PRESS RELEASE
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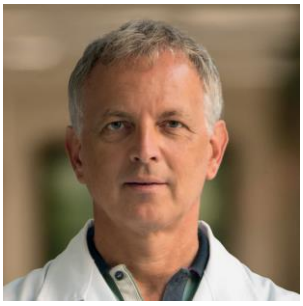
2019 LOUIS-JEANTET PRIZES

The 2019 Louis-Jeantet Prizes are awarded to LUIGI NALDINI, Director of the San Raffaele Telethon Institute for Gene Therapy and Professor at the San Raffaele University in Milan, Italy and to BOTOND ROSKA, a founding director of the Institute of Molecular and Clinical Ophthalmology Basel (IOB) in Switzerland.

The LOUIS-JEANTET FOUNDATION grants the sum of CHF 500,000 for each prize, of which CHF 450,000 is for the continuation of the prize-winner's research and CHF 50,000 is for their personal use.

The prize-winners are conducting fundamental and translational research that is of considerable significance for medicine.



LUIGI NALDINI, of Italian nationality, is awarded the 2019 Jeantet-Collen Prize for Translational Medicine for his pioneering work taking gene therapy from the bench to the bedside, allowing to treat several genetic diseases.

Gene therapy can treat inherited diseases by replacing a malfunctioning gene with a functional copy of it in the affected cells, or by editing the mutant genomic sequence to the common version. Luigi Naldini has been a pioneer in the development of safe lentiviral vectors for gene therapy, having achieved success in the treatment of patients suffering from several rare fatal disorders.

Luigi Naldini will use the prize money to pave the way to the next generation of safer and more effective hematopoietic stem cell therapies based on precision genetic engineering by targeted gene editing.

BOTOND ROSKA, of Hungarian nationality, is awarded the 2019 Louis-Jeantet Prize for Medicine for the discovery of basic principles of visual information processing and the development of therapeutic strategies, such as gene therapy, to restore vision in retinal disorders.

Vision is a key sense for humans and dysfunction of the visual system can lead to blindness. Botond Roska and his team aim to find ways to repair visual dysfunction by investigating how the retina, thalamus, and cortex function at the level of cell types and circuits. They then use the acquired knowledge to understand disease mechanisms and to develop treatments.

Botond Roska will use the prize money to develop methods to further understand the functional architecture of the human retina in both normal and disease states.

THE AWARD CEREMONY WILL BE HELD IN GENEVA (SWITZERLAND) ON **WEDNESDAY, 10 APRIL 2019**.

LUIGI NALDINI

Luigi Naldini received his M.D. from the University of Turin, Italy and his Ph.D. from the University of Rome. In the mid 90's he moved to the Salk Institute in San Diego, USA, to carry out his research work in the laboratories of Inder Verma and Didier Trono, where they pioneered the development of lentiviral gene transfer vectors from HIV. He then spent 2 years in the biotechnology industry in San Francisco, before moving back to academia in Turin. Since 2008 he has been the Director of the San Raffaele Telethon Institute for Gene Therapy of Milan (SR-Tiget) and Professor at the "Vita Salute" San Raffaele University in Milan, Italy.

Luigi Naldini was elected as a member of the European Molecular Biology Organization (EMBO). He has been President of the European Society of Gene and Cell Therapy (ESGCT) and was appointed as an expert on the *Human Gene Editing Study* of the US National Academies of Sciences and of Medicine. He was awarded the Outstanding Achievement Award from the American Society of Gene and Cell Therapy (ASGCT) in 2014 and from the ESGCT in 2015, the Jimenez Diaz Prize in 2016, and the Beutler Prize from the American Society of Hematology in 2017.

Gene therapy, from bench to bedside

Gene therapy is becoming established as a new pillar of precision medicine. This comeback following some early setbacks is broadly credited to the development of new gene transfer vectors which are proving safer and more effective than earlier technologies in clinical trials. Luigi Naldini, in the laboratory of Inder Verma and Didier Trono, took advantage of the infectious ability of HIV to obtain a highly efficient vector for gene transfer into human cells. Naldini then went on to further engineer vector design and provide a safer gene transfer platform.

The demonstration of effective disease correction in animal models, coupled with the improved safety of the vector, moved lentiviral vectors to clinical testing, some of which were first performed in humans by Naldini and his team at SR-Tiget. Nearly one hundred patients suffering from rare fatal disorders such as X-linked adrenoleukodystrophy, metachromatic leukodystrophy, Wiskott Aldrich syndrome and other severe combined immunodeficiencies, have been treated today with hematopoietic stem cells engineered with lentiviral vectors to replace the inherited defective gene. The vast majority of them were reported to be in good condition after 10 years and leading a normal or near-normal life, whereas they would probably have already succumbed to their disease if left untreated. More recently, this strategy has been applied to the treatment of beta-thalassemia. Meanwhile, Luigi Naldini's experimental research has continued to bring about innovative solutions to further enhance the efficacy and safety of gene therapy, such as targeted gene editing. These studies open the way to correct, rather than replace, genes, a potentially revolutionary approach that may substantially expand the scope and power of genetic manipulation.

BOTOND ROSKA

Botond Roska was born in 1969 in Hungary. He obtained his M.D. at the Semmelweis Medical School, a Ph.D. in neurobiology from the University of California, Berkeley and studied genetics and virology as a Harvard Society Fellow at Harvard University and the Harvard Medical School. He has been leading a research group at the Friedrich Miescher Institute in Basel since 2005. In 2010 he became Professor at the Medical Faculty of the University of Basel and he is now a founding director of the Institute of Molecular and Clinical Ophthalmology Basel (IOB).

Botond Roska was elected as a member of the European Molecular Biology Organization (EMBO) in 2011. He has received numerous awards, including the Viva Award in 2010, the Alcon Award in 2011, the Alfred Vogt Award in 2013, the Cogan Award in 2016, the Bressler Prize and the Alden W. Spencer Award in 2018.

Vision and vision restoration

Dysfunction of the visual system, leading to visual handicap or blindness, is critical in humans. Blindness has a drastic effect on day-to-day life but, unfortunately, sight-restoring therapy for the visually impaired and blind is still a major unmet medical need. To find ways to repair visual dysfunction, Botond Roska and his co-workers investigate the retina, thalamus and cortex at the level of cell types and circuits. The knowledge they are acquiring has already improved our understanding of disease mechanisms and opened up paths to potential treatments.

Botond Roska's research group has illustrated how cell types in the visual system interact in local and long-range circuits and extract features from the visual scene. Combining this knowledge with human genetics and molecular techniques, they have provided insights into the mechanisms of cell type-specific genetic diseases. Bringing their knowledge of visual circuits together with technologies such as optogenetics, they have designed novel therapies for restoring vision in genetic forms of blindness. The essence of these optogenetic therapies is to deliver genetically encoded light sensors to strategically important retinal cell types in the blind retina. Once there, non-photoreceptor cells are converted into photosensors or the photosensitivity of native photoreceptors compromised by disease is restored. These artificial photoreceptors then drive vision. Botond Roska's work illustrates how insights into the organization of the cell types and circuits of the nervous system, when combined with cellular engineering, could be used to design new therapies to fight blindness.

THE LOUIS-JEANTET PRIZES

Every year, the Louis-Jeantet Prizes distinguish leading-edge researchers who are active in the member states of the Council of Europe.

As one of the best-endowed awards in Europe, the Louis-Jeantet Prizes foster scientific excellence. They are not intended solely as the recognition of work that has been completed, but also to encourage the continuation of innovative research projects. When the research being recognised is close to practical applications for combating illnesses affecting humankind, one of the Louis-Jeantet Prizes converts into a Jeantet-Collen Prize for Translational Medicine, supported by generous donations from the Désiré Collen Stichting.

Established in 1986, the Louis-Jeantet Prizes have thus far been awarded to 90 researchers: 27 in the United Kingdom; 17 in Switzerland; 15 in Germany; 14 in France; 4 in Sweden; 3 in the Netherlands; 2 each in Austria, Belgium, Finland, Norway and Italy. Among the 90 prize-winning researchers, 12 subsequently won the Nobel Prize for physiology or medicine, or the Nobel Prize for chemistry.

Since 1986, a total sum of more than CHF 60m has been awarded by the Foundation to the 90 prize-winners for the continuation of their work.

THE LOUIS-JEANTET FOUNDATION

Founded in 1983, the Louis-Jeantet Foundation is the legacy of Louis Jeantet, a French businessman and a citizen of Geneva by adoption. Its aim is to move medicine forward and to defend the role and identity of European biomedical research vs. international competition. Established in Geneva, the Foundation is part of an open Europe and devotes its efforts to recognizing and fostering medical progress for the common good.

The Louis-Jeantet Foundation allocates some CHF 3m each year to promoting biomedical research. It invests this sum for European and for local research projects. At the local level, the Foundation encourages teaching and the development of research at the Faculty of Medicine of the University of Geneva.

Since 2010, EMBO and the Louis-Jeantet Foundation jointly promote the leading-edge research work of the winners of the Louis-Jeantet Prizes. In this context, the journal *EMBO Molecular Medicine* features special contributions by the prize-winners and hosts the Louis-Jeantet prize-winners' lectures given during the annual EMBO Congress.

For more information, please contact:

Gisou VAN DER GOOT
Secretary of the Scientific Committee of the Louis-Jeantet Foundation
Tel: + 41 (0)21 693 14 82
E-mail : vandergoot@jeantet.ch
Website: www.jeantet.ch

Luigi NALDINI
Tel: +39 02 2643 4681
E-mail : naldini.luigi@hsr.it
Website: http://www.sr-tiget.it/

Botond ROSKA
Tel: +41 (0)79 852 20 60
E-mail : botond.roska@iob.ch
Website: www.iob.ch

www.jeantet.ch

https://twitter.com/LouisJeantetFDN