professor of Science and Mathematics at the University of Massachusetts Boston. Prior to that (1994-2012) she was Professor of Urology and Associate Director of the Graduate Program in Cellular and Molecular Biology and faculty in the Programs in Cancer Biology and Department of Computational Medicine and Bioinformatics at the University of Michigan. She also served as Associate Director, Prostate/Urologic Oncology Program, University of Michigan Comprehensive Cancer Center and is a past President of the Society for Basic Urologic Research. Professor Macoska has led peer-reviewed and funded research for >20 years that is focused on elucidating the molecular genetic alterations and dysfunctional inter- and intra-cellular signaling mechanisms that promote prostate pathobiology. Research in the Macoska laboratory is currently focused on: 1) Defining the mechanisms through which dysfunctional paracrine interactions between diverse cell types – epithelial, fibroblastic, endothelial, leukocytic – develop consequent to the aging process, and how these dysfunctional interactions contribute to the development of benign and malignant disease; 2) Elucidating the intracellular mechanisms through which growth factors, particularly CXC-type chemokines, secreted by aging stromal fibroblasts and inflammatory cells stimulate cellular proliferation and myofibroblast phenoconversion, and the association of these pathobiologies, particularly tissue fibrosis, with organ dysfunction and malignancy; 3) Understanding the similarities and differences applied math. Mr. Larkin worked as a consultant for technology in Healthcare and related start-ups before becoming VP and CTO for ShipLogix. Mr. Larkin then joined Microsoft and later joined the start-up team for Microsoft’s Health Solutions Group. After growing this global business, Mr. Larkin joined GE in Performance Solutions in 2011. Mr. Larkin has since become GE Healthcare’s Leader for Analytics and General Manager for Life Sciences Software. More recently, he has been working on a project called “Industrial Internet,” which outlines GE’s approach to bringing digital intelligence to the physical world of healthcare and Life Sciences.

CHRISTOPHE LE TOURNEAU
MD, PhD, Head of Early Trials, Institut Curie

Christophe Le Tourneau has been appointed as a senior Medical Oncologist at the Institut Curie in November 2009. He is heading the Early Phase Clinical Trials Program as well as the Head and Neck Clinic. He is also involved in precision medicine clinical trials, having been the PI of the SHIVA trial. Christophe Le Tourneau was certified in Medical Oncology in 2005 and got his PhD in Clinical Epidemiology in 2007. He did a 2-year Clinical Research Fellowship at Princess Margaret Hospital in Toronto, Canada, in the Drug Development Program. His main interests are precision medicine, phase I clinical trials with a special attention at the methodology to conduct these trials, as well as Head and Neck oncology. Christophe Le Tourneau is the principal investigator of several phase I trials, as well as of clinical trials in Head and Neck oncology. He has published over 85 peer-reviewed papers in international journals.

JILL A. MACOSKA
PhD, Director, Center for Personalized Cancer Therapy, The University of Massachusetts, Boston

Professor Macoska is the Alton J. Brann Endowed Chair and Distinguished Professor of Science and Mathematics at the University of Massachusetts Boston. Prior to that (1994-2012) she was Professor of Urology and Associate Director of the Graduate Program in Cellular and Molecular Biology and faculty in the Programs in Cancer Biology and Department of Computational Medicine and Bioinformatics at the University of Michigan. She also served as Associate Director, Prostate/Urologic Oncology Program, University of Michigan Comprehensive Cancer Center and is a past President of the Society for Basic Urologic Research. Professor Macoska has led peer-reviewed and funded research for >20 years that is focused on elucidating the molecular genetic alterations and dysfunctional inter- and intra-cellular signaling mechanisms that promote prostate pathobiology. Research in the Macoska laboratory is currently focused on: 1) Defining the mechanisms through which dysfunctional paracrine interactions between diverse cell types – epithelial, fibroblastic, endothelial, leukocytic – develop consequent to the aging process, and how these dysfunctional interactions contribute to the development of benign and malignant disease; 2) Elucidating the intracellular mechanisms through which growth factors, particularly CXC-type chemokines, secreted by aging stromal fibroblasts and inflammatory cells stimulate cellular proliferation and myofibroblast phenoconversion, and the association of these pathobiologies, particularly tissue fibrosis, with organ dysfunction and malignancy; 3) Understanding the similarities and differences
between disease pathobiologies to better stratify patients for appropriate treatment strategies, and 4) Translating laboratory-based knowledge to the development of clinically efficacious biomarkers and therapeutics.

**STEPHANIE MARRUS**

MBA, Director, Entrepreneurship Center, University of California, San Francisco

Stephanie Marrus is Director of the Entrepreneurship Center at the University of California, San Francisco (UCSF), the leading graduate healthcare/life science and medical institution in the US. Her mission is to start companies from UCSF inventions and to build the entrepreneurial ecosystem at the University. During a 25-year plus business career, she has worked with hundreds of companies in science- and technology-based industries as a corporate executive, business consultant and mentor, many with their technological roots at MIT, Harvard, Berkeley, Stanford and UCSF. In addition to her business career, she served as Deputy Secretary of Economic Affairs for a Massachusetts governor where she led economic development, business policy and chaired an environmental cleanup fund board. She has been president of a medical research foundation and serves on the Advisory Board of a social venture startup accelerator. Stephanie has had roles as CEO, COO and VP Business Development for public and private companies, led business development and M&A transactions, headed corporate development, strategy, marketing, communications and investor relations. Her sector experience is in technology, life sciences/healthcare and consumer products. She holds an MBA from the Wharton School, University of Pennsylvania, an MA from Columbia University and an AB from Cornell University.

**TRAVIS MCCREADY**

JD, President & CEO, Massachusetts Life Sciences Center

Travis McCready joined the Massachusetts Life Sciences Center as the organization’s second President & CEO in October 2015. In this position, Mr. McCready is responsible for leading the ongoing implementation of the $1 billion Massachusetts Life Sciences Initiative, a public-private partnership that has contributed to Massachusetts’ emergence as the global leader in life sciences. McCready directs and oversees the Center’s investment strategy, along with the agency’s operations, programs and partnerships. Previously Travis served as the Vice President for Programs at the Boston Foundation, directing the distribution of nearly $20 million in discretionary grants through a competitive process to support nonprofit organizations and programs that serve the people of Greater Boston. Prior to that, Travis was the first Executive Director of the Kendall Square Association, responsible for building a global brand for Kendall Square, engaging in business and economic development, and ensuring the on-going vitality of one of the Commonwealth’s most economically robust districts. He has also previously held the COO & CFO positions at the Massachusetts Convention Center Authority, where he oversaw all operations and finance for the Commonwealth’s three convention centers and the Boston Common Garage as well as previously serving as Chief of Staff of the Boston Foundation, and Director of Community Affairs for Harvard University. Travis received his B.A. from Yale University and J.D. from the University of...
Iowa, and began his law career as a corporate attorney in Minneapolis, MN. Travis serves on the boards of the American Repertory Theater in Cambridge, the Massachusetts Institute for a New Commonwealth, and the Center for Collaborative Leadership at UMass Boston. He has served on the Economic Development Planning Council under two governors, including co-chairing the subcommittee on innovation and entrepreneurship. In 2009, he was named one of Boston’s top “40 under 40” young business leaders by the Boston Business Journal. He is a frequent speaker on economic development strategy as it relates to the convergence of private, public and not-for-profit institutional sectors.

MICHEL MEIGNAN
MD, PhD, Professor of Nuclear Medicine, Henri Mondor University Hospital

Michel Meignan is a Professor of Nuclear Medicine and former Head of the department of Nuclear Medicine at Henri Mondor University Hospital, Créteil, France. His main fields of research are interim positron emission tomography (PET) and quantitative PET in lymphoma. He is the Director of the research group “Imaging for evaluation of therapy” (UMR 7054, Créteil, France) and the Scientific Coordinator of the LYSA-IM platform and a member of the scientific board of the Lymphoma Study Association (LYSA). Founder of the International Workshops on PET in Lymphoma (Deauville, France in 2009, and Menton, France in 2010, 2011,2012,2014), he is the author of around 190 scientific publications in international journals (impact factors RG score= 43,7).

BRITTANY MCDONOUGH
Senior Manager of Business Development, Massachusetts Office of International Trade and Investment (MOITI)

Brittany McDonough oversees MOITI activities related to Europe, the Middle East and the Americas. Ms. McDonough specializes in international entrepreneurship and supports cluster development in the clean energy, technology and financial services industries. Prior to joining MOITI, Ms. McDonough served as the executive assistant to the Office of the Governor’s Legal Counsel. Ms. McDonough graduated Cum Laude from Suffolk University with a bachelor’s degree in Political Science. At Suffolk, she served as the United Nation’s Studies Program undergraduate president as well as on the executive board for Model United Nations. Ms. McDonough is a 2014 graduate of Emerge Massachusetts.

FANNY MOCHEL
MD, PhD, Head of Metabolic Research Group, ICM

Fanny Mochel is an associate professor of genetics at the University Pierre and Marie Curie (UPMC). She received her MD in Genetics in 2005 at the University Paris Descartes, her PhD in Neuroscience in 2010 at UPMC and is board certified in inborn errors of metabolism.
Dr Mochel runs a neurometabolic clinic at La Pitié-Salpêtrière university hospital and is the head of the Neurometabolic research group. She is co-chair of the French society for inborn of errors of metabolism in adults and a scientific board member of the Fondation Lejeune. Her research is focused on the characterization and treatment of brain energy deficiencies in neurodegenerative diseases, especially Huntington disease. Her major areas of expertise are the identification of neurometabolic biomarkers in vitro (metabolomics) and in vivo (nuclear magnetic resonance spectroscopy) as well as therapeutic approaches targeting the Krebs cycle (patents WO 2008068230 A1 and WO 2014093901 A1).

Since March 2007, Sergio Roman-Roman is Head of the Translational Research Department at Institut Curie (Paris, France) where he coordinates the preclinical oncology programs and heads a group of scientists and physicians involved in translational research projects. From 2002 to 2007, he was Head of in vitro Pharmacology and Responsible of Prospective Discovery Portfolio Management at ProSkelia Pharmaceuticals (Founding member), bought in 2004 by ProStrakan. From 1998 to 2002, he led a Molecular and Cell Biology laboratory at Aventis Pharma (Bone Diseases group). Sergio Roman-Roman also managed a laboratory in the Department of Immunology, Roussel-Uclaf and Hoechst-Marion-Roussel (1992-1998). Prior to entering the pharmaceutical industry, he trained as a Post-doc fellow at INSERM U333 (Prof. Thierry Hercend) Institut Gustave-Roussy (Villejuif, France). Overall, Sergio Roman-Roman has published more than 60 publications in peer-reviewed Journals and has a H-factor=20 with >2,000 citations.

Don Pettini has been designing and implementing large-scale technology solutions for the past 25 years, including in his role as Chief Technology Officer (CTO) for over 10 years in several companies. At Oracle, Don is responsible for Enterprise Health Analytics and the analytics platform that supports on premise and cloud deployed offerings, namely Enterprise Healthcare Analytics, Translational Research Center and Health Sciences Network. Previously, Don was a co-founder and Chief Technology Officer at Humedica now Optum Analytics), a next-generation clinical informatics company. Additionally, Don brought his extensive background in data security and privacy to various positions at AltaVista and Digital Equipment Corporation.

Sergio Roman-Roman
PharmD, PhD, Head of Translational Research, Institut Curie

Since March 2007, Sergio Roman-Roman is Head of the Translational Research Department at Institut Curie (Paris, France) where he coordinates the preclinical oncology programs and heads a group of scientists and physicians involved in translational research projects. From 2002 to 2007, he was Head of in vitro Pharmacology and Responsible of Prospective Discovery Portfolio Management at ProSkelia Pharmaceuticals (Founding member), bought in 2004 by ProStrakan. From 1998 to 2002, he led a Molecular and Cell Biology laboratory at Aventis Pharma (Bone Diseases group). Sergio Roman-Roman also managed a laboratory in the Department of Immunology, Roussel-Uclaf and Hoechst-Marion-Roussel (1992-1998). Prior to entering the pharmaceutical industry, he trained as a Post-doc fellow at INSERM U333 (Prof. Thierry Hercend) Institut Gustave-Roussy (Villejuif, France). Overall, Sergio Roman-Roman has published more than 60 publications in peer-reviewed Journals and has a H-factor=20 with >2,000 citations.

Don Pettini
Sr. Director, Healthcare Product Strategy, Health Sciences Global Business Unit, Oracle

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EMANUELA ROMANO
MD, PhD, Head of Immunotherapy Unit, Institut Curie

Dr. Romano is a physician-scientist specializing in melanoma, tumor immunotherapies, and early-phase clinical trials. In October 2015, she has been appointed Clinical Director of the Immunotherapy Unit at the Institut Curie to foster the development of immunotherapies with a strong translational research component. From 2011 to 2015, Dr. Romano worked as attending physician at the Service of Oncology, University Hospital of Lausanne and as group leader of the Laboratory of Tumor Immunobiology at the Ludwig Institute for Cancer Research. She completed her studies at the University La Sapienza and the National Cancer Institute of Rome - Italy, and subsequently joined the Memorial Sloan-Kettering Cancer Center in New York for a postdoctoral training in tumor immunology. Her research interests lie in the direct interrogation of the human tumor microenvironment by multiple biological and bioinformatics means, with the goal to elucidate immune-protective and immune-escape mechanisms, and develop and implement new modalities to use the immune system to treat cancer.

MICHELE RUSSELL-EINHORN
Senior Director, Office for Human Research Studies, Dana-Farber Cancer Institute

Michele Russell-Einhorn is an expert in the field of research administration, cancer center scientific review, and the protection of human subjects in research. She is currently the Senior Director of the Office for Human Research Studies at the Dana-Farber Cancer Institute and is responsible for the management and support of scientific review and institutional review board review, as well as other regulatory matters, involving all cancer relevant research involving human subjects conducted at the five Harvard clinical institutions under the umbrella of the Dana-Farber/Harvard Cancer Center. Trained as a lawyer, she has over 30 years of professional experience including service as the Conflicts of Interest Attorney for the National Institutes of Health; Director of Regulatory Affairs for the U.S.D.H.H.S Office for the Protection from Research Risks (OPRR) and its successor office, the Office for Human Research Subjects (OHRP); Director in the Global Pharmaceuticals Practice at PWC; as well as the Associate General Counsel for the J. Craig Venter Institute. She is a co-chair of the U.S.D.H.H.S. Secretary’s Advisory Committee on Human Research Protections, Subcommittee on Subpart A; as well as a founder and leader of the IRB Directors Group of the National Comprehensive Cancer Center. She served as the Co-Chair for three years and a core planning committee member for five years of the annual Ethics in Research Conference sponsored by Public Responsibility in Medicine and Research; and, a member of the Vanderbilt University Steering Committee for an NIH Grant on Alternative IRB Models. She participates each year as a
José-Alain Sahel is Professor of Ophthalmology at Pierre & Marie Curie University, Sorbonne Universities and Cumberlege Professor of Biomedical Sciences at the Institute of Ophthalmology-University College London, Chairman of the Departments of Ophthalmology at the Quinze-Vingts National Eye Hospital and the Rothschild Ophthalmology Foundation. He is founder and director of the ‘Institut de la Vision’ in Paris (18 principal investigators, more than 250 staff members). The primary focus of his research is understanding the mechanisms associated with retinal degeneration, together with conception, developing and evaluation of innovative treatments (medicinal products, gene therapy and stem-cell therapy, retinal prosthesis) for currently untreatable retinal diseases. José-Alain Sahel oversees more than 80 clinical trials and chairs a network of more than 90 European clinical trial centers on retinal diseases. He is Member of the Academy of Sciences-Institut de France, the German National Academy of Sciences Leopoldina and the Academia Ophthalmologica Internationalis.

Gilles Salles is a Professor of Medicine at the University of Lyon, France. He heads the Department of Hematology at Lyon-Sud University hospital, Lyon, France, and the «Clinical and Experimental Model of Lymphomagenesis» research team of the INSERM Cancer Research Center of Lyon. He is the Chairman of CALYM, the lymphoma-devoted Carnot institute, the President of the Lymphoma Study Association (LYSA), a worldwide renowned cooperative group in the lymphoma field, and the President of the Lymphoma Academic Research Organization (LYSARC), a premier lymphoma academic clinical research organization. He is in charge of clinical research within the executive board of Hospices Civils de Lyon. He is particularly interested in the clinical and biological study of malignant lymphomas with emphasis on the description and validation of prognostic factors as well as clinical trials in indolent lymphomas. As Coordinator or Co-Investigator in many clinical trials and studies, Gilles Salles is the author of more than 300 articles in international peer-reviewed journals including The Lancet, New England Journal of Medicine, Journal of Clinical Oncology and Blood.
JONATHAN S. STAMLER

MD, Director, Harrington Discovery Institute

Director, Harrington Discovery Institute, is an internationally renowned physician-scientist, recognized for the discovery of protein S-nitrosylation, a ubiquitous mechanism for controlling protein function that operates across phylogeny and in all cell types and tissues, and that is commonly dysregulated in disease. His work on S-nitrosylation is notable for providing new insights into mammalian physiology, including the regulation of cardiac contractility and the discovery of red blood cell-mediated vasodilation, which is essential for oxygen delivery to all tissues. Dr. Stamler’s laboratory is also known for groundbreaking work on hemoglobins in mammals, microbes and worms, in the process discovering the vasoregulatory role of a third gas (nitric oxide) in the mammalian respiratory cycle (thereby redefined as a 3-gas system: NO/O2/CO2) as well as new enzymatic functions of hemoglobins that protect against nitric oxide in lower organisms. Dr. Stamler also identified the molecular mechanism of nitroglycerin bioactivation and tolerance. Dr. Stamler is a graduate of Brandeis University in Boston, and received his medical degree from Mount Sinai School of Medicine in New York. He completed his internship, residency and fellowships in cardiovascular and pulmonary medicine at Harvard Medical School and Brigham and Women's Hospital, Boston. He was on the faculty at Harvard University before spending 16 years at Duke University and the Howard Hughes Medical Institute, Durham, NC. Dr. Stamler relocated to Case Western Reserve University School of Medicine and University Hospitals Case Medical Center in 2010, where he holds the Reitman Family Distinguished Chair of Cardiovascular Innovation, is a professor of medicine and biochemistry and director of the Institute for Transformative Molecular Medicine as well as director of the Harrington Discovery Institute. Dr. Stamler has published more than 275 original articles and edited two books. He has co-founded seven biotechnology companies and licensed two additional programs to major pharmaceutical companies. He holds more than 125 patents and patent applications, and has been recognized by several awards and prizes.

KENICHI TAKESHITA

MD, Vice-President Clinical R&D, Celgene Corporation

Kenichi Takeshita, Corporate Vice President, Clinical Research and Development, Celgene, has led the clinical program in lymphoma and chronic lymphocytic leukemia (CLL) since 2008. This resulted in the approval of lenalidomide in mantle cell lymphoma, Istodax in peripheral and cutaneous T cell lymphomas, as well as initiating the on-going lenalidomide registration trials in follicular and large cell lymphomas and evaluation of newer Celgene pipeline assets in CLL and lymphoma, including pleotropic pathway modulators (CC-122), epigenetic hypomethylating agents (CC-486), immuno-oncology assets (durvalumab) and CAR T cell technology. Dr Takeshita also served as the founding general manager of Celgene KK (Japan) from 2005 to 2007, starting the Japanese Revlimid program resulting in approvals in multiple myeloma and deletion 5q myelodysplastic syndrome. Prior to joining the biotechnology field, Dr Takeshita was on the faculty in Hematology at Yale University School of Medicine USA and New York University School of Medicine, USA. Dr Takeshita received an A.B. Degree in
PIERRE TERVÉ
Vice-President Technic & Scientific Advisor, Keosys

Pierre Tervé, MSE, is co-founder and Vice-President – Technical & Scientific Advisor of Keosys, a company specializing in the management of imaging in clinical trials (Imaging CRO) and medical imaging software design. He began his career with Alcatel in 1997 and was in charge of several telecom-oriented projects as a Project Manager. When creating Keosys in 2001, he took over the management of the research team and the development of medical imaging softwares for nuclear medicine and radiology until 2007. He was then in charge of the commercial development of the product line devoted to nuclear medicine in France. By 2010 he developed collaborative research programs and partnerships with various international academics groups as Chief Scientific Officer. He build the Advanced Imaging Solutions division (AMI) that he lead during one year before taking in charge several the Big Data Projects department. He also works as a temporary teacher at the Ecole Centrale de Nantes and Polytech’ Nantes university, France. He is a member of several works groups of the Quantitative Imaging Biomarker Alliance (QIBA).

ROBERT G. URBAN
PhD, Head of Johnson & Johnson Innovation Center, Boston

Robert leads Johnson & Johnson’s Boston Innovation Center. Johnson & Johnson Innovation Centers are home to investment teams that work closely with entrepreneurs to advance products and technology up to proof-of-concept across the pharmaceuticals, medical device & diagnostic and consumer sectors. Their investments focus on people and programs that have the potential to not just advance healthcare, but to transform it. Located in Boston, Menlo Park, London and Shanghai, the J&J Innovation Centers seek to simplify and expedite the transformation of discoveries into healthcare products that improve lives across the world. Prior to J&J, Robert was the founding Executive Director of MIT’s Institute for Integrative Cancer Research. Robert has co-founded multiple biopharmaceutical companies, held a variety of R&D leadership/operational roles and served as an advisor or board member in the industry. Robert received his Bachelor of Arts degree in Microbiology and his Ph.D. in Microbiology and Immunology from the University of Texas system and was an Irvington Fellow in structural immunology at Harvard University.

Molecular Biology from Harvard University, an M.D. from Yale University and post-doctoral training in developmental biology at Yale and in hematopoiesis at University of Tokyo.
MICHEL VELLARD

PhD, VP Research, Ultragenyx

As a postdoctoral fellow, Dr. Michel Vellard started to work on rare diseases. In the pediatric department at UCLA Harbor Medical Center he researched the gene responsible for lysosomal disease cystinosis. Dr. Vellard studied extensively with Emil Kakkis at Harbor, specifically on lysosomal diseases and enzyme replacement therapy (ERT). For 14 years, Dr. Vellard worked at BioMarin in increasing scientific management capacities. During his tenure, he was involved with most of the products developed there, and managed several programs including TFEB (collaboration with TIGEM/A. Ballabio), Duchenne Muscular Dystrophy (Phase I) and particularly an ERT for Morquio syndrome, the latest (VIMIZIM) has been approved in 2014. Since 2014 he is the VP of Research at Ultragenyx, a company exclusively centered on rare genetic diseases. Michel received his B.S. in Natural and Life Sciences and M.S. in Molecular and Cellular Genetics from the University of Lyon I, France. He obtained his Ph.D. in Virology, at Institut Pasteur, from University of Paris VI, VII, XI, France. Michel holds multiples patents and has authored and co-authored many research publications.
Presenting organizations

**Brain Sciences Foundation**
The Brain Sciences Foundation (BSF) is a 501(c)3 nonprofit organization committed to promoting awareness of neurological health and improving methods for effective treatment. BSF is a highly interdisciplinary organization, drawing together a wide range of scientific disciplines concerned with the understanding of brain function and dysfunction. BSF has a distinguished, multidisciplinary team of fellows with extensive knowledge of neuronal processes and degeneration at all stages. Recent research initiatives focus on the development of functional brain and neuron interfacing abilities.

[www.brainsciences.org](http://www.brainsciences.org)

**Celgene**
Celgene seeks to deliver truly innovative and life-changing drugs for our patients. Their vision as a company is to build a major global biopharmaceutical corporation while focusing on the discovery, the development, and the commercialization of products for the treatment of cancer and other severe, immune, inflammatory conditions.

[www.celgene.com](http://www.celgene.com)

**Institut du Cerveau et de la Moelle Epinière**
ICM is one of the largest translational Neuroscience research facility in the world, with +500 researchers at the heart of the 1st Neurology practice in France. Home for +20 start-up companies, ICM primes integrated programs at the crossroads of biotech, medtech and digital healthcare.


**Institut Curie**
Institut Curie is a Comprehensive Cancer Centre that brings together a Research Centre and a Hospital. The institute is active in various topics (Biology, Physics, Chemistry and Medicine), and has been able to create conditions that foster collaborations.

[www.curie.fr/en](http://www.curie.fr/en)

**Dana farber Cancer Institute**
Since its founding in 1947, Dana-Farber Cancer Institute in Boston, Massachusetts has been committed to providing adults and children with cancer with the best treatment available today while developing tomorrow’s cures through cutting-edge research. Read about our history, our breakthroughs, and the resources that help us support the health of our neighborhoods and communities.

[www.dana-farber.com](http://www.dana-farber.com)
Epizyme
Epizyme is a clinical stage biopharmaceutical company that is creating novel epigenetic therapies for cancer patients. We have built a proprietary library of more than 30,000 histone methyl transferase inhibitors and have clinical programs targeting the HMTs EZH2 and DOT1L.
www.epizyme.com

FDA
The Food and Drug Administration (FDA or USFDA) is a federal agency of the United States Department of Health and Human Services. The FDA is responsible for protecting and promoting public health through the regulation and supervision of food safety, tobacco products, dietary supplements, prescription and over-the-counter pharmaceutical drugs (medications), vaccines, biopharmaceuticals, blood transfusions, medical devices, electromagnetic radiation emitting devices (ERED), cosmetics, animal foods & feed and veterinary products.
www.fda.gov

GE Healthcare
GE Healthcare provides transformational medical technologies and services that are shaping a new age of patient care. Their expertise in medical imaging and information technologies, medical diagnostics, patient monitoring systems, drug discovery, biopharmaceutical manufacturing technologies, performance improvement and performance solutions services help to deliver better care to more patients.
www3.gehealthcare.fr/

Harrington Discovery Institute
The Harrington Discovery Institute at University Hospitals in Cleveland, Ohio—part of The Harrington Project for Discovery & Development—aims to advance medicine and society by enabling our nation’s most inventive physician-scientists to turn their discoveries into medicines that improve human health.
www.uhhospitals.org/services/harrington-discovery-institute

Centre Henri Becquerel
The Centre Henri Becquerel is a comprehensive cancer center located in Rouen, France, belonging to the Unicancer group of French comprehensive cancer centers. It was created in 1960, and named after the French physicist who shared the discovery of radioactivity with Marie and Pierre Curie. It has three missions: patients care, research and teaching. In research it has two main branches: basic research and clinical research. The center has an oncologic genetics laboratory dedicated to the study of genetic changes in tumor tissue and the special causes of tumor development. The Centre Henri Becquerel is also distinguished for its many studies and publications in the field of lymphoma, in the framework of the LSYA group (a component of the CALYM Carnot institute) activities.
www.becquerel.fr
Henri Mondor Hospital
Henri Mondor hospital is a university hospital located in Créteil, France, belonging to the Albert Chenevier - Henri Mondor hospital group. It offers close to 1,000 beds and is operated by 4,000 healthcare professionals, who treat a year around 40,000 patients. It covers the majority of medical and surgical disciplines, to the exception of mother & child care, and offers post-acute and rehabilitation cares. It is a teaching and research center, host of 9 INSERM and 2 CNRS research units, as well as many university and hospital labs.
www.chu-mondor.aphp.fr

J&J Innovation Center
Johnson & Johnson Innovation Centers are home to investment teams that work closely with entrepreneurs to advance products and technology up to proof-of-concept across the pharmaceuticals, medical device & diagnostic and consumer sectors.
www.jnjinnovation.com

Keosys
Keosys Medical Imaging uses technology and innovation to provide flexible medical imaging solutions for clinical trials and multisite academic studies. Headquartered in France, Keosys has become a European leader in Imaging Data Management Systems with a fully integrated workflow. Keosys also provides advanced medical imaging software that is valuable for 2D and 3D image viewing and analysis.
www.keosys.com

LabCentral
LabCentral is a first-of-its-kind shared laboratory space designed as a launchpad for high-potential life sciences and biotech startups. It offers fully permitted laboratory and office space for as many as 25 startups comprising approximately 100 scientists and entrepreneurs.
www.labcentral.org

The Lymphoma Study Association (LYSA)
LYSA, a world-renowned cooperative group, brings together medical professionals specializing in the field of lymphoma in order to promote basic and clinical research and disseminate its results, improve prevention, management, and treatment of lymphoma patients.
www.lysa-lymphoma.org/

LYSARC
LYSARC is a non-profit organization, a European academic leader in lymphoma clinical research operations, devoted to the running of those studies accepted by the LYSA board of directors.
www.lysarc.org/
MOITI
The Massachusetts Office of International Trade and Investment (MOITI) is the Commonwealth’s primary international business development agency charged with promoting trade and investment with global partners in Massachusetts and around the world. Their office is dedicated to building successful international collaborations and partnerships essential to bringing new economic opportunities and investments to the Commonwealth. MOITI’s mission is simple: strengthen the Massachusetts economy, create jobs and increase the Commonwealth’s international presence.
http://www.mass.gov/hed/economic/eohed/moiti/

Moderna
Moderna is pioneering messenger RNA Therapeutics™, an entirely new in vivo drug technology that produces human proteins, antibodies and entirely novel protein constructs inside patient cells, which are in turn secreted or active intracellularly.
www.modernatx.com

Center for Neurological Imaging, Brigham and Women’s Hospital
The Center for Neurological Imaging is a joint venture between the Departments of Neurology and Radiology at Brigham and Women’s Hospital. It consists of two sections: the Image Analysis Laboratory (IAL), and the Magnetic Resonance Imaging Facility (MRI). State-of-the-art imaging systems and a powerful computer facility provide outstanding capability for imaging the human central nervous system, and then quantify and visualize CNS structures. Resources are also committed towards the creation of easier ways to manage patient data for doctors.
www.cni.bwh.harvard.edu

Novartis
Novartis provides innovative healthcare solutions that address the evolving needs of patients and societies. Headquartered in Basel, Switzerland, Novartis offers a diversified portfolio to best meet these needs: innovative medicines, eye care and cost-saving generic pharmaceuticals. Novartis is the only global company with leading positions in these areas.
www.novartis.com

Oracle
Oracle is focused on making high quality healthcare synonymous with cost effective care and believes the sophisticated tools used to expand “Precision Medicine” should be leveraged to make “Population Health” a broad success with the support of modern, agile, business systems. Oracle, and its partners, have embraced this challenge and built the most powerful and flexible tools that enable healthcare providers and payers to embrace new care models, foster change, engage a modern workforce, and create optimized business processes.
www.oracle.com
Institut Pasteur
Institut Pasteur, a private foundation with officially recognized charitable status set up by Louis Pasteur in 1887, is today an internationally renowned center for biomedical research with a network of 33 institutes worldwide.
www.pasteur.fr/en

Center for Personalized Cancer Therapy (CPCT)
The Center for Personalized Cancer Therapy (CPCT) is a joint program of the University of Massachusetts Boston and the Dana Farber/Harvard Cancer Center (DF/HCC). A scientific focus of the center will be to elucidate how lifestyle choices, particularly those that promote diet-induced obesity and type 2 diabetes mellitus (‘diabesity’), modulate the tumor microenvironment, and how such modulation affects tumor initiation, progression, and response to therapy.
www.umb.edu/cpct

Sanofi Genzyme
Sanofi Genzyme, the specialty care business unit of Sanofi, focuses on rare diseases, multiple sclerosis, oncology, and immunology. We help people with debilitating and complex conditions that are often difficult to diagnose and treat. Our approach is shaped by our experience developing highly specialized treatments and forging close relationships with physician and patient communities. We are dedicated to discovering and advancing new therapies, providing hope to patients and their families around the world.
www.sanofigenzyme.com

Spark Therapeutics
Spark is a gene therapy leader seeking to transform the lives of patients suffering from debilitating genetic diseases by developing one-time, life-altering treatments. Spark’s initial focus is on treating rare diseases where no, or only palliative, therapies exist.
www.sparktx.com

Entrepreneurship Center at UCSF
The Entrepreneurship Center at UCSF provides the knowledge, support and connections needed by our community of scientists and clinicians who want to commercialize their inventions through a startup. They aim to empower their community by providing courses, programs and a network to expose them to the entrepreneurial mindset and the Silicon Valley ecosystem that is the world’s standard. They support entrepreneurial interests in all areas of life science/healthcare, whether therapeutics, research tools, medical devices, diagnostics, digital health or Big Data.
www.ita.ucsf.edu/entrepreneurship-center
Ultragenyx

Ultragenyx is a clinical-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases. Founded in 2010, the company has rapidly built a diverse portfolio of product candidates with the potential to address diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no approved therapies.

www.ultragenyx.com

Institut de la Vision

Institut de la Vision is one of the most important research centers in Europe on eye diseases. It brings together researchers, clinicians and industrial partners to enable sharing of skills and ideas, and to foster the translation of fundamental discoveries into new treatments.

www.institut-vision.org
Conference location
MIT Media Lab, 75 Amherst St, Cambridge, MA 02139

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