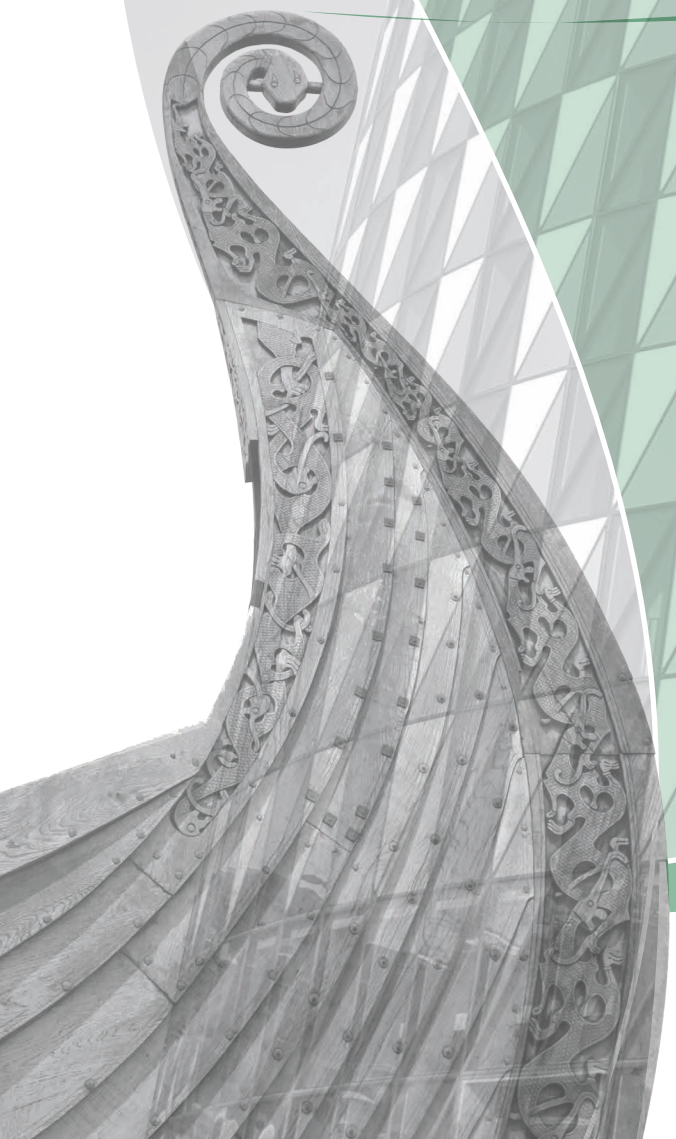
Days of Molecular Medicine 2015

Emerging Partnerships in Translational Science

Stockholm, Sweden, May 7-8, 2015



Organized by:

Days of Molecular Medicine Global Foundation

Karolinska Institutet

Fondation IPSEN

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WELCOME

Dear Participant,

On behalf of the DMM Global Foundation, Karolinska Institutet and Fondation IPSEN, we would like to welcome you to the 2015 DMM meeting

Partnerships in Translational Science and Medicine:
Academia, Hospitals, Foundations, and the Private Sector

This year's meeting is unique, focusing on the importance of partnerships, by highlighting joint programs where foundations and the philanthropic community, the business sectors and medical and research institutions have successfully joined together to further advance human health. Some of these stories are driven by families who are directly affected by these diseases, and who have put their trust in science in an effort to find a way to help their loved ones. At the same time, the pharmaceutical and biotechnology sectors are forging novel approaches to integrate their efforts at the earliest stages with leading physicians and scientists. These innovative partnerships are clearly emerging as a new force to advance translational medicine.

Each year the DMM meeting topic is vastly different, but one consistent focus has been to support the participation of young scientists from all parts of the world. We hope that these prime examples of emerging partnerships are inspiring and informative to the next generation of physicians and scientists.

We also would like to take this opportunity to thank our dedicated sponsors, who have helped make this meeting possible. We are most appreciative for your participation and support for DMM 2015.

We are thrilled that you have joined us and hope that you enjoy the outstanding list of speakers.

With very best wishes,
Sincerely,

Megan Donovan-Chien
Vice President, DMM Global Foundation

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THE 2015 MEETING

The DMM 2015 meeting, entitled "Partnerships in Translational Science and Medicine: Academia, Hospitals, Foundations, and the Private Sector", will highlight the growing importance and impact of novel partnerships to support studies in the clinical setting by bringing together an international roster of effective partnerships working on a broad spectrum of diseases at the scientific, educational, and infrastructural levels. In addition, the meeting aims to discuss how a re-thinking of partnerships beyond conventional boundaries are becoming an essential part of translational biology and medicine. Accordingly, the meeting will be designed to include a joint talk form both partners, the institutional partner as well as the recipient scientist, where the time is split between both parties.

Organizers:

Karolinska Institutet *(Stockholm, Sweden)*, DMM Global Foundation *(Cambridge, USA)* in collaboration with the Fondation IPSEN *(Paris, France)*

Program Committee:

Hans-Gustaf LJUNGGREN *(Karolinska Institutet)*, Kenneth R. CHIEN *(Karolinska Institutet)*, Urban LENDAHL *(Karolinska Institutet)*, Göran HANSSON *(Karolinska Institutet)*, Anna WEDELL *(Karolinska Institutet)*, Yves Christen *(Fondation IPSEN)*, Susan SOLOMON *(NY Stem Cell Foundation)*

Meeting Coordinators:

Megan DONOVAN-CHIEN *(DMMGF)*, Katarina DRAKENBERG *(Karolinska Institutet)*, Jacqueline MERVAILLIE *(Fondation IPSEN)*

MOST RECENT DMM MEETINGS

2006 • Inflammatory Pathways in Disease *(Stockholm, Sweden, May 24-27)*

2007 • Emerging Technologies in Cancer Biology *(Cambridge, USA, May 22-24)*

2008 • Molecular Medicine in Cognitive Dysfunction *(Stockholm, Sweden, April 17-18)*

2009 • Human Genetics, Stem Cells and Physiology:
The Future of Individualized Medicine *(Boston, USA, May 7-9)*

2010 • Systems biology approaches to cancer and metabolic disease
(Stockholm, Sweden, May 20-22)

2011 • Re-engineering regenerative medicine *(Hong Kong, China, November 10-12 - in collaboration with the University of Hong Kong and the support of the Croucher Foundation)*

2012 • From rare to care *(Vienna, Austria, October 8-10)*

THE DMM SERIES

Over the past 15 years, DMM has become one of the most prestigious international meetings dedicated to promoting translational science and molecular medicine. The meeting was initiated with the vision that humans themselves will become the primary model organisms for understanding human biology and disease, which has increasingly become enabled by major leaps in core technology spanning the field of genetics, imaging, stem cell biology, and biotechnology. The meeting is designed to break new ground in a specific arena that is viewed as being timely as well as critical to translational science at the highest level, and is not intended to highlight a single disease area or technology.

Days of Molecular Medicine Global Foundation

The DMM Global Foundation

The DMM Global Foundation is a non-profit organization dedicated to forming a bridge between advances in molecular medicine and global cardiovascular health, through educational programs and research collaborations. Our primary focus is to promote global collaboration in the field of molecular medicine through the organization of conferences with other leading international institutions. A key to the success of these conferences is our dedication to support young physician-scientists, from all parts of the world, to participate in these conferences and to expand the international biomedical network to further advance global health issues. In addition, we are developing a Global Health initiative designed to identify, design, and catalyze promising research collaborations that uncover new scientific approaches to global cardiovascular health.

Karolinska Institutet

Karolinska Institutet

Karolinska Institutet is one of the world´s leading medical universities. Its vision is to significantly contribute to the improvement of human health. Karolinska Institutet accounts for over 40 per cent of the medical academic research conducted in Sweden and offers the country´s broadest range of education in medicine and health sciences. The Nobel Assembly at Karolinska Institutet selects the Nobel laureates in Physiology or Medicine.

FONDATION IPSEN

Fondation IPSEN

Created in 1983 under the auspices of the Fondation de France, the Fondation IPSEN tracks progress in biomedical research with the continuing aim of highlighting fundamental advances. Staying away from passing trends, the ambition of the Fondation IPSEN is to identify emerging knowledge and new paradigms, and to foster the most promising interconnections between domains that have not previously been communicating. Because it was necessary to concentrate its efforts, the topics selected reflect some of the most important challenges for the current evolution of the world as well as of knowledge: the aging of populations; the spectacular development of neuroscience and its contribution to the understanding of cognitive mechanisms; the interactions between the great biological systems, such as the nervous and endocrine systems, and the medical challenges posed by the biomedical revolution, particularly in the science of cancer. Over the last 32 years, the Fondation IPSEN has organized over 250 meetings and produced several hundreds publications; more than 250 scientists and biomedical researchers have been awarded prizes and research grants.

PROGRAM

THURSDAY, MAY 7

9:00 – 9:10 am

Welcome Remarks

Anders HAMSTEN *(Vice-Chancellor, KI)* and **Kenneth CHIEN** *(Professor, KI)*

9:10 – 10:00 am

Keynote

Göran HANSSON *(Professor at Karolinska Institute, Board Member of the Nobel Foundation)*
The Nobel Prize - A century of private-public partnership for the benefit to mankind

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Session I

Cell Based Therapy/ Regenerative Medicine Alliances

Chairs: **Kenneth CHIEN** *(KI)* and **Thomas SCHWARZ-ROMOND** *(Executive Publisher, Elsevier - Journals)*

10:00 – 10:40 am

Lee RUBIN *(Professor, Department of Stem Cell and Regenerative Biology, Harvard University)*
Discovering new treatments for Spinal Muscular Atrophy

10:40 – 11:00 am Break

11:00 – 11:40 am

Noelle FREY *(Assistant Professor of Medicine, University of Pennsylvania)*
CAR T Cells: The realization of a personalized cellular therapy for CD19+ malignancies

11:40 – 12:20 pm

Susan SOLOMON *(Co-Founder and CEO, New York Stem Cell Foundation)*
Getting to cures: A new model of high-tech collaborative research

12:20 – 1:00 pm Lunch

Session II

Genetic and Gene Therapy Alliances

Chairs: **Anna WEDELL** *(Professor, KI)* and **Orla SMITH** *(Managing Editor, AAAS/Science Translational Medicine)*

1:00 – 1:40 pm **Robert MacLAREN** *(Professor of Ophthalmology, University of Oxford)*
Gene therapy with adeno-associated viral (AAV) vectors to treat retinal disease

1:40 – 2:20 pm **Alain FISCHER** *(Professor of Pediatric Immunology, Université Paris-Descartes)*
Gene therapy of immunodeficiencies, safety, efficacy, sustainability

2:20 – 3:00 pm **Michael WIGLER** *(Professor of Genetics, Cold Spring Harbor Laboratory)*
Spontaneous mutations for autism specturm disorders

3:00 – 3:20 pm Break

3:20 – 4:00 pm **Jean-Pierre CHANGEUX** *(Professor, Institut Pasteur, and Collège de France)*
Pasteur-Kavli partnership : from the concept of allosteric interaction to the design of allosteric modulators

4:00 – 4:40 pm **Chris HEMPEL** *(Founder, Addi and Cassi Fund)*
Citizen science – A mom's unexpected and remarkable journey into world of science

4:40 – 5:20 pm **Karen SLIWA** *(Director, Hatter Institute; Professor, University of Capetown)*
Heart disease in Africa – Gaps and opportunities

6:00 – 8:00 pm Speakers dinner *(Transportation provided)*

FRIDAY, MAY 8

Session III

Biotechnology Alliances

Chairs: **Hans-Gustaf LJUNGGREN** *(Dean of Research, KI)*, and **Richard HORTON** *(Editor-in-Chief, Lancet)*

9:00 – 9:40 am **Frank ACCURSO** *(Professor of Pediatrics, University of Colorado)*
Partnerships in cystic fibrosis care and research: An academician's perspective on KalydecoTM and beyond

9:40 – 10:20 am **Frederick DEWEY** *(Director, Translational Genetics, Regeneron)*
Natural human gene knockouts in the discovery of new drug targets

10:20 – 11:00 am **Eric REIMAN** *(CEO, Banner Research; Professor of Psychiatry, University of Arizona)*
Preventing Alzheimer's together

11:00 – 11:20 am Break

11:20 – 12:00 pm **Ricardo DOLMETSCH** *(Global Head of Neuroscience, Novartis)*
A vision for the future of drug discovery in neuroscience

12:00 – 12:40 pm **Mene PANGALOS** *(Executive Vice-President, Innovative Medicines and Early Development, AstraZeneca)*
Creating an open research ecosystem where science thrives

12:40 – 1:40 pm Lunch

Session IV

Global Health Alliances

Chairs: **Yves CHRISTEN** *(Chairman, Fondation IPSEN)* and **Rui-Ping XIAO** *(Director, Institute of Molecular Medicine, Peking University; Associate Editor, New England Journal of Medicine)*

1:40 – 2:20 pm **Richard HORTON** *(Editor-in-Chief, Lancet)*
Partnerships for global health: successes, questions, and opportunities

2:20 – 3:00 pm **Michael WATSON** *(Executive Vice-President, Vaccination Policy and Advocacy, Sanofi Pasteur)*
Intended and unintended consequences of global health alliances in the vaccination ecosystem and implications for all public health partnerships

3:00 – 3:40 pm **Garry JENNINGS** *(Director and CEO, Baker IDI Heart and Diabetes Institute)*
Australian Indigenous Health – global challenge and local solutions

3:40 – 4:20 pm **Kartik CHANDRAN** *(Associate Professor of Microbiology and Immunology, Albert Einstein College of Medecine)*
Studying Niemann-Pick Type C disease to illuminate Ebola

Closing Remarks

Hans-Gustaf LJUNGGREN *(Dean of Research, KI)*

Reception for all in Aula Medica

SPEAKER ABSTRACTS

Göran HANSSON

Professor, Karolinska Institutet
Board Member of the Nobel Foundation
Stockholm, Sweden

Göran K Hansson is Professor of Cardiovascular Research at Karolinska Institute and works in the Department of Medicine at Karolinska University Hospital and and the Center for Molecular Medicine. He received his MD and PhD at Gothenburg University School of Medicine in Sweden, was a postdoctoral fellow at the University of Washington in Seattle, WA, USA, and has been Professor of Cell Biology at Gothenburg University and Leducq Visiting Professor at Harvard Medical School in Boston, MA, USA.
Dr Hansson is Vice Chairman of the Board of Directors of the Nobel Foundation and has been a member of the Nobel Assembly at Karolinska Institute since 1997. He chaired its Nobel Committee 2004-6 and was Secretary and Director of the Medical Nobel Institute 2009-2014. From 1 July 2015, he will be the Permanent Secretary of the Royal Swedish Academy of Sciences. He is a member of Academia Europaea and has received several awards and honorary doctorates/ professorships for his contributions to medicine.
Dr Hansson´s research deals with immune and inflammatory mechanisms in atherosclerosis. In 1985, he discovered that atherosclerosis involves an inflammatory immune response. His work has shown that low-density lipoprotein (LDL) elicits an autoimmune response, and that immunosuppressive drugs inhibit arterial restenosis, a principle used in current therapy.

NOTES

The Nobel Prize - A century of private-public partnership for the benefit to mankind

In his will of 1895, Alfred Nobel stipulated that his estate should be used to finance prizes in science, literature and peace. Whereas the Royal Swedish Academy of Sciences was to select laureates in physics and chemistry, the prize in "physiology or medicine" was given to Karolinska Institutet. However, Karolinska Institutet did not feel it had sufficient resources and was at first sceptical to the task. A little over a hundred years later, it is a responsibility that is gladly accepted and the Institute is part of a private-public network that also involves the other Prize awarding institutions as well as the private Nobel Foundation that coordinates and finances the Nobel Prize activities.

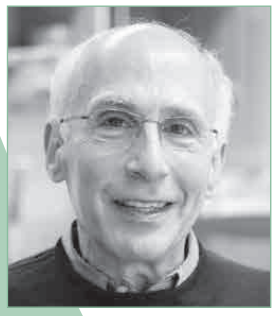
Today, the Nobel Laureates in Physiology or Medicine are appointed by the Nobel Assembly at Karolinska Institutet, which consists of 50 members elected from the professors at Karolinska Institutet. What constitutes a great medical discovery that benefits humanity? This is the question that the Nobel Assembly and its working body, the Nobel Committee, has been tasked with answering.

In order to get a Nobel Prize, you have to make a major discovery that shifts the paradigm in medical science. You then have to make the international science community recognise the greatness of your discovery so that they nominate you as a candidate. Last, but not least, your discovery has to convince the Nobel Assembly at Karolinska Institutet, who has to compare it with those of many other outstanding scientists around the globe.

The careful work by the Nobel Committees and the prize-awarding institutions safeguards the esteem of the Nobel Prizes, widely regarded as the most prestigious awards given for intellectual achievement in the world.

Lee RUBIN

Professor
Department of Stem Cell and Regenerative Biology,
Harvard Stem Cell Institute, Harvard University
Cambridge, USA



Dr. Rubin has a broad experience in both academia and industry, particularly in the realms of cell-based assays and drug discovery. Prior to coming to Harvard, he was Chief Scientific Officer of Curis, Inc., a Cambridge-based biotechnology company, where his group identified the first small molecule regulators of the hedgehog signaling pathway. One of these antagonists was developed by Genentech and is now (as Erivedge) approved as the first oral treatment for metastatic basal cell carcinoma. At Harvard, much of his work is focused on finding key molecular mediators of different neurodegenerative diseases and on searching for effective preclinical therapeutic candidates. His research takes advantage of his group's ability to produce large numbers of patient-derived induced pluripotent stem cell lines and of effective means of deriving differentiated neurons from them. They have set up an array of techniques that allow them to identify early cellular and physiological changes in neurons as they become diseased. For example, they have identified new targets for the treatment of the motor neuron disorders Spinal Muscular Atrophy and Amyotrophic Lateral Sclerosis. They are also studying Psychiatric disorders, Parkinson's disease and Alzheimer's disease. Recently, his group discovered that a circulating protein, GDF11, has the ability to reverse some of the changes in the CNS associated with aging. They are actively exploring the therapeutic implications of these observations as well.

Dr. Rubin received his PhD in neuroscience from the Rockefeller University and had postdoctoral training, also in Neuroscience, at Harvard Medical School and Stanford University School of Medicine.

NOTES

Discovering new treatments for Spinal Muscular Atrophy

Spinal Muscular Atrophy (SMA) is a childhood motor neuron disease caused by a mutation in the gene *Survival of Motor Neuron 1 (SMN1)*. As an early onset monogenic condition, it is, seemingly, the simplest of all neurodegenerative diseases. The number of SMA patients, about 30,000 in the US, is similar to the number with Amyotrophic Lateral Sclerosis (ALS or Lou Gehrig's disease), a late onset, predominantly non-familial disorder associated with many different individual mutations. In spite of that, until about 10 years ago, it was less well recognized and relatively understudied. I will review the progress that has been made in the last few years, including studies done using SMA patient-derived motor neurons. I will also emphasize the key role that the SMA Foundation has played in supporting research and in facilitating industry involvement to find effective therapeutics.

Noelle FREY

Assistant Professor
Perelman Center for Advanced Medicine, University of Pennsylvania
Philadelphia, USA

Noelle Frey, MD, MSCE is an Assistant Professor of Medicine and the Associate Director of Bone Marrow Transplant Cellular Therapeutics at the University of Pennsylvania. She received her BS from Yale Univeristy and received her Medical Degree from Columbia University. She completed her residenecy and fellowship training at UPENN where she remains on faculty. Dr Frey was a recipient of the Leukemia and Lymphoma Special Fellow in Clinical Research and Recipient of the Robert Austrian Award for Translational Research. Dr. Frey's clinical and research interests focus on early phase clinical trials using novel therapeutics in patients with leukemia.

NOTES

CAR T Cells: The realization of a personalized cellular therapy for CD19+ malignancies

Chimeric Antigen Receptors (CARs) are engineered and introduced into a patient's own T cells redirecting them to target specific tumor antigens including CD19. This novel cellular therapeutic approach is now being developed clinical by several programs to treat patients with relapsed CD19+ malignancies including acute lymphoblastic leukemia (ALL), chronic lymphocytic leukemia (CLL) and non-Hodgkin lymphoma (NHL). High complete remission rates have been observed in patients with ALL, many who have been successfully bridged to a curative stem cell transplant with this approach. Excitingly, in our experience, some remissions have been remarkably durable even without a consolidative SCT, a finding which correlates with in vivo T cell persistence and B cell aplasia over time. At the University of Pennsylvania, the development of our CAR Therapy Program results from years of collaborative efforts among scientists led by Dr. Carl June, the success of which was dependent in large part on philanthropic funding. In addition, the ability to continue to move this therapy forward to larger multicenter trials, has relied on a collaborative partnership within the pharmaceutical industry.

Susan SOLOMON

Co-Founder and CEO
The New York Stem Cell Foundation (NYSCF)
New York, USA

Susan L. Solomon is Chief Executive Officer and Co-Founder of The New York Stem Cell Foundation (NYSCF) Research Institute, the leading non-profit research institute dedicated to bringing scientific research to the clinic. Funded by private philanthropy, NYSCF accelerates stem cell research through the 45 scientists in the independent NYSCF Laboratory and over 50 collaborations with prestigious academic institutions, disease foundations, and industry leaders worldwide. Since its founding in 2005, NYSCF has invested over $140 million in stem cell research to find new treatments and cures for the devastating diseases of our time.

A longtime health-care advocate, Ms. Solomon is a founding member and current President of NYAMR (New Yorkers for the Advancement of Medical Research), is on the Executive and Nominating Committees for the Alliance for Regenerative Medicine; the Board of Directors for the Centre for Commercialization of Regenerative Medicine; the Board of Directors and Nominating Committee for the College Diabetes Network; the Board of Directors of the Regional Plan Association; the Editorial Board of Regenerative Medicine*; and is a past member of the Board of Directors of the Juvenile Diabetes Research Foundation, New York Chapter. Ms. Solomon was also a member of the inaugural Strategic Planning Committee of the Empire State Stem Cell Board. In December 2012, Ms. Solomon received the Stem Cell Action Leadership Award from the Genetics Policy Institute. In March 2008, she received a New York State Women of Excellence Award from the Governor of New York and, in September 2008, she received the Triumph Award from the Brooke Ellison Foundation for her work in establishing NYSCF.*

Prior to founding NYSCF, Ms. Solomon, a former attorney at Debevoise & Plimpton, spent much of her career in the private sector. She established and ran Solomon Partners LLC to provide strategic management consulting to Fortune 500 corporations, major cultural institutions, foundations and other non-profit organizations. She was the founding Chief Executive Officer of Sothebys.com. She has served as Chairman and Chief Executive Officer of Lancit Media Productions, an Emmy award-winning television production company, and as the Founder and President of Sony Worldwide Networks. Ms. Solomon has also held executive positions at MacAndrews and Forbes Holdings and MMG Patricof and Co.

She received her BA cum laude from New York University and her JD from Rutgers University School of Law, where she was an editor of the Law Review.

NOTES

Getting to cures: A new model of high-tech collaborative research

The New York Stem Cell Foundation (NYSCF) combines the depth of a highly focused research institute with the breadth of a wide-ranging philanthropic organization. Since its establishment in 2005, NYSCF has invested over $140 million in stem cell research and continues to conduct the most advanced stem cell research internationally, both in its own laboratory in New York City, and also in collaboration with major medical research institutions around the world.

As a privately funded, independent stem cell research laboratory, NYSCF is closing the gap that exists between academic institutions and pharmaceutical companies, by advancing external research collaborations and creating new technology platforms for use within the larger research community. Through specific programs focused on assay development, compound screening, and target validation, as well as through the development of the NYSCF Global Stem Cell Array, NYSCF is advancing research in the pursuit of cures. The NYSCF Global Stem Cell Array is a new technology platform for the derivation and manipulation of stem cell lines as well as the differentiation of adult cell types in a high-throughput, parallel process using automation. The platform is generating panels of stem cell lines from thousands of genetically diverse individuals representing the majority of the human population, including both diseased patients and controls. NYSCF research has also resulted in the first human embryonic stem cell line derived from a patient with a disease as well as the development of a technique to prevent mitochondrial diseases, mitochondrial replacement therapy.

NYSCF also supports an international community of over 120 collaborating scientists including 50 early career scientists at the postdoctoral level, 30 early career professors, and 45 scientists in its own laboratory. In addition, NYSCF educates the public about the promise of stem cell research through public panels and talks, and convenes annually the preeminent international conference on translational stem cell research for the scientific community.

Robert MacLAREN

Professor
Gene and Stem Cell Therapy for Retinal Diseases Laboratory, Oxford University
Oxford, UK

Robert MacLaren is a Professor of Ophthalmology at the University of Oxford, Honorary Consultant Ophthalmologist at the Oxford Eye Hospital, Honorary Professor of Ophthalmology at the UCL Institute of Ophthalmology, Honorary Consultant Vitreoretinal Surgeon at Moorfields Eye Hospital, Faculty Member and Founding Theme Leader of the Moorfields-UCL Institute of Ophthalmology Biomedical Research Centre. He is also a Fellow of the Royal College of Ophthalmologists, Fellow and former King James IV Professor of Surgery at the Royal College of Surgeons of Edinburgh, and Bodley Fellow of Merton College Oxford. In 2015 he will receive The John Marshall Award, RP Fighting Blindness. He was recipient of the annual Jessie Mole Medal for retinitis pigmentosa research in 2014; together with The Euretina Lecture, awarded annually to the lead retina specialist in Europe. His research is dedicated to finding new treatment for blindness, using stem cell-based approaches, gene therapy or electronic retinas.

NOTES

Gene therapy with adeno-associated viral (AAV) vectors to treat retinal disease

The small target size, privileged immune status and ease of safe access makes the eye an ideal target organ for gene therapy. Added to that there are many single gene disorders that cause currently incurable blindness which have a devastating socioeconomic burden. Our current laboratory work focusses on using adeno-associated viral (AAV) vectors to treat common genetic disorders of the photoreceptors and the retinal pigment epithelium (RPE). We have recently shown safety and proof of principle in a gene therapy trial to treat choroideremia, an X-linked retinal degeneration first described in the Nineteenth Century, using an AAV vector encoding Rab escort protein 1 (REP1). The combination of clear efficacy, safety and relative ease of production with small volumes, makes retinal gene therapy using AAV vectors an attractive option for developing a licensed gene therapy treatment for regulatory approval. Our work is also supported by a significant investment made by the Wellcome Trust through Syncona Partners into the Nightstar programme, which underpins vector production and provides an expert commercial team to support the academics in translating the scientific progress into a real treatment for the benefit of patients.

Alain FISCHER

Professor
[1] Collège de France, [2] INSERM U1163 & Imagine Institute,
[3] Paris Descartes University – Sorbonne Paris Cité, [4] Unité d'Immunologie et Hématologie Pédiatrique, Assistance Publique – Hôpitaux de Paris
Paris, France

Dr Alain Fischer studied medicine, with a specialization in pediatrics and pediatric immunology at the Université of Paris, where he received his medical and doctoral degrees. After completing a postdoctoral fellowship at the University College London, he started independent research in a unit of the National Health Institute of Medical Research (INSERM) at the Necker Hospital in Paris. Since 1991, he has been the director of an INSERM unit for "normal and pathological development of the immune system." Since 2009, he is the director of the Institute for Genetic Diseases (Imagine) at Necker University Hospital. Dr Fischer also served as a professor of pediatric immunology at the Université Paris Descartes. From 1996 to 2012, he has served as the director of the pediatric immunology department at the Necker Hospital. Dr Fischer is presently Professor at College de France (Chaire de Médecine Expérimentale). Dr Fischer's main research interests are in gene therapy, genetics of immunological disorders, primary immunodeficiency diseases, and the development of the lymphoid system. He has been author or co-author of over 600 publications on these topics. Dr Fischer received the Louis Jeantet Prize for Medicine in 2001 and the Japan Prize 2015. Since 2002, Dr Fischer has been a member of the French Academy of Science and the European Molecular Biology Organization.

NOTES

Gene therapy of immunodeficiencies, safety, efficacy, sustainability

Gene therapy can be viewed today as an additional therapeutic option for some inherited diseases and particular forms of cancer. Retrovirally-mediated gene transfer into hematopoietic progenitor cells has been shown to be efficient in providing sustained correction of 2 forms of severe combined immunodeficiencies (SCIDX1), thus providing a proof of principle for gene therapy. Nevertheless, its development has been hampered by the occurrence of genotoxicity in some patients, shown to be caused by viral enhancer-mediated transactivation of oncogene(s). Development of self-inactivated retro viral (both γ RV and lenti) vectors may have overcome this risk as based on the results of several otherwise successful clinical studies (SCID, Wiskott Aldrich adreno leukodystrophy, metachromatic leukodystrophy) performed over the last 7 years. This is opening a path toward broader application in the field of genetic diseases of the hematopoietic system. Future strategy may consider gene editing by tailored made endonucleases and homologous recombination templates. The major challenge ahead for gene therapy all together consists in the large-scale production of adequate vectors.

Michael WIGLER

Professor
Human Genetic Disorders, Population Genetics,
Cancer Genomics Program, Cold Spring Harbor Laboratory
Cold Spring Harbor, USA

Dr. Michael Wigler received his B.A. in mathematics from Princeton University in 1970, and his Ph.D. in Microbiology in 1978 from Columbia University. Upon completion of the doctoral degree, he went to Cold Spring Harbor Laboratory, where is now Professor. Together with Axel and Silverstein at Columbia, he discovered a technique still used for engineering mammalian cells to produce medicinally useful proteins. His team was the first to isolate a mammalian gene using gene transfer and among the first to identify a mutant human oncogene. His laboratory discovered the involvement of three members of the RAS *family in human cancer; demonstrated the inheritance of DNA methylation patterns; pioneered the use of yeast as a model to explore more complex organisms, leading to an understanding of the* RAS *signaling pathway; co-invented (with Clark Still of Columbia University) encoded combinatorial synthesis, which has accelerated the discovery of new drug candidates; together with Nikolai Lisitsyn invented RDA, a method for comparative genome analysis that led to the discovery of the* PTEN *tumor suppressor (with Ramon Parsons of Columbia University) and the Kaposi's sarcoma virus (by others at Columbia University); and developed representational genomic approaches that are used widely in genotyping. Dr. Wigler's research is presently focused on the genomics of cancer and the genetics of autism and related disorders. Together with Jim Hicks and Nick Navin his laboratory demonstrated the feasibility of single cell sequencing for genomic analysis and expects this work will eventually improve the targeting of cancer treatments and lead to early and less invasive tests for cancer. His studies in human genetics led to the discovery of a vast unsuspected source of genetic variability known as copy number variation (CNV), and to the hypothesis that spontaneous mutation is a major contributor of autism. Dr. Wigler was elected to the National Academy of Sciences in 1989 and to the American Academy of Arts & Sciences in 1999, and is the recipient of many awards, including the Double Helix Medal in 2007.*

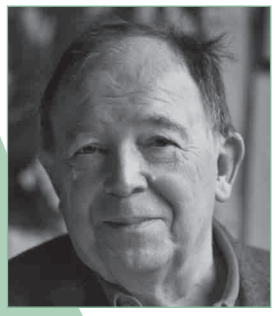
NOTES

Spontaneous mutations for autism specturm disorders

The last decade has witnessed remarkable progress in the understanding of the genetics of autism spectrum. The previous model of this complex disorder based on common genetic variants has proven barren, neither yielding gene discovery nor explaining the epidemiology of the disorder. Rather the theory of rare variants, arising de novo and inferred to be of strong and dominant effect, has led to the discovery of new causative mutations in specific genes, an enumeration of the number of gene targets, and plausible models for the epidemiology based on both germline and transmitted variants. These discoveries pave the way for early diagnosis, stratification, and ultimately improvement in treatments. The genetic models for autism appear to apply as well to other pediatric disorders that dramatically reduce fecundity. The relationship between autism, the action of purifying selection, and intellectual ability will be discussed. The history of these findings, from the discovery of the prevalence of de novo copy number variations, to genome sequencing of families with affected children, will be traced in the context of the enlightened philanthropy.

Jean-Pierre CHANGEUX

Professor
Kavli Institute for Brain, Mind UCSD, and Institut Pasteur
Paris, France



Jean-Pierre Changeux PhD is International Faculty at the Kavli Institute for Brain & Mind University of California San Diego and Professor at the Collège de France & Institut Pasteur, Paris.
His PhD studies with Jacques Monod, led to the discovery that chemical signals regulate the biological activity of proteins by acting at "allosteric" sites distinct from the biologically active sites via a conformational change (1961-1965). He then proposed (1964,1966) that this type of regulation applies to receptor mechanisms engaged in the transmission of chemical signals in the nervous system and through his life-time work, validated this insight. His studies were initiated by the first identification of a neurotransmitter receptor: the nicotinic acetylcholine receptor together with Lee & Kasai (1970) and culminated by a contribution to establishing the 3-D structure and conformational transition of prokaryotic orthologs of nicotinic receptors by X-ray crystallography and molecular dynamics (2005-15). Changeux and his colleagues also deciphered the topology of allosteric modulatory sites for pharmacological ligands (1996-2011), thereby substantiating a novel strategy of drug design based on allosteric modulation.
Moving to neuronal networks, Changeux, together with Courrège & Danchin (1973, 1976) formulated and experimentally tested the theory that long-term epigenesis of neuronal networks occurs by the activity-dependant selective stabilization and elimination of developing synapses.
Last, in particular with Dehaene, he proposed and tested models for defined cognitive tasks and their pharmacological modulation (1991-2015) in particular, a neuronal hypothesis for conscious processing, implicating a "global neuronal workspace" composed of a brain-scale horizontal network of long axon neurons (1998-2015).
Changeux has published several books including Neuronal Man *(1985),* What Makes Us Think? *(with Paul Ricoeur) (2002),* Physiology of truth *(2002).*
His academic accolades include the Gairdner award (1978), the Wolf prize (1983), the Goodman and Gilman Award in drug receptor pharmacology (1994), the Balzan Prize (2001), the US National Academy of Sciences Award in Neurosciences (2007) & the Japanese Society for the Promotion of Science Award for Eminent Scientists,Tokyo (2012).

NOTES

Pasteur-Kavli partnership : from the concept of allosteric interaction to the design of allosteric modulators

This work was initiated at the Pasteur Institute and continued at the Kavli Institute for Brain & Mind in San Diego with the aim to develop new strategies for drug design. It is based upon the concept of allosteric interaction (1) initially proposed to account for the inhibitory feedback mechanism mediated by bacterial regulatory enzymes. Contrasting the classical mechanism of competitive, steric, interaction between ligands for a common site, allosteric interactions take place between topographically distinct sites and are mediated by a discrete and reversible conformational change of the protein.

The concept was soon extended to membrane receptors for neurotransmitters (2) and shown to apply to the signal tranduction process which, in the case of the acetylcholine nicotinic receptor (nAChR), links the ACh binding site to the ion channel (3). Pharmacological effectors, such as Ca^{++} ions and ivermectin, referred to as allosteric modulators, were discovered and shown to enhance the transduction process by binding to sites distinct from the orthosteric ACh site and the ion channel (4). The recent X-ray structures, at atomic resolution, have revealed the resting and active conformations of prokaryotic and eukaryotic homologs of the nAChR, in combination with atomistic molecular dynamics simulations (5). Two distinct quaternary transitions in the transduction process with tertiary changes could be visualized which profoundly modify the boundaries between subunits. These interfaces host orthosteric and allosteric modulatory sites which exhibit structural organizational changes in the course of the transition. The model emerging from these studies has lead to the conception and development of several new pharmacological agents.

Looking for chemical therapies against Autism, a strategy was elaborated on the basis of brain genes expression data. Thus, the concept of coherent-gene groups controlled by transcription factors (TFs) was used, resulting in the design of allosteric modulators targeted toward specific TFs expressed at critical periods of brain development (6).

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Chris HEMPEL

Founder
Addi & Cassi Fund
Reno, USA

Chris Hempel is the co-founder of Spark Public Relations, one of the largest privately held and nationally recognized public relations agencies in the United States with offices in San Francisco and New York. Ms. Hempel has 20 years of experience creating global public relations campaigns for some of the most well-known Silicon Valley startups and Fortune 500 companies. Prior to starting her own public relations agency, Ms. Hempel worked at Netscape, the world's first Web browser software company, where she led corporate public relations.

In 2007, Ms. Hempel learned that her now 11-year-old identical twins, Addison and Cassidy, suffer from a rare and fatal condition called Niemann-Pick Type C disease (NPC). NPC is a genetic cholesterol disorder that progressively damages the nervous system and is commonly referred to as "Childhood Alzheimer's." Since her twins received this unimaginable diagnosis, Ms. Hempel has been on a mission to find a cure and has worked tirelessly with leading doctors and researchers around the world to develop lifesaving treatments.

In 2009, Ms. Hempel and her husband received approval from US FDA to treat their twins with a non-toxic sugar compound that they developed called cyclodextrin. The Hempel's story of developing this novel compound to save their twins' lives made international headlines and the family has been covered by many major media outlets including the Wall Street Journal and CNN. The FDA gave clearance for the twins' physician to begin the world's first cyclodextrin treatments directly into Addi and Cassi's bloodstreams (April 2009) and their brains (October 2010). Today, over 30 NPC children around the world are being treated with cyclodextrin.

In 2011, Ms. Hempel received a Google news alert on a story from Science Daily about a new study published in the journal Nature: «Ebola virus entry requires the cholesterol transporter Niemann-Pick C1.» In other words, a person without a functioning NPC1 gene such as her twins appear to be incapable of contracting the Ebola virus. In the paper, the researchers noted the skin cells for their discovery had come from the Coriell Institute for Medical Research, a biobank and independent non-profit organization for human genomics in Camden, New Jersey. Several years before, Hempel had donated skin cells from Cassi and Addi to Coriell for scientists to use in lab-based research. This surprising link between this rare genetic disease and the deadly Ebola virus is now leading to many breakthrough studies.

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Citizen Science - A mom's unexpected and remarkable journey into world of science

Chris Hempel has spent the last eight years fighting for the lives of her 11 year old identical twin daughters, Addison and Cassidy. Both have Niemann-Pick Type C, an incredibly rare hereditary disease that's caused primarily by mutations in the NPC1 gene, which is responsible for the body's ability to metabolize cholesterol. It's estimated that there are currently just 500 known cases in the world of Niemann-Pick Type C, a neurodegenerative condition that causes delay and loss of cognitive and motor function, neurological problems and seizures. The disease causes a harmful buildup of lipid proteins in organs including the spleen, liver, lungs, bone marrow and brain, and eventually proves fatal; patients usually die before they reach adolescence.

Out of necessity, Ms. Hempel has embarked on a deep journey into the scientific research community to try and help her daughters. She has contacted doctors and scientists around the world in her quest to save their lives, which even led her to doctors at the Karolinska Institute and the University of Gothenberg. Her family's journey has been chronicled in The Wall Street Journal and on CNN as she and her husband worked to developed a new drug for their daughters despite having no scientific backgrounds. Many unexpected things have occurred during Ms. Hempel's remarkable journey into the world science. She will share her journey with us today which includes genetically engineering mice and donating her tissues samples to science.

Karen SLIWA

Director, Hatter Institute for Cardiovascular Research in Africa
Professor, University of Cape Town
Cape Town, South Africa

Prof. Karen Sliwa, MD, PhD, FESC, FACC is the Director of the Hatter Institute for Cardiovascular Research in Africa (www.hatter.uct.ac.za), University of Cape Town and Director of the Soweto Cardiovascular Research Unit (www.socru.org), University of the Witwatersrand, Johannesburg, South Africa.
In addition she holds is a Professorial Fellow at both the Baker Institute, Melbourne, Australia and at the Population Health Research Institute, McMaster University, Hamilton, Canada.
Professor Karen Sliwa has been extensively involved in creating worldwide awareness of Peripartum Cardiomyopathy, one of her main areas of clinical and pathophysiological research. Recent progress in understanding underlying pathophysiology enabled by novel experimental approaches, together with a unique international effort to join forces between Western countries and developing countries, has led to great progress in awareness and management of the disease In order to coordinate efforts even better, a Study Group, 'Peripartum Cardiomyopathy', of the Heart Failure Association of the European Cardiac Society, chaired by Prof. Karen Sliwa and Prof. B. Pieske, was formed in 2009. A Position Statement, highlighting the need for larger awareness worldwide was published in 2010. A large Registry on Peripartum Cardiomyopathy was approved by the European Cardiac Society and has commenced 2012 in the ESC countries and affiliated regions.
Karen Sliwa has, together with her long-term collaborator, Prof. Simon Stewart, Head Preventive Cardiology, Baker IDI, Melbourne, Australia, established a population study in Africa called the 'Heart of Soweto Study' *to investigate the prevalence, presentation and management of cardiac disease in an urban African population. This study, on more than 8000 patients, highlighted the high prevalence of hypertension, obesity and cardiac disease in women of childbearing age. She has recently expanded her population studies (under the umbrella of the* 'Heart of Africa studies') *to other African countries, including Mozambique, Nigeria, Tanzania, Kenya and Sudan. She has designed a number of innovative research programs and leveraged funding for several major research projects, not only in South Africa and the rest of Africa, but also internationally. She has published more than 150 articles which includes 11 in* The Lancet *(IF 39.9) and her work is highly cited.*
In recognition of her work Prof Sliwa has received several awards, including the South Africa/Germany Year of Science Celebrations Award (2012) and the German Cardiac Society Paul Morawitz Award for Exceptional Cardiovascular Research (2013).
Karen Sliwa serves on a number of editorial boards and is also an editorial consultant to 'The Lancet'. Furthermore, she is the president elect of the South African Heart Association and on the board of the South African Heart Failure Society (Hefssa; www.hefssa.org), which was established under her leadership 2005. She also has been elected to serve on the Research Committee of the World Heart Federation.

NOTES

Heart disease in Africa – Gaps and opportunities

Africa is a continent characterized by marked ethnic, socio-demographic and economic diversity, with profound changes in many regions over the past two decades. This diversity has an impact on cardiovascular disease (CVD) presentation and outcomes. Within Africa and within the individual countries one can find regions having predominantly communicable diseases such as rheumatic heart disease, tuberculous pericarditis or cardiomyopathy and others having a marked increase in non-communicable disease, such as hypertension and hypertensive heart disease. Poor data and low investment in research and development in almost all African countries have a profound effect, complicating the effective planning of health care. Therefore I initiated a number of cohort studies in Soweto, South Africa - 'Heart of Soweto Studies' (8000 patients) to investigate the prevalence, presentation and management of cardiac disease in an urban African population. The first paper published in the Lancet in 2008 highlighted worldwide attention to the large and complex burden due to CVD in African populations. Up until that stage the focus on health challenges for Africans was mainly on infectious disease, thus ignoring the large burden of hypertension leading to heart failure and stroke amongst many other cardiovascular conditions. This very large research project resulted in >25 publications to date describing e.g. the impact of HIV/AIDS on CVD, the prevalence of rheumatic heart disease diagnosed in adulthood, the spectrum of conditions leading to heart failure, but also resulted in important publications on the normal ECG in Africans and the lipid profile in that population. I expanded my population studies (under the umbrella of the 'Heart of Africa studies') to other African countries.

One of my main research areas over the past 15 years has been on cardiac disease and pregnancy, with a particular focus on Peripartum Cardiomyopathy a disease common in African women with a mortality of 20%. Close collaboration with a biologist led to unique translational research involving several animal models and human biological samples providing breakthrough information on the pathogenesis of human PPCM. This led to a promising new therapy in less than a decade.

Frank ACCURSO

Professor
Pediatrics, University of Colorado, and Cystic Fibrosis Clinical Research, Children's Hospital Colorado
Aurora, USA

Dr. Frank Accurso is Professor of Pediatrics at the University of Colorado School of Medicine and Director of Cystic Fibrosis Clinical Research at Children's Hospital Colorado. Cystic Fibrosis is an inherited condition that results in death at an early age primarily through lung disease. Dr. Accurso's research has contributed to early diagnosis in CF through newborn screening in order to prevent development of complications and allow treatment to begin in young children. He has also contributed to understanding infection and the body's response to infection in order to develop new treatments for CF. Throughout these investigations, he has worked closely with academicians, government agencies and industry partners to establish a team approach to improving life for individuals with CF. For the past eleven years he has worked on a precision medicine approach to treatment of patients with a specific mutation, one of hundreds, in CF. This work, involving thousands of patients and researchers, resulted in the approval of a very effective new drug, Kalydeco, (Vertex Pharmaceuticals) in 2012. Dr. Accurso receives support for research from the Cystic Fibrosis Foundation, the National Institutes of Health, a number of private companies and the Children's Hospital Colorado Research Institute. He is also a member of the Board of Trustees of the Cystic Fibrosis Foundation. Dr. Accurso is most grateful to all the individuals with CF and their families for their support through the years.

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Partnerships in cystic fibrosis care and research: An academician's perspective on KalydecoTM and beyond

Cystic fibrosis (CF), a single gene disorder affecting several organs, leads to early death. While CF is a rare condition, successes in CF research provide insight into potentially productive pathways for other serious diseases. Kalydeco TM, a recently approved drug, treats the basic pathophysiologic defect in CF by improving function of the abnormal protein, the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR). Tracing development of KalydecoTM, termed a CF modulator, highlights the importance of partnerships. The discovery of the CFTR followed identification of the CF gene in 1989. Ten more years of intense efforts characterizing the CFTR genetically, in model systems, and in affected individuals, provided a "biologic tool kit" that was used to develop new treatments. In 1999, Bob Beall, Chief Executive Officer of the US CF Foundation, proposed the concept of "Venture Philanthropy" to make CF research attractive to industry by providing initial funding. Venture philanthropy involved careful monitoring of milestones. In the case of KalydecoTM, high throughput screening was used by Aurora BioScience, later acquired by Vertex Pharmaceuticals, to identify candidates that were improved by medicinal chemistry. Testing of Kalydeco was possible initially only because of the CF Foundation Therapeutics Development Network which standardized procedures and protocols and guided dozens of centers around the world in clinical research. Other key elements were the use of the US CF Foundation patient registry, involvement of personnel at CF care centers, and tremendous enthusiasm for research by individuals with CF and their families. Another four to ten CFTR modulators will likely be developed in the coming years through venture philanthropy. Beyond that, there is great interest in gene and RNA editing and stem cell biology, since it is recognized that a cure rather than a treatment will require these techniques. Partnerships involving individuals with CF, their families, private foundations, governmental agencies, private and public payers for care, caregivers, hospitals and universities have all played a role in CF research and will likely continue to play a role in the future.

Frederick DEWEY

Director
Translational Genetics, Genetics Center, Regeneron Pharmaceuticals, Inc.
Tarrytown, USA

Frederick Dewey received his AB in Chemistry and Physics from Harvard University, and MD, with concentration in cardiovascular and pulmonary sciences, from Stanford University. He received clinical training in internal medicine and cardiovascular medicine at Stanford Hospital and Clinics, and research training in human genetics via the Stanford Clinical Investigator Pathway. His research work has focused on gene discovery in familial and complex cardiovascular disease using high throughput sequencing, and application of exome and genome sequencing in clinical care. He joined the Regeneron Genetics Center, a wholly-owned subsidiary of Regeneron Pharmaceuticals, in 2014 as Director of Translational Genetics.

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Natural human gene knockouts in the discovery of new drug targets

It has been estimated that every human being carries 100-200 "natural human gene knockouts"-DNA variants in protein-coding regions of the genome that partially or completely inactivate gene products. When coupled with information about clinical phenotypes, these natural human gene knockouts can illuminate biological function of gene products and further our understanding of disease pathogenesis. In select cases, gene-inactivating mutations that confer protection from disease may guide the way to new therapeutic targets or confirm existing targets. With the advent of next generation sequencing facilitating genomics on massive scales, properly powered studies of human knockouts are finally possible. Here, we describe an integrated approach to sequencing-based discovery of naturally occurring human knockouts that spans genetic trait architectures, from small collections of highly related individuals with extreme phenotypic traits, to geographically- and reproductively-isolated "founder" populations harboring frequent instances of highly impactful alleles, to large scale sampling of outbred populations with broad allelic diversity and rich "real-world" phenotypic data provided by electronic medical records. In collaboration with the Geisinger Health System and academic groups worldwide, we report early findings from whole exome sequencing of over 30,000 individuals spanning these population architectures and study designs. We find that the majority of genes encoding drug targets and related proteins harbor mutations that are predicted to partially or completely inactivate their gene products. We highlight examples of protective and harmful clinical associations with inactivating mutations in these genes that support and invalidate therapeutic targets. We also describe exome-wide scans for gene-inactivating mutations that nominate novel candidate genes and targets in multiple cardiometabolic traits and diseases. These early insights suggest that large-scale discovery of human gene knockouts will contribute to the next wave of drug target discovery and validation.

Eric REIMAN

CEO, Banner Alzheimer´s Institute
Professor, Department of Psychiatry, University of Arizona
Phoenix, USA

Dr. Reiman is Executive Director of the Banner Alzheimer's Institute, Chief Executive Officer of Banner Research, Clinical Director of Neurogenomics at the Translational Genomics Research Institute, Professor of Psychiatry at the University of Arizona, University Professor of Neuroscience at Arizona State University, and Director of the Arizona Alzheimer's Consortium. His research interests include brain imaging, genomics, the unusually early detection and tracking of Alzheimer's disease (AD), and the accelerated evaluation of AD prevention therapies. He is an author of more than 250 publications, a principal investigator of several NIH grants, a leader of the Alzheimer's Prevention Initiative (API), and a recipient of the Potamkin Prize.

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Preventing Alzheimer's together

What will it take to find an effective Alzheimer's disease (AD) prevention therapy within ten years? It will take brain imaging measurements, other biomarkers, and cognitive measurements that track the preclinical stages of AD; prevention trials of promising disease-modifying treatments using those cognitive endpoints in persons at the highest imminent risk for the clinical onset of AD; incorporation of a theragnostic biomarker development strategy into these and other therapeutic trials to provide the evidence needed for regulatory agencies to conclude that a treatment's biomarker effects in 24-month prevention trials are reasonably likely to predict a clinical benefit; use of these reasonably likely surrogate endpoints to rapidly evaluate the range of promising prevention therapies in wider group of persons at risk for; enrollment registries to accelerate enrollment in the trials; new ways for public and private stakeholders to work together; data and sample sharing after the trials are over; and consensus building mechanisms about the appropriate ways to conduct AD prevention trials. It will take the right sense of urgency, funding and financial incentives and investigational therapies that turn out to work. It will take us all. My colleagues and I have been using brain imaging, other biomarkers, cognitive tests, and improved data analysis techniques to detect and track the earliest changes in cognitively unimpaired persons at differential genetic risk for late-onset and autosomal dominant AD, helping to inform the understanding of preclinical AD and set the stage for the accelerated evaluation of AD prevention therapies. We created the Alzheimer's Prevention Initiative (API) to start evaluating promising prevention therapies, find even faster ways to evaluate the range of promising therapies, and find ones that work as quickly as possible. With support from NIH, philanthropy and industry partners, API includes a potentially license-enabling prevention/theragnostic biomarker development trial of an anti-amyloid therapy in cognitively unimpaired members of the world's largest early onset autosomal dominant AD kindred, a complementary prevention/theragnostic biomarker development trial in cognitively unimpaired APOE4 homozygotes, data and sample sharing commitments, and large Alzheimer's prevention registries to support enrollment in these and other trials. In this presentation, I will review our scientific journey, suggest how public and private stakeholders can work together to address their complementary and converging goals, and propose what it will take to find effective AD prevention therapies as soon as possible.

Ricardo DOLMETSCH

Global Head
Department of Neuroscience, Novartis Institutes for Biomedical Research
Cambridge, USA

Dr. Dolmetsch's group is responsible for leveraging advances in human genetics to model and treat neuropsychiatric and neurodegenerative diseases. Areas of focus include genomics, induced pluripotent stem cells and brain circuitry.
Ricardo joined Novartis in August 2013 from the Stanford University School of Medicine, where he was an Associate Professor of Neurobiology. He was also the Senior Director of Molecular Networks at the Allen Institute for Brain Research. His lab studied the molecular roots of autism and other neurodevelopmental disorders, including the role of calcium channel signaling. They recently used induced pluripotent stem cells to develop an in vitro model of Timothy syndrome, a rare disease with a variety of symptoms, including the characteristic features of autism.
Ricardo received his B.S. from Brown University in 1990 and Ph.D. from Stanford University in 1997. He completed a postdoctoral fellowship at Harvard Medical School in Michael Greenberg's lab.

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A vision for the future of drug discovery in neuroscience

Drug development for diseases of the nervous system has been particularly difficult over the last two decades. It has been hampered by a poor understanding of the pathophysiology of disease, the lack of predictive preclinical models, and the difficulty of conducting clinical trials. At Novartis we have rebuilt the neuroscience team with the goal of innovating in three important areas. First, we are leveraging advances in human genetics to identify drug targets that are closely tied to neurologic and psychiatric disease and therefore have an increased chance of leading to drugs that work in the clinic. Second, we are investing in making more predictive preclinical models by taking advantage of innovations in stem cell technology to develop human neuronal models of psychiatric and neurological disease. Finally we are exploring new ways of conducting clinical trials by carefully selecting patient populations, developing new ways of measuring clinical outcomes and using innovative clinical trial designs.
I will present examples of projects where we are starting to put these approaches into practice. Our goal is to improve the success of drug development in neuroscience by systematically improving multiple stages of the drug development process.

Mene PANGALOS

Executive Vice-President
Innovative Medicines & Early Development Unit, AstraZeneca
London, UK

Mene Pangalos, Ph.D. is Executive Vice President of AstraZeneca's Innovative Medicines and Early Development Biotech Unit. A member of the company's Senior Executive team, Mene has overall responsibility for the company's small molecule discovery research and early development activities.

As one of AstraZeneca's leading scientists Mene has published more than 140 peer-reviewed articles in scientific journals and has served as an editor of books and journals in neuroscience. Mene completed his undergraduate degree in Biochemistry at Imperial College of Science and Technology and earned a PhD in Neurochemistry from the Institute of Neurology, both at the University of London. He is a Visiting Professor of Neuroscience at King's College London.

In the UK, Mene sits on the Medical Research Council (MRC), the council for the National Centre for Universities and Business, the Prime Minister's Research Champion Group for Dementia and is part of the Ministerial Industry Strategy Group. He is also a Fellow of the Society for Biology, an Associate of the Royal College of Science and holds memberships with the American Society of Neuroscience and the British Pharmacology Society.

Throughout his career Mene has been recognised for driving forward scientific innovation. At Wyeth his group was recognised by R&D Directions magazine as having the Best Central Nervous System Pipeline, while in 2008 Mene was awarded an Innovation in Industry award by the New York Academy of Sciences for his outstanding contribution to neuroscience research and drug discovery.

Since joining AstraZeneca in 2010, Mene has been instrumental in transforming the company's commitment to science. He has led the transformation of R&D productivity through the development and implementation of the "5R" framework (recently published in Nature Reviews Drug Discovery); driven greater collaboration with academic, NGO and peer organisations; pioneered programmes to promote more open innovation and fostered a science driven culture that rewards truth-seeking behaviours.

Mene is also overseeing the creation of AstraZeneca's new £330 million research centre in Cambridge - a state of the art of facility designed to stimulate collaborative scientific innovation and which will play an important role in the future success of the UK life science industry.

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Creating an open research ecosystem where science thrives

Creating a permeable research environment where scientists share their ideas more freely, collaborate on projects and drive scientific innovation is key to delivering the next generation of life changing medicines. In this address Dr. Mene Pangalos, Executive Vice President of AstraZeneca's IMED Biotech Unit will highlight the pioneering approaches AstraZeneca has taken to transform its research culture and connect like-minded scientists from industry, academia and the not-for-profit sector.

Scientific leadership and high quality scientific collaboration are the foundations of successful research and development. This presentation will highlight AstraZeneca's commitment to scientific depth and call out examples of some truly ground-breaking collaborations that have been developed to share compounds and expertise. These include agreements with the Medical Research Council (UK) and with the National Institute of Health (US), which have enabled academic researchers, financed with public funds, to explore the potential of discontinued compounds that could lead to the development of new medicines for disease areas such as Alzheimer's, cancer and peripheral artery disease.

The presentation will also outline AstraZeneca's innovative agreement with the Karolinska Institut (KI) that challenged the historical collaboration model of "big Pharma" supplying funds to support specific research. Instead AstraZeneca placed their scientists into the KI labs, working side by side with KI scientists to address issues with CV disease - a completely different approach to solving the complex challenges and unmet needs in this disease area.

Finally, the presentation will highlight how AstraZeneca's Open Innovation platform is providing new opportunities to link AZ expertise, resources and technology with external scientists to deliver scientific breakthroughs.

The aim of the presentation is to stimulate further dialogue about how applying scientific rigour and embracing innovative collaborations can not only change the way pharmaceutical and academic researchers approach the discovery of new treatments, but by doing so redefine the future of healthcare.

Richard HORTON

Editor-in-Chief
Editorial Department, The Lancet
London, UK

Richard Horton is Editor-in-Chief of The Lancet. *He was born in London and is half Norwegian. He qualified in physiology and medicine with honours from the University of Birmingham in 1986. He joined* The Lancet *in 1990, moving to New York as North American Editor in 1993. Richard was the first President of the World Association of Medical Editors and he is a Past-President of the US Council of Science Editors. He is an honorary professor at the London School of Hygiene and Tropical Medicine, University College London, and the University of Oslo. He has also received honorary doctorates in medicine from the University of Birmingham, UK, and the Universities of Umea and Gothenburg in Sweden. In 2011, he was appointed co-chair of the UN's independent Expert Review Group on Information and Accountability for Women's and Children's Health, part of whose remit is to monitor progress of the UN Secretary-General's Global Strategy for Women's and Children's Health. He is a Senior Associate of the UK health-policy think-tank, the Nuffield Trust. Richard received the Edinburgh medal in 2007 and the Dean's medal from Johns Hopkins School of Public Health in 2009. He has written two reports for the Royal College of Physicians of London:* Doctors in Society *(2005) and* Innovating for Health *(2009). He wrote* Health Wars *(2003) about contemporary issues in medicine and health, and he has written regularly for* The New York Review of Books *and the* TLS. *He has a strong interest in global health and medicine's contribution to our wider culture. In 2011, he was elected a Foreign Associate of the US Institute of Medicine.*

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Partnerships for global health: successes, questions, and opportunities

The Millennium Development Goal era has seen great success as political commitments became translated into health improvements across broad reaches of infectious disease and maternal and child health. Partnerships were often critical to these achievements. But not all partnerships have worked. And difficult questions about partnerships have not always been satisfactorily answered. To realize future opportunities in global health, a new approach to partnership is required – one in which rigorous independent accountability is embedded as part of a commitment to collaboration.

Michael WATSON

Executive Vice-President
Vaccination Policy and Advocacy, Sanofi Pasteur
Lyon, France

Michael Leads the Sanofi Pasteur Vaccination Policy team that is responsible for the interface between Sanofi Pasteur and Global and Regional Governmental and Non-Governmental Public Health organisations involved in immunisation Policy, Funding, Financing and, Implementation, Advocacy and issue resolution. He recently completed his term as chair of the Vaccines Committee of the International Federation of Pharmaceutical Manufacturers Association, (IFPMA), he leads the Pandemic Influenza Preparedness Group of the IFPMA, is on the Board of Vaccines Europe and leads their R&D Working Group, is a Steering Committee of the New York Academy of Sciences Vaccines Group and is Chair of the Sanofi Pasteur HIV steering committee.

He is a UK trained physician who led the teams that developed and licensed Pediacel® in the UK and Gardasil® in Europe. He has worked on most classes of vaccines, including Hexavalent infant vaccines.

He was previously Head of R&D and US site head at the UK Biotech company, Acambis PLC, based in Boston, US, until it was acquired by sanofi pasteur. At Acambis he oversaw the licensure of the smallpox vaccine ACAM2000®, the R&D of Chimerivax® based vaccines against Japanese encephalitis, and West Nile Fever, an M2e universal Influenza vaccine, ACAM-FLU-A™, and the worlds most advanced C. difficile vaccine, as well as a number of pre-clinical stage projects including an HSV-2 vaccine and a number of innovative new vaccine delivery platforms.

He is currently chairing the Vaccine's Europe (VE) R&D working group which is working with EATRIS, Sclavo Vaccines Association (SVA) and European Vaccine Institute (EVI) to lead the FP7 funded IPROVE project intended to develop a vaccines R&D roadmap for Europe.

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Intended and unintended consequences of global health alliances in the vaccination ecosystem and implications for all public health partnerships

Vaccination is almost universally accepted as one of public health's best accelerators. In most settings it qualifies as a true public good. Because vaccination and public health are social and community undertakings, and not purely medical events, they rely on successful partnership for their success. Vaccination is not a case by case decision taken between healthcare provider and patient but it is rather a collective undertaking governed by a national, regional or global policy. Delivery and access to vaccination rely on having health systems and national infrastructures able to get vaccines to people and people to vaccines. Vaccine research, development, supply, delivery and monitoring require close partnership between the researchers, funders, financers, purchasers, providers, producers and people. Since the beneficiaries are more often healthy people than patients, vaccination requires specific planning and social sciences-based research, expertise and engagement to ensure that vaccination is accepted and ideally demanded by people. Finally, the more effectively that vaccination can be integrated vertically and horizontally the better it is for all of healthcare. Whilst partnership is a constant of vaccination, there are some partnerships that stand out more than others. The two eradication efforts were based on strong partnership. Smallpox is won and Polio is tantalizingly close. The meningococcal C conjugate development, initially for the UK, was a great example of how policy visionaries, national and industry scientists and regulators combined to find the most pragmatic route to address a public health problem. This was later adapted and repeated through the meningococcal A conjugate vaccine development project for sub-Saharan Africa. GAVI is by definition an alliance that brings together industry, policy makers, financers, CSOs, advocates, funders and others to address the pre-existing market-failure in the very poorest countries. SARS, HIV, TB, Malaria, Ebola, and pandemic flu are all examples of how important horizontal and vertical partnership are to vaccination but Ebola has served to remind us that the partnerships are far from idea. There are also many examples of partnership through technology transfers, coverage initiatives and initiatives to support affordability of vaccination. Public health partnerships are long term. Like marriages we must accept that few will be perfect and that they will bring us both intended and unintended consequences. Success relies on us being prepared to continuously communicate, express our needs and concerns and to respond and adapt. Our experience in vaccination partnership has given us a few learnings that could be applied to all public health partnerships:

Recognize the huge value of successful partnership but also recognize that we must look for and adapt to unintended consequences.

Successful partnerships start and stay with a clear shared vision of the intent with accompanying and aligned goals and metrics.

Partnership should reward quality, reliability, innovation and impact as well as affordability.

Incentives and metrics of success must be aligned with the goals and we must beware of being distracted by cognitively attractively simple metrics that are not aligned with the holistic goals.

Be sure to balance short term static efficiency goals with longer term dynamic efficiency goals.

Finally all partners should be fully into the ecosystem stewardship and those with perceived conflicts of interest should be managed rather than excluded.

Garry JENNINGS

Director and CEO
Baker IDI Heart and Diabetes Institute
Melbourne, Australia

Garry Jennings AO is the Director and CEO of the Baker IDI Heart and Diabetes Institute in Melbourne, an independent, internationally-renowned medical research facility whose work extends from the laboratory to wide-scale community studies, and clinical practice. He is past President of the Association of Australian Medical Research Institutes, the High Blood Pressure Research Council of Australia, the Asia Pacific Society of Hypertension and Head of a WHO Collaborating Centre for Research and Training in Cardiovascular Health.

A cardiologist, he has a distinguished career in clinical practice and research. His research interests cover the causes, prevention and treatment of cardiovascular diseases and have received national and international awards. He has published several books on heart disease for the general public and over 400 research publications cited more than 17000 times.

Australia has high life expectancy and low rates of cardiovascular disease and diabetes but this is not true of the aboriginal and Torres Strait communities where there is a significant gap in life expectancy largely due to these conditions. Almost a decade ago Garry Jennings established an aboriginal health research program in Alice Springs. This occurred with the support not only of mainstream research funding agencies but also Trusts, Foundations, and wealthy individuals from the major urban centres on the East Coast of Australia. The mission is to help close the gap and the program has since spread nationally and internationally to groups addressing indigenous health around the world. Locally a consortium of aboriginal controlled health services, local hospital services, other NGO's and universities has been formed by Baker IDI in order to provide scale and efficiency for research in remote areas.

NOTES

Australian Indigenous Health – global challenge and local solutions

The health of the indigenous population aboriginal and Torres Strait Islanders is Australia's most blatant health disparity. In 2010–12, Indigenous life expectancy was 69.1 years for males and 73.7 years for females. The gap in life expectancy between Indigenous and non-Indigenous Australians was 10.6 years for males and 9.5 years for females. Between 2005–07 and 2010–12, there was a small reduction in the gap of 0.8 years for males and 0.1 years for females. Between 2008 and 2012, chronic disease (including circulatory disease, cancer, diabetes, kidney and respiratory diseases) accounted for 70 per cent of Indigenous deaths and accounted for 81 per cent of the gap in death rates between Indigenous and non-Indigenous Australians. Since 2006 the gap widened for cancer mortality and there has been no improvement for diabetes or external causes of death such as suicide and transport accidents[1].

The underlying causes are both complex and multiple, it is unhelpful to consider this solely from a medical perspective or solely as a consequence of dispossession, disharmony, poverty and other socio-cultural changes inflicted on the communities. These are all important. From a scientific perspective possible causes range from those with an anthropological base (Thrifty Gene), social and cultural influences arising from disadvantage, biological factors, particularly the classical cardiovascular risk factors, early life events and epigenetics to health system failure and access. It is important to better understand the relative contributions to define the best targets for improvement. This requires simultaneous discovery, evaluation and implementation. Nowhere in the health system should the cycle between discovery and evidence based implementation be shorter. Much of this research will be at the translational and health services end but there is also a huge potential in a better understanding of the underlying mechanisms, particularly early life events and the probable role of epigenetic changes influencing gene function affecting cardiovascular and metabolic health of young and middle aged adults.

Baker IDI a multidisciplinary cardiovascular, diabetes and metabolism research institute based in Melbourne turned its attention to this disparity 8 years ago, establishing a facility in Alice Springs and developing links with local communities, health providers and government as well as other research institutions. Collectively the alliance has scale that no single institution could bring to this remote location. The work has harnessed goodwill amongst local philanthropists, and Foundations as well as mainstream research funding. Similar disparities occur to a greater extent amongst indigenous and minority communities around the world leading to partnerships in Malaysia, North America and New Zealand. Hopefully local solutions will help inform healthcare and research tailored to indigenous communities around the world

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Kartik CHANDRAN

Associate Professor
Department of Microbiology & Immunology, Albert Einstein College of Medecine
Bronx, USA

Dr. Kartik Chandran is Associate Professor of Microbiology and Immunology and Harold and Muriel Block Faculty Scholar in Virology at the Albert Einstein College of Medicine. He received his PhD at the University of Wisconsin-Madison and carried out postdoctoral research at Harvard Medical School and the Brigham and Women's Hospital in Boston, before assuming his position at Einstein in 2007. Dr. Chandran has spent his entire research career studying viruses. In the past decade, he and his group have made seminal contributions to understanding how Ebola virus invades its host cells, including the discovery of a novel host-programmed disassembly mechanism used by Ebola and the identification of lysosomal cholesterol transport protein Niemann-Pick C1 (NPC1) as the long-sought filovirus receptor. The Chandran Lab is currently exploring the implications of these findings for co-evolution between Ebola and its animal hosts, genetic resistance of humans to Ebola infection, and the discovery of broad-spectrum drugs targeting Ebola virus.

NOTES

Studying Niemann-Pick Type C disease to illuminate Ebola

The ongoing Ebola epidemic in West Africa has claimed over 10,000 lives, highlighting the urgent need for antiviral drugs that block infection by Ebola virus. Unfortunately, our limited understanding of the mechanism used by Ebola to invade host cells challenges the development of such drugs. To help overcome this obstacle, we performed genetic screens for human host factors essential to Ebola infection. Unexpectedly, our strongest hit in these screens was Niemann-Pick C1 (NPC1), a highly conserved lysosomal membrane protein involved in cholesterol transport. Genetic loss of NPC1 is a cause of the lysosomal storage disorder Niemann-Pick type C (NPC) disease in humans, and the extensive body of work on NPC1 and NPC disease has played a crucial role in advancing our studies. Our studies with primary cells from NPC patients and in a mouse model of NPC disease have revealed that NPC1 is a critical receptor for Ebola invasion into cells, and shed light on the unprecedented mechanism by which Ebola coöpts NPC1. In collaboration with NPC researchers, we are developing small molecule inhibitors of NPC1 with antiviral potential. Our ongoing experiments show that NPC1 is a genetic determinant of Ebola susceptibility in bats, the suspected natural hosts for Ebola, and suggest the remarkable possibility that variation in bat NPC1 genes has been shaped by an evolutionary arms race between virus and host. Finally, we are exploring the possibility, suggested by our mouse and bat studies, that mutations in NPC1 could help humans survive Ebola infection.

SPEAKER & CHAIR LIST

ACCURSO Franck
Pediatrics, University of Colorado, and Cystic Fibrosis Clinical Research, Children's Hospital Colorado, Aurora, USA
Frank.Accurso@ucdenver.edu

CHANDRAN Kartik
Department of Microbiology & Immunology, Albert Einstein College of Medecine Bronx, USA
kartik.chandran@einstein.yu.edu

CHANGEUX Jean-Pierre
Kavli Institute for Brain & Mind UCSD & Institut Pasteur Paris, France
changeux@pasteur.fr

CHIEN Kenneth R.
Department of Cell & Molecular Biology & Medicine, Karolinska Institutet Stockholm, Sweden
kenneth.chien@ki.se

CHRISTEN Yves
Fondation IPSEN
Paris, France
yves.christen@ipsen.com

DEWEY Frederick
Translational Genetics, Genetics Center, Regeneron Pharmaceuticals, Inc. Tarrytown, USA
frederick.dewey@regeneron.com

DOLMETSCH Ricardo
Department of Neuroscience, Novartis Institutes for Biomedical Research Cambridge, USA
ricardo.dolmetsch@stanford.edu

FISCHER Alain
Collège de France & Unité d'Immunologie et Hématologie Pédiatrique, Assistance Publique – Hôpitaux de Paris, Université Paris Descartes, Paris, France
alain.fischer@inserm.fr

FREY Noelle
Perelman Center for Advanced Medicine, University of Pennsylvania Philadelphia, USA
noelle.frey@uphs.upenn.edu

HAMSTEN Anders
Vice-Chancellor, Karolinska Institutet
Stockholm, Sweden
rektor@ki.se

HANSSON Göran K.
Department of Medicine, Karolinska Institutet
Stockholm, Sweden
goran.hansson@ki.se

HEMPEL Chris
Addi & Cassi Fund
Reno, USA
chris@hempelfamily.com

HORTON Richard
Editorial Department, The Lancet
London, UK
richard.horton@lancet.com

JENNINGS Garry
Baker IDI Heart and Diabetes Institute
Melbourne, Australia
garry.jennings@bakeridi.edu.au

LENDAHL Urban
Department of Cell and Molecular Biology (CMB), Karolinska Institutet
Stockholm, Sweden
Urban.lendahl@ki.se

LJUNGGREN Hans-Gustaf
Human NK Cells in Health and Disease Group, Karolinska Institutet
Stockholm, Sweden
hans-gustaf.ljunggren@ki.se

MacLAREN Robert
Gene and Stem Cell Therapy for Retinal Diseases Laboratory, Oxford University
Oxford, UK
robert.maclaren@eye.ox.ac.uk

PANGALOS Mene
Innovative Medicines & Early Development Unit, AstraZeneca
London, UK

REIMAN Eric
Banner Alzheimer´s Institute, and Department of Psychiatry, University of Arizona
Phoenix, USA
eric.reiman@bannerhealth.com

RUBIN Lee
Department of Stem Cell and Regenerative Biology, Harvard University
Cambridge, USA
lee_rubin@harvard.edu

SCHWARZ-ROMOND Thomas
Executive Publisher, Elsevier – Journals
Berlin, Germany
t.schwarz-romond@elsevier.com

SLIWA Karen
Hatter Institute for Cardiovascular Research in Africa, University of Cape Town
Cape Town, South Africa
karen.sliwa-hahnle@uct.ac.za

SMITH Orla M.
Editorial Department, AAAS/ Science Translational Medicine
Washington, USA
osmith@aaas.org

SOLOMON Susan L.
The New York Stem Cell Foundation (NYSCF)
New York, USA

WATSON Michael
Vaccination Policy and Advocacy, Research & Development, Sanofi Pasteur
Lyon, France
Michael.Watson@sanofipasteur.com

WEDELL Anna
Department of Molecular Medicine and Surgery, Karolinska Institutet
Stockholm, Sweden
Anna.Wedell@ki.se

WIGLER Michael H.
Human Genetic Disorders, Population Genetics, Cancer Genomics Program, Cold Spring Harbor Laboratory, Cold Spring Harbor, USA
wigler@cshl.edu

XIAO Rui-Ping
Laboratory of Signal Transduction, Institute of Molecular Medicine,
Peking University
Beijing, China
xiaor@grc.nia.nih.gov

PARTICIPANT LIST

From the Karolinska Institutet:

ALEXEYENKO Andrey	Science for Life Laboratory (SciLifeLab)	andrej.alekseenko@scilifelab.se
ALVES LOPES João Pedro	Department of Woman's and Children's Health (KBH)	joao.lopes@ki.se
AMIN Risul	Department of Clinical Science, Intervention and Technology (CLINTEC)	risul.amin@ki.se
ANNUSVER Karl	Biomedicine Master	karl.annusver@gmail.com
ARVIDSSON Per	Department of Medical Biochemistry and Biophysics (MBB)	Per.Arvidsson@scilifelab.se
ASGHAR Muhammad	Department of Medicine, Solna (MedS)	Asghar.Muhammad@ki.se
AZIMI Alireza	Department of Oncology-Pathology (OnkPat)	alireza.azimi@ki.se
BARRENETXEA Jon	Department of Neuroscience (Neuro)	jonbarrene@hotmail.com
BARRIENTOS-SOMARRIBAS Mauricio	Department of Cell and Molecular Biology (CMB)	mauricio.barrientos@ki.se
BASINOU Vasiliki	Department of Physiology and Pharmacology (FyFa)	vasiliki.basinou@ki.se
ELIKOV Sergey	Department of Cell and Molecular Biology (CMB)	sergey.belikov@ki.se
BERSANI Cinzia	Department of Oncology-Pathology (OnkPat)	cinzia.bersani@ki.se
BIGNAMI Francesca	Unit for Bioentrepreneurship, Department of Learning, Informatics, Management and Ethics (LIME)	francesca.bignami@ki.se
BRODIN Johanna	Department of Microbiology, Tumor and Cell Biology (MTC)	johanna.brodin@ki.se

BRUNNER Andrä	Department of Cell and Molecular Biology (CMB)	andra.brunner@ki.se
BUYANDELGER Byambajav	Integrated Cardio Metabolic Centre (ICMC), Department of Medicine	byambajav.buyandelger@ki.se
CECCATELLI Sandra	Department of Neuroscience (Neuro)	sandra.ceccatelli@ki.se
CHEMIN Karine	Center for Molecular Medicine (CMM)	karine.chemin@ki.se
CHEN Geng	Department of Cell and Molecular Biology (CMB)	geng.chen@ki.se
CHEN Xingdong	Department of Medical Epidemiology and Biostatistics (MEB)	xingdong.chen@ki.se
CHEN Xu	Department of Medical Epidemiology and Biostatistics (MEB)	xu.chen@ki.se
CHERNOBROVKIN Alexey	Department of Medical Biochemistry and Biophysics (MBB)	alexey.chernobrovkin@ki.se
CHNG Junhong	Department of Microbiology, Tumor and Cell Biology (MTC)	junhong.chng@ki.se
CHRAST Roman	Department of Neuroscience (Neuro)	roman.chrast@ki.se
CRISBY Milita	Department of Neurobiology, Care Sciences and Society (NVS)	milita.crisby@ki.se
CROSETTO Nicola	Department of Medical Biochemistry and Biophysics (MBB)	nicola.crosetto@scilifelab.se
DAHLLÖF Göran	Department of Dental Medicine (Dentmed)	goran.dahllof@ki.se
DARABI Hatef	Department of Medical Epidemiology and Biostatistics (MEB)	hatef.darabi@ki.se
DIAZ GALLO NORLEN Lina Marcela	Department of Medicine, Solna (MedS)	lina.diaz@ki.se
DOUAGI Lyadh	Department of Medicine, Huddinge (MedH)	iyadh.douagi@ki.se
DYCZYNSKI Matheus	CCK - Department of Oncology-Pathology (OnkPat)	matheus.dyczynski@ki.se
EDLUNDH-ROSE Esther	Centre for Allergy Research	esther.edlundh-rose@ki.se
EKBLAD Åsa	Department of Clinical Sciences, Intervention & Technology	asa.ekblad@ki.se

ELSÄSSER Simon	Department of Medical Biochemistry and Biophysics (MBB)	simon.elsasser@ki.se
ENGSTRAND Lars	Department of Microbiology, Tumor and Cell Biology (MTC)	lars.engstrand@ki.se
FAGERSTEDT Sara	Department Clinical Science and Education, Södersjukhuset	sara.fagerstedt@ki.se
FALCK Sandra	Department of Biosciences and Nutrition (BioNut)	sandra.falck@ki.se
FARAH Abdirahman	Biomedicine Master	Abdirahman.farah@live.se
FERNANDEZ Alejandro	Department of Microbiology, Tumor and Cell Biology (MTC)	alejandro.woodbridge@ki.se
FOO THAMS Kylie	Department of Medicine	kylie.foo@ki.se
FUKS Jonas	Department of Medicine, Huddinge (MedH)	fuks@kth.se
GARCIA PASCUAL Carmen Maria	Department of Woman's and Children's Health (KBH)	carmen.garcia.pascual@ki.se
GERTOW Karl	Department of Medicine, Solna (MedS)	karl.gertow@gmail.com
GRACIAS Aileen	Department of Neuroscience (Neuro)	aileen.gracias@ki.se
GU Harvest	Department of Molecular Medicine and Surgery (MMK)	harvest.gu@ki.se
GUO Shiyuan	Biomedicine Master	shiyuan.guo@u.nus.edu
GUSTAFSSON SHEPPARD Nina	Department of Medical Biochemistry and Biophysics (MBB)	nina.sheppard@scilifelab.se
GYARMATI Peter	Department of Microbiology, Tumor and Cell Biology (MTC)	peter.gyarmati@ki.se
GÖNDÖR Anita	Department of Microbiology, Tumor and Cell Biology (MTC)	anita.gondor@ki.se
HAMBARDZUMYAN Karen	Unit for Clinical Therapy Research, Inflammatory Diseases (ClinTRID)	karen.hambardzumyan@ki.se
HAMMARSJÖ Anna	Department of Molecular Medicine and Surgery (MMK)	anna.hammarsjo@ki.se

HASSAN Halima	Biomedicine Masters	halima.ayan@gmail.com
HE Xiaobing	Department of Cell and Molecular Biology (CMB)	Xiaobing.he@ki.se
HEIDARI Ghazal	Department of Laboratory Medicine (LABMED)	ghazal.heidari.hamedani@ki.se
HELLEDAY Thomas	Department of Medical Biochemistry and Biophysics (MBB)	thomas.helleday@ki.se
HOREJS Christine-Maria	Department of Medical Biochemistry and Biophysics (MBB)	christine-maria.horejs@ki.se
HU Yi	Department of Microbiology, Tumor and Cell Biology (MTC)	yi.hu@ki.se
HUSSAIN Shafaat	Department of Medicine	shafaat.hussain@ki.se
HÖKFELT Tomas	Department of Neuroscience (Neuro)	Tomas.Hokfelt@ki.se
ISLAM Md. Shahidul	Department of Clinical Sciences and Education, Södersjukhuset	shahidul.islam@ki.se
IVARSSON Niklas	Department of Physiology and Pharmacology (FyFa)	niklas.ivarsson@ki.se
JAFF Nasren	Department of Clinical Neuroscience (CNS)	nasren.jaff@ki.se
JERHAMMAR Fredrik	Department of Oncology-Pathology (OnkPat)	fredrik.jerhammar@ki.se
JIANG xia	Institute of Environmental Medicine	xia.jiang@ki.se
JIAO Xiang	Department of Molecular Medicine and Surgery (MMK)	xiang.jiao@ki.se
JOHANSSON Anne-Sofie	Department of Medicine, Huddinge (MedH)	anne-sofie.johansson@ki.se
JOUHILAHTI Eeva-Mari	Department of Biosciences and Nutrition (BioNut)	eeva-mari.jouhilahti@ki.se
KAISER Ylva	Department of Medicine, Solna (MedS)	ylva.kaiser@ki.se
KARAMPELIAS Christos	Department of Cell and Molecular Biology (CMB)	christos.karampelias@ki.se
KITAMBI Satish	Department of Microbiology, Tumor and Cell Biology (MTC)	satish.kitambi@ki.se

KOZANLI Eda	Biomedicine Master - Department of Physiology and Pharmacology (FyFa)	eda.kozanli@stud.ki.se
KRAUT Claudia	Department of Cell and Molecular Biology (CMB)	claudia.kraut@gmx.net
KUBLICKIENE karolina	Department of Medicine, Solna and Department of Clinical Science, Intervention and Technology (CLINTEC)	karolina.kublickiene@ki.se
KUMAR Lalit	Department of Woman's and Children's Health (KBH)	Lalit.kumar@ki.se
KUMAR Parvin	Department of Molecular Medicine and Surgery (MMK)	parvin.net@gmail.com
KUREK Magdalena	Department of Woman's and Children's Health (KBH)	magdalena.kurek@ki.se
LAGUNA FERNANDEZ Andres	Department of Medicine, Solna (MedS)	andres.laguna.fernandez@ki.se
LANNER Fredrik	Department of Clinical Science, Intervention and Technology (CLINTEC)	fredrik.lanner@ki.se
LARSSON Lars	Department of Physiology and Pharmacology (FyFa)	lars.larsson@ki.se
LEND Kristina	Department of Medicine, Solna (MedS)	kristina.lend@ki.se
LI Xiaofei	Department of Medicine, Solna (MedS)	xiaofei.li@ki.se
LIM Sharon	Department of Microbiology, Tumor and Cell Biology (MTC)	sharon.lim@ki.se
LIMISZEWSKA Agnieszka	Department of Neuroscience (Neuro)	agnieszka.limiszewska@ki.se
LINDBERG Tülay	Department of Dental Medicine (Dentmed)	tulay.lindberg@ki.se
LINDGREN Gustaf	Department of Medicine, Solna (MedS)	gustaf.lindgren@ki.se
LITWIN Hannah	Department of Medical Biochemistry and Biophysics (MBB)	hannah.litwin@ki.se
LIU Jianping	Department of Biosciences and Nutrition (BioNut)	jianping.liu@ki.se
LIU xiao	Department of Medical Biochemistry and Biophysics (MBB)	li.liu@ki.se

LOÖHR Matthias	Department of Clinical Science, Intervention and Technology (CLINTEC)	matthias.lohr@ki.se
LUNDIN Natalie	Department of Medicine	natalie.lundin@karolinska.se
LUNDQVIST Johanna	Bioentrepreneurship Master	johanna.lundqvist@stud.ki.se
LÖVDAHL Cecilia	Innovation Office	cecilia.lovdahl@ki.se
MANOUILENKO Irina	Department of Clinical Neuroscience (CNS)	Irina.Manouilenko@ptj.se
MIJWEL Sara	Department of Physiology and Pharmacology (FyFa)	sara.mijwel@ki.se
MINTS Michael	Department of Oncology-Pathology (OnkPat)	michael.mints@ki.se
MOHAMMED Shafeeq	Department of Medicine	mshafeeqahmed88@gmail.com
MOUSAVI Malahat	Institute of Environmental Medicine	malahat.mousavi@ki.se
MOZAFFARI Fariba	Department of Oncology-Pathology (OnkPat)	fariba.mozaffari@ki.se
MUDRY Jonathan	Department of Molecular Medicine and Surgery (MMK)	jonathan.mudry@ki.se
NAWAZ Imran	Department of Microbiology, Tumor and Cell Biology (MTC)	imran.nawaz@ki.se
NI Ruiqing	Department of Neurobiology, Care Sciences and Society (NVS)	Ruiqing.ni@ki.se
NIGUS FIKRIE Telele	Department of Laboratory Medicine (LABMED)	nigus.fikrie.telele@ki.se
NIKKOLA Matti	Department of Cell and Molecular Biology (CMB)	matti.nikkola@ki.se
NILSSON Ann-Sofie	Department of Medical Biochemistry and Biophysics (MBB)	ann-sofie.nilsson@ki.se
NORBERG Jessica	Department of Clinical Science, Intervention and Technology (CLINTEC)	jessica.norberg@ki.se
NORLIN Emilia	Division of Obstetrics and Gynecology	emilia.norlin@stud.ki.se
NOUHI Mona	Department of Physiology and Pharmacology (FyFa)	mona.nouhi@ki.se

ÖKTEM Ozan	Department of Cell and Molecular Biology (CMB)	ozan.oktem@ki.se
OLAUSON Hannes	Department of Clinical Science, Intervention and Technology (CLINTEC)	hannes.olauson@ki.se
OLOFSSON Jessica	Department of Physiology and Pharmacology (FyFa)	jessica.olofsson@ki.se
OMELYANENKO Anna	Department of Physiology and Pharmacology (FyFa)	anna.omelyanenko@ki.se
PALUMBERI Domenico	Department of Neuroscience (Neuro)	domenico.palumberi@ki.se
PARIDA Shreemanta	Division of Therapeutic Immunology (TIM), Department of Laboratory Medicine (LABMED)	shreemanta.parida@ki.se
PARK Jung-sub	Department of Physiology and Pharmacology (FyFa)	jung-sub.park@ki.se
PARK Se Whee	Biomedicine Master	se.whee.park@stud.ki.se
PAULSSON-BERNE Gabrielle	Department of Medicine, Solna (MedS)	Gabrielle.Berne@ki.se
PETTERSSON Amanda	Department of Physiology and Pharmacology (FyFa)	amanda.pettersson@ki.se
PINHEIRO Tiago	Department of Cell and Molecular Biology (CMB)	tiago.pinheiro@hotmail.com
PINTOR Otto	Department of Laboratory Medicine (LABMED)	otto.pintor.gallego@stud.ki.se
PLATTEN Michael	Unit for Clinical Therapy Research, Inflammatory Diseases (ClinTRID)	michael.platten@stud.ki.se
POU Christian	Department of Cell and Molecular Biology (CMB)	christian.pou@ki.se
QUADIR Md Anwarul	Public Health - Health Economics	md.quadir@stud.ki.se
QUECKBORNER Suzanna	Department of Woman's and Children's Health (KBH)	suzanna.queckborner@ki.se
RABENHORST Uta	Department of Biosciences and Nutrition (BioNut)	uta.rabenhorst@ki.se

RAHMAN Mizanur	Institute of Environmental Medicine	mizanur.rahman@ki.se
RAUTIAINEN LAGERSTRÖM Susanne	Institute of Environmental Medicine	Susanne.rautiainen@ki.se
RENGBY Olle	Department of Medical Biochemistry and Biophysics (MBB)	olle.rengby@ki.se
ROOS Per	Nutritional Epidemiology	per.roos@ki.se
ROSSI Francesca	Department of Neuroscience (Neuro)	frances01.rossi@gmail.com
RUSSOM Aman	Science for Life Laboratory (SciLifeLab)	aman.russom@scilifelab.se
RYDEN AULIN Monica	Department of Medicine, Solna (MedS)	monica.ryden.aulin@ki.se
SACHWEH Marijke	Department of Microbiology, Tumor and Cell Biology (MTC)	marijke.sachweh@ki.se
SALASOVA Alena	Department of Medical Biochemistry and Biophysics (MBB)	alena.salasova@ki.se
SALMA Jahan	Department of Neuroscience (Neuro)	jahan.salma@ki.se
SANGFELT olle	Department of Cell and Molecular Biology (CMB)	olle.sangfelt@ki.se
SANTORO Federica	Department of Cell and Molecular Biology (CMB)	federica.santoro@ki.se
SCHALLING Martin	Department of Molecular Medicine and Surgery	martin.schalling@ki.se
SCHEYNIUS Annika	Department of Medicine, Solna (MedS)	annika.scheynius@ki.se
SCOBIE Martin	Diivision of Translational Medicine	Martin.Scobie@scilifelab.se
SELIVANOVA Galina	Department of Microbiology, Tumor and Cell Biology (MTC)	galina.selivanova@ki.se
SHAFQAT Jawed	Department of Medical Biochemistry and Biophysics (MBB), Lab. for Chemical Biology at Karolinska Institutet (LCBKI)	jawed.shafqat@ki.se
SHANG Ming-Mei	Department of Medicine	mingmei.shang@ki.se
SINTOU Nikolina	Department of Medical Biochemistry and Biophysics (MBB)	nikolinasintou@gmail.com

SOLSJÖ Peter	Department of Learning, Informatics, Management and Ethics (LIME)	peter.solsjo@ki.se
SONG Jie	Department of Medical Epidemiology and Biostatistics	jie.song@ki.se
SRAMKOVA Zuzana	Department of Physiology and Pharmacology (FyFa)	zuzana.sramkova@ki.se
STERNUDD Katarina	University Administration - Press Office	katarina.sternudd@ki.se
STRODTHOFF Daniela	Department of Medicine, Huddinge (MedH)	daniela.strodthoff@ki.se
SUN Jitong	Institute of Environmental Medicine	jitong.sun@ki.se
TAIPALE Minna	Department of Biosciences and Nutrition (BioNut)	minna.taipale@ki.se
TAMMIMIES Kristiina	Department of Woman's and Children's Health (KBH)	kristiina.tammimies@ki.se
TAYLAN Fulya	Department of Molecular Medicine and Surgery (MMK)	fulya.taylan@ki.se
TERENIUS Lars	Department of Clinical Neuroscience (CNS)	lars.terenius@ki.se
THEBO Lina	Department of Microbiology, Tumor and Cell Biology (MTC)	lina.thebo@ki.se
TOSKAS Konstantinos	Department of Neuroscience (Neuro)	konstantinos.toskas@ki.se
URBONAVICIUTE Vilma	Department of Medical Biochemistry and Biophysics (MBB)	vilma.urbonaviciute@ki.se
VALLIN Karl	Department of Medical Biochemistry and Biophysics (MBB)	karl.vallin@scilifelab.se
VALTER Kadri	Toxicology, Institute of Environmental Medicine	kadri.valter@ki.se
VAN VOLLENHOVEN Ronald	Department of Medicine, Solna (MedS)	ronald.van.vollenhoven@ki.se
VANGETI Sindhu	Department of Medicine	sindhu.vangeti@ki.se
VEMULA Manidee Gupta	Department of Neuroscience (Neuro)	manideep.vemula@ki.se

VIDAL Anna	Department of Neurobiology	annav07@hotmail.com
VILLABLANCA Eduardo	Department of Medicine, Solna (MedS)	eduardo.villablanca@ki.se
VIRDING Susanne	Department of Medical Biochemistry and Biophysics (MBB)	susanne.virding@ki.se
VON WALDEN Ferdinand	Department of Woman's and Children's Health (KBH)	Ferdinand.vonwalden@ki.se
VOS Joelle	Unit for Bioentrepreneurship, Department of Learning, Informatics, Management and Ethics (LIME)	joelle.vos@ki.se
VOSOUGH Massoud	Department of Laboratory Medicine (LABMED)	massoud.vosough@ki.se
WAHLUND Casper	Translational Immunology Unit	casper.wahlund@ki.se
WANG Xinming	Department of Neuroscience (Neuro)	xinming.wang@ki.se
WEI Bei	Department of Biosciences and Nutrition (BioNut)	bei.wei@ki.se
WIDEGREN Ulrika	Department of Molecular Medicine and Surgery (MMK)	ulrika.widegren@ki.se
WITMAN Nevin	Department of Cell and Molecular Biology (CMB)	Nevin.Witman@ki.se
WOLK Alicja	Institute of Environmental Medicine	Alicja.Wolk@ki.se
WORKU KALU Amare	Department of Laboratory Medicine (LABMED)	amare.worku.kalu@ki.se
XU Dawei	Department of Medicine	Dawei.Xu@ki.se
XU Shaohua	Department of Neuroscience (Neuro)	shaohua.xu@ki.se
YAN Qinzi	Department of Microbiology, Tumor and Cell Biology (MTC)	qinzi.yan@ki.se
YANG Jiangning	Department of Medicine, Solna (MedS)	jiangning.yang@ki.se
YANG Shanzheng	Department of Medical Biochemistry and Biophysics (MBB)	shanzheng.yang@ki.se

YANG Ting	Department of Physiology and Pharmacology (FyFa)	ting.yang@ki.se
YAO Ning	Department of Physiology and Pharmacology (FyFa)	ning.yao@ki.se
YEUNG Maggie	Department of Cell and Molecular Biology (CMB)	maggie.yeung@ki.se
YILMAZ Marlene	Department of Cell and Molecular Biology (CMB)	marleneyilmaz@hotmail.com
ZAOUTER Cecile	Department of Cell and Molecular Biology (CMB)	cecile.zaouter@licr.ki.se
ZARAVINOS Apostolos	Department of Laboratory Medicine (LABMED)	apostolos.zaravinos@ki.se
ZHANG Yin	Department of Microbiology, Tumor and Cell Biology (MTC)	yin.zhang@ki.se
ZHENG Xiaowei	Department of Molecular Medicine and Surgery (MMK)	xiaowei.zheng@ki.se
ZHOU yan	Department of Oncology-Pathology (OnkPat)	Yan.zhou@ki.se

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