VareseNews

San Raffaele, a $1.5 million award to study rare genetic diseases

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Funds of $1.5 million over 5 years: Raffaella Di Micco, a researcher at the San Raffaele-Telethon Institute for gene therapy (SR-Tiget), in Milan, has received a prestigious acknowledgement, the **Robertson Stem Cell Investigator Award, from the New York Stem Cell Foundation**, which is given to scientists who are considered innovators capable of exploring and opening new paths in the field of scientific stem cells research. Her functional studies may suggest new therapeutic approaches to gene and cell therapy for the treatment of hereditary blood diseases. This is the first time that this award has come to Italy.

At SR-Tiget, Raffaella Di Micco leads a research team who study the molecular mechanisms that govern the response of human haematopoietic stem cells to different genetic engineering strategies, such as gene therapy and gene editing. “I’m particularly proud of this award, because it’s the first time ever that it’s been given to a researcher who works in Italy,” the Raffaella said, on the spur of the moment. “Thanks to this award, our team will be able to investigate how to make even more efficient and effective the innovative therapeutic strategies based on engineering blood stem cells, which, over the last few years, have achieved extraordinary results in the treatment of rare genetic diseases. In our project, we’re also going to use the CRISPR technique, a system of genetic scissors developed by Emmanuelle Charpentier and Jennifer Doudna, who, for this, received the 2020 Nobel Prize for Chemistry, which allows intervening very precisely on DNA, and which has opened important prospects also for rare genetic diseases.” This award to Raffaella Di Micco comes just a few days after the Nobel announcements that have proudly rewarded the contribution of women researchers to scientific progress.

In the meantime, Raffaella di Micco, who was **the face of the Telethon campaign on the awareness of rare genetic diseases, “Io per Lei”**, on Mothers’ Day 2019, has become the mother of Tommaso, who was born last August. An authentic Neapolitan, Raffaella is a citizen of the world, by “will” of scientific research; today, after a post-doctoral experience of 5 years at New York University, her home is SR-Tiget in Milan. Indeed, she is what people call a “returning brain”. She chose Italy, non only because it is her homeland, but because, “compared to many other institutes around Europe, SR-Tiget gave me the opportunity to combine my interest in basic research with the clinical application of the results of the research paths for which it is a world leader.”

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