

Else Kröner Fresenius Prize for Medical Research 2020

Prof. Dr. Alessandro Aiuti, a physician and research scientist based in Milan is the winner of the Else Kröner Fresenius Prize for Medical Research 2020, which is awarded by the EKFS in recognition of Aiuti's significant scientific contributions to the development of gene therapies.



Prize for Medical Research 2020: Alessandro Aiuti

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Prof . Dr. Michael Madeja,
scientific director and member of the management board at EKFS

“Still young by comparison, this year the prize is being awarded for the third time. It honors research scientists for pioneering contributions in the areas of medical science. A major percentage of the prize money flows into the prizewinner’s research and is supposed to contribute to further groundbreaking findings and medical breakthroughs in the future as well.”

Newly developed gene therapies against hereditary diseases

With his work, Alessandro Aiuti has made a major contribution to ensuring that patients with rare, hereditary immune defects and metabolic diseases can be successfully treated with new gene therapies.

With the gene therapy from Milan there is an alternative for these children. After successful clinical trials, the gene therapy developed for ADA-SCID patients was approved as a pharmaceutical remedy in Europe.

The therapy is based on the patient's own living cells whose DNA is modified. For the treatment, certain blood stem cells (CD34+) are collected from the patient. Outside the body, these cells are treated using a viral vector to introduce the correct version of the gene for the ADA enzyme. The genetically modified cells are returned to the patient's bloodstream via intravenous infusion. Portions of the modified cells subsequently establish themselves in bone marrow again. The patient now has blood stem cells that function properly and produce lymphocytes to defend against infections – presumably on a life-long basis.

36

children from 19 countries

were treated with the gene therapy.



The correct version of the gene for the ADA enzyme is introduced to blood stem cells
(© Telethon Foundation)

Alessandro Aiuti,

“In more than 80 percent of the cases, the treatment has had such an impact that no enzyme replacement therapy or transplantation is needed.”

The decision regarding the prize recipient was made by a ten-member international jury composed of renowned research scientists in the fields of genome editing and gene therapy along with delegates from the Scientific Commission at EKFS. Hildegard Büning, chairwoman of the jury and president of the European Society for Gene and Cell Therapy (ESGCT), substantiates the jury’s decision.



00:00



00:32

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Prize winner 2020: Alessandro Aiuti who works at the San Raffaele-Telethon Institute for Gene Therapy (SR- the Vita Salute San Raffaele University

Together with his team, he pushed forward the development of successful gene therapy approaches for diseases such as Wiskott-Aldrich Syndrome or Metachromatic Leukodystrophy. He achieved a widely recognized success as a key figure in the development of a gene therapy for ADA-SCID (Adenosine Deaminase Severe Combined Immuno Deficiency). This gene therapy and the achievement of its market approval in Europe is considered to be one of the most important results in the development of gene therapies worldwide. "An international leader who will continue to write gene therapy history," the jury said in its statement.

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(© EKFS)

Alessandro Aiuti,

San Raffaele Telethon Institute for Gene Therapy, Vita Salute San Raffaele University

“Without effective therapy, the children rarely survive for more than 2 years because any infection can become perilous for them.”

Successful treatment of ADA-SCID

In the case of the rare immune disorder ADA-SCID, which exclusively afflicts young children and occurs about 15 times a year in Europe, a defective ADA gene within the genome disrupts lymphocyte development, leaving the young patient's body defenseless against infections. Standard for this therapy is a bone marrow transplantation from a fully matched sibling.

However, a suitable donor is available only for a minority of patients.

Alessandro Aiuti wants to use the prize money from EKFS to optimize the therapies further and map out the healing mechanisms involved in a better fashion. The scientist sees another major challenge in conveying the acquired knowledge beyond the successful gene therapies from Milan to as many other genetic disorders as possible.

Alessandro Aiuti



Alessandro Aiuti was born in Rome in 1966 and studied medicine there at Sapienza University. Following a stay at Harvard Medical School in Boston, Massachusetts in the USA, he received his doctorate in Human Biology in 1996 from Sapienza University. Since 1997 he has been active at the San Raffaele Scientific Institute in Milan, where he meanwhile also teaches as a professor at the Vita Salute San Raffaele University. He is furthermore Deputy Director of Clinical Research at the San Raffaele Telethon Institute for Gene Therapy and Head of the Pediatric Immunohematology Unit at San Raffaele Hospital.

Aiuti is the author of numerous and highly acclaimed publications. Over the course of his career he has received a number of prizes from national and international institutions. Aiuti is a member of the board of the European Society of Gene and Cell Therapy, and a member of the EMA Committee for Advanced Therapies since 2019.

Aiuti is married to Laura since 1993 and has two daughters, Francesca and Camilla. He likes to travel and go to the cinema, and he enjoys sports, especially sailing, skiing and running (often with his dog).

Jury



An international jury comprised of ten renowned researchers in the fields of genome editing and gene therapy together with representatives from the Scientific Commission at EKFS decided on who shall receive the prize.

- Hildegard Büning, Hannover Medical School (Germany), jury chairwoman
- Michele Calos, Stanford University (USA)
- Nathalie Cartier, French Institute of Health and Medical Research, Paris (France)
- Stefan Endres, Ludwig-Maximilians-University Munich (Germany)
- Guangping Gao, University of Massachusetts (USA)
- Christine Klein, University of Lübeck (Germany)
- Luigi Naldini, San Raffaele Telethon Institute for Gene Therapy, Milan (Italy)
- Amit Nathwani, University College London (United Kingdom)

- Virginijus Šikšnys, Vilnius University (Lithuania) Adrian Thrasher, University College London (United Kingdom)

Postponed: Prize Presentation on May 31, 2021



Due to the corona pandemic, the festive ceremony for the Else Kröner Fresenius Award for Medical Research 2020 has been postponed to May 31, 2021.

Award Presentation tentatively on

Monday, the 31st of May, 2021, 6 p.m. to 7:45 p.m., followed by a reception in Frankfurt am Main (the exact location shall be announced on a timely basis)

Within the framework of the festivities the foundation is also hosting a scientific symposium on the topic of "Genome Editing and Gene Therapy" on the same day (see below).

Event not open to the public – by invitation only. You can find more information [→ here](#).

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