CONFIDENTIAL

2017 CANADA GAIRDNER AWARDS RECOGNIZE BASIC AND TRANSFORMATIVE RESEARCH IN CANADA AND INTERNATIONALLY

EMBARGOED UNTIL MARCH 28, 2017 at 8:00 a.m. EST

TORONTO, ON (March 28, 2017) - The Gairdner Foundation is pleased to announce the 2017 Canada Gairdner Award laureates, recognizing some of the most significant medical discoveries from around the world. The Awards carry an honorarium of $100,000 for each of the seven awards and will be presented at a Gala in Toronto on October 26, 2017.

The selections for the **2017 Canada Gairdner International Awards**, recognizing five individuals from various fields for seminal discoveries or contributions to biomedical science, are below.

Dr. Akira Endo

President, Biopharm Research Laboratories; Distinguished Professor Emeritus, Tokyo University of Agriculture and Technology, Tokyo, Japan

Awarded "For the first discovery and development of statins, inhibitors of cholesterol biosynthesis that have transformed the prevention and treatment of cardiovascular disease."

The Work:
Dr. Endo discovered the first statin drug, compactin, and demonstrated its clinical efficacy. Statins are a class of drugs with remarkable cholesterol-lowering properties that have revolutionized the prevention and treatment of coronary heart disease (CHD). They lower the part of cholesterol known as "bad cholesterol", technically known as low density lipoprotein or LDL cholesterol. Dr. Endo sifted through thousands of organisms, hunting for natural substances(products) that block a key enzyme in the biochemical pathway that produces cholesterol, a major contributor to CHD. The organism he found does exactly that and his work stimulated Merck to launch a drug-development program that led, 20+ years ago, to the first statin approved for medical use. This advance paved a path for other pharmaceutical companies to follow.

The Impact:
Statins are now routinely used to prevent and treat CHD throughout the world. Although CHD is aggravated by multiple risk factors, reducing LDL levels alone makes a significant impact. By discovering statins, Dr. Endo ushered in a new era in preventing and treating CHD and it is estimated that millions of people have extended their lives through statin therapy.

Dr. David Julius

Professor and Chair of the Department of Physiology and the Morris Herzstein Chair in Molecular Biology and Medicine, UCSF, San Francisco, California, USA

Awarded "for determining the molecular basis of somatosensation- how we sense heat, cold and pain"

The Work:
Dr. Julius has used distinctive molecules from the natural world – including toxins from tarantulas and coral snakes, and capsaicin, the molecule that produces the "heat" in chili peppers – to understand how signals responsible for temperature and pain sensation are transmitted by neural circuits to the brain.

In his research Julius has homed in on a class of proteins called TRP (pronounced "trip") ion channels to discover how the chemical compound responsible for the spicy heat of chili peppers – called capsaicin – elicits a burning sensation when eaten or touched. The research led to the

identification and cloning of the specific protein responsible, named TRPV1. On the flip side, Julius has used menthol, a natural cooling agent, to identify a receptor for “real” cold. This protein, named TRPM8, is a close molecular cousin of TRPV1, pointing to a common mechanism for sensing temperature. As in the case of TRPV1, this ion channel contributes to hypersensitivity to cold, such as that experienced after chemotherapy or other types of nerve injury.

The Impact:
Somatosensation, our sense of touch and pain, serves as a warning system to guard us against injury. While critical to our survival and well-being, this system can become hypersensitive, resulting in chronic pain. This work helps to explain how such positive and negative aspects of pain sensation arise – insight that is critical to understanding the genesis of chronic pain syndromes. One indication of the importance of this work to medicine is the interest in TRP channels as potential targets for a new generation of painkillers.

Dr. Lewis E. Kay

Professor, Departments of Molecular Genetics, Biochemistry and Chemistry, University of Toronto; and Senior Scientist, The Hospital for Sick Children, Toronto, Ontario, Canada

Awarded “For the development of modern NMR spectroscopy for studies of biomolecular structure dynamics and function, including applications to molecular machines and rare protein conformations”

The Work:
Professor Kay and his coworkers have made important contributions to the field of biomolecular nuclear magnetic resonance (NMR) spectroscopy, with the development of methods that are used to ‘visualize’ protein molecules in their natural solution environment and to obtain information about how their shapes evolve in time, leading to biological function. These methods have shed light on how molecules involved in neurodegeneration can form abnormal structures that ultimately lead to diseased states. In addition, his work has extended our understanding of how cellular machines function and how the communication between different parts of these machines can be targeted for the development of drugs in the fight against certain cancers.

The Impact:
His research has expanded our understanding of the flexible nature of protein structure and the importance of flexibility to both function and malfunction. This, in turn, has led to new insights into what the key regions of molecules might be for drug targeting. The methods developed by Dr. Kay are used in labs around the world, including those researching illnesses such as diabetes, cancer and cardiovascular disease. The tools developed by his research group are disseminated freely and are extensively used worldwide.

Dr. Rino Rappuoli

Chief Scientist and Head External R&D at GSK Vaccines, Siena, Italy

Awarded “For pioneering the genomic approach, known as reverse vaccinology, used to develop a vaccine against meningococcus B which has saved many lives worldwide”

The work:
Dr. Rappuoli is a pioneer in the world of vaccines and has introduced several novel scientific concepts. First, he introduced the concept that bacterial toxins can be detoxified by manipulation of their genes (genetic detoxification, 1987). Next, the concept that microbes are better studied in the context of the cells they interact with (cellular microbiology, 1996), and then the use of genomes to develop new vaccines (reverse vaccinology, 2000). In the process of reverse vaccinology the entire genomic sequence of a pathogen is screened using bioinformatics tools to help determine which genes code for which proteins, against which vaccines can be developed.

The impact:
Dr. Rappuoli also worked on several molecules which became part of licensed vaccines. He characterized a molecule, CRM197, that today is the most widely used carrier for vaccines against Haemophilus influenzae, meningococcus and pneumococcus. Later he developed a vaccine against pertussis containing genetically detoxified pertussis toxin and the first conjugate vaccine against meningococcus C that eliminated the disease in the United Kingdom in 2000. His work on reverse vaccinology led to the licensure of the first meningococcus B vaccine approved in Europe and Canada in 2013 and USA in 2015.

Dr. Huda Y. Zoghbi

Professor Baylor College of Medicine, Investigator Howard Hughes Medical Institute, and Director of the Jan and Dan Duncan Neurological Research Institute at Texas Children's Hospital, Houston, Texas, USA

Awarded "For the discovery of the genetic basis of Rett syndrome and its implications for autism spectrum disorders"

The Work:
Trained as a child neurologist, Zoghbi could not bear the plight of children affected by devastating neurological diseases so she pursued research in hope of helping her patients. After encounters with patients with Rett syndrome—a disorder that strikes after a year of normal development and presents with developmental regression, social withdrawal, loss of hand use and compulsive wringing of the hands, seizures and a variety of neurobehavioral symptoms—she decided to find its genetic roots. The biggest challenge was that Rett syndrome is typically a sporadic disorder (one in a family) and the genome was neither mapped nor sequenced. Zoghbi's perseverance paid off when after a 16-year search she discovered that Rett syndrome is caused by mutations in *MECP2*. Zoghbi revealed the importance of MeCP2 for the function of various neuronal subtypes and pinpointed the contributions of various neuronal subtypes in the brain to various neuropsychiatric features. Zoghbi also provided evidence that the brain is exquisitely sensitive to the levels of MeCP2 and that doubling MeCP2 levels causes progressive neurological deficits in mice. This disorder is now recognized as *MECP2* Duplication Syndrome in humans. Her recent work showed the symptoms of adult mice modeling the duplication disorder can be reversed using antisense oligonucleotides that normalize MeCP2 levels.

The Impact:
The discovery of the Rett syndrome gene provided a straightforward diagnostic genetic test allowing early and accurate diagnosis of the syndrome. It also revealed that mutations in *MECP2* can also cause a host of other neuropsychiatric features ranging from autism to juvenile onset schizophrenia. Further, it provided evidence that an autism spectrum disorder (ASD) or an intellectual disability disorder (IDDs) can be genetic even if it is sporadic (not inherited). Today we know that dozens of ASDs and IDDs are caused by sporadic new mutations. Moreover, her discovery opened up a new area of research on the role of epigenetics in neuropsychiatric phenotypes. Her use of an antisense oligonucleotide to lower MeCP2 levels provides a potential therapeutic strategy for the *MECP2* duplication syndrome and inspires similar studies for other duplication disorders.

The 2017 **John Dirks Canada Gairdner Global Health Award** is awarded for a scientific advancement that has made a significant impact on health in the developing world. This year's award is given to Dr. Cesar Victora.

Dr. Cesar Victora

Emeritus Professor, Federal University of Pelotas, Pelotas, Brazil

Awarded "For outstanding contributions to maternal and child health and nutrition in low and *middle income countries, with particular focus on the impact of exclusive breastfeeding on infant mortality and on the long-term impact of early-life nutrition."*

The work:
Dr. Victora's career has focused on the factors affecting maternal and child health in low- and middle income countries. He has concentrated in the three main areas of child health and nutrition, health program monitoring and evaluation, and health equity. Returning to Brazil after his doctorate, he helped set up one of the longest running birth cohort studies in the world, the 1982 Pelotas Birth Cohort, in which 6,000 individuals are being followed up with to the present time. His studies helped establish the influence of the first 1,000 days (from conception until the age of two years) on lifelong outcomes, including chronic diseases and human capital.

The Impact:
Possibly, Dr. Victora's greatest contribution to Public Health was his work in the 1980s with the first study showing the importance of exclusive breastfeeding for preventing infant mortality. His findings contributed to global policy recommendations by UNICEF and the World Health Organization for mothers to breastfeed their infants exclusively for the first six months of life. More recently, his long-term birth cohorts documented the benefits of breastfeeding for adult intelligence, education and income, as well as the long-term consequences of early-life undernutrition. Victora also made important contributions on how to evaluate the impact of health programs on child mortality and on the study of social inequalities in child health.

The **2017 Canada Gairdner Wightman Award**, given to a Canadian who has demonstrated outstanding leadership in medicine and medical science throughout his/her career, is awarded to:

Dr. Antoine M. Hakim

Emeritus Professor, Neurology, University of Ottawa, Ottawa, Ontario, Canada

Awarded "For outstanding research into stroke and its consequences and championing stroke prevention and treatment in Canada and beyond"

The Work:
Dr. Hakim is one of Canada's most distinguished scientists who has earned a world-renowned reputation for his leadership in neuroscience research with an emphasis on stroke research. In the early 1980's Dr. Hakim characterized a penumbral region around a stroke's ischemic core — brain tissue with enough energy to survive for a short time after blood loss and with the potential to regain normal function if blood flow was restored. Dr. Hakim, who joined the University of Ottawa in 1992, led the charge to set up the Canadian Stroke Network, a Network of Centres of Excellence; he then partnered with the Heart and Stroke Foundation and other organizations to develop and apply a nation-wide Canadian Stroke Strategy. This work was critical to changing attitudes towards stroke which went from being a devastating condition to one that is preventable, treatable and repairable.

The Impact:
In 2006, Dr. Hakim and colleagues published the first 'Canadian Best Practice Recommendation for Stroke Care' (updated in 2008, 2010 and 2012) and developed performance indicators and toolkits for healthcare providers to set up stroke units and improve emergency medical services. They also instituted a multi-layered national education program to enhance stroke prevention and the delivery of acute stroke care through coordination of services and implementation of best practice. Within five years of the Strategy's implementation, Ontario alone saw referrals to stroke prevention clinics increase by 34% and stroke patient admissions decrease by 11%.

Nominations for the 2018 Canada Gairdner Awards are open and the deadline is October 1, 2017. Visit Gairdner.org for more details.

About the Canada Gairdner Awards:

Each year seven Canada Gairdner Awards are presented to honour the world's most significant biomedical and global health researchers. The Canada Gairdner International Award is given to five individuals for outstanding international biomedical research, while the Canada Gairdner Wightman Award is given to an individual leader in Canadian medicine. The John Dirks Canada Gairdner Global Health Award recognizes a contribution to health in the developing world. These seven awards both distinguish Canada as a leader in science and elevate the profile of science in Canada They are Canada's only globally known and respected international science awards. All laureates are chosen by an international adjudication committee and all choices are deemed final.

About the Gairdner Foundation:

The Gairdner Foundation was established through the pioneering gift of James Gairdner in 1957, a man who had always been active in the community and embodied a sense of social consciousness. He was inspired to make a difference and wanted to celebrate excellence in science internationally. Sixty years later and with more than 360 awardees from 30 countries recognized for their innovative work, the Gairdner Foundation has lived up to the original expectations of its founder. Of the Gairdner laureates, 84 have gone on to receive the Nobel Prize in Physiology or Medicine. The Canada Gairdner Awards promote a stronger culture of research and innovation across the country through our Outreach Programs including lectures and research symposia. The programs brings current and past laureates to a minimum of 15 universities across Canada to speak with faculty, trainees and high school students to inspire the next generation of researchers. Annual research symposia are organized across Canada to provide Canadians access to leading science through Gairdner's convening power.

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