

## Genenta and Ospedale San Raffaele Announce a Strategic Collaboration with Amgen to Explore Hematopoietic Stem Cell Gene Therapy in Oncology

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**Genenta Science and San Raffaele Hospital and Scientific Institute** (Ospedale San Raffaele, OSR) today announced a research collaboration agreement with Amgen to evaluate the efficacy of novel gene therapy strategies for tumor treatment.

The collaborative agreement brings Genenta, OSR and Amgen together in a research partnership that aims to evaluate and further develop an innovative therapy based on gene transfer into autologous hematopoietic Stem Cells (HSC) to express specific anti-tumor proteins in their progeny. The platform is based on a combination of transcriptional and microRNA-mediated control to preferentially direct anti-tumor proteins, such as immune activators, to tumor-infiltrating macrophages, thus increasing local efficacy while reducing the systemic toxicity, originally developed at OSR in the laboratory of angiogenesis and tumor targeting by the team of Luigi Naldini and exclusively licensed to Genenta.

The scientific research at OSR, Milano (Italy), will be led by Luigi Naldini, M.D., Ph.D., Director of the Division of Regenerative Medicine, Stem Cells and Gene Therapy of the San Raffaele Scientific Institute and of the San Raffaele Telethon Institute of Gene Therapy (SR- Tiget), and a world-renowned expert in lentiviral vector based gene therapy.

Luigi Naldini comments: "The highly encouraging results of clinical trials of HSC gene therapy performed at SR-Tiget for some genetic diseases have opened the way to explore new ways to genetically modify HSC and, rather than replacing malfunctioning genes, instruct these cells and their progeny to better fight cancer; our collaborating with Amgen will bring crucial experience, skills and resources to stringently evaluate the potential of our new strategy in oncology"

"Amgen is the perfect partner for us due to their leadership in oncology and commitment in exploring innovative technologies," stated **Pierluigi Paracchi**, Genenta's Chairman and Chief Executive Officer. "We are proud to announce this collaboration to exploit our HSCs gene therapy platform".

The goal of the collaboration is to gain mechanistic insight into myeloid-targeted HSCs gene therapy approaches for therapeutic use in oncology.

## **About Genenta Science**

Genenta Science – gene therapy for tumor treatment, <a href="www.genenta.com">www.genenta.com</a> – develops a gene transfer strategy into autologous hematopoietic stem cells (HSCs) to target interferon-α expression to tumor-infiltrating monocytes/macrophages. An HIV-derived and genetically disabled viral vector – Lentivirus – delivers the gene into the HSCs. Founders: Pierluigi Paracchi, Ospedale San Raffaele, Luigi Naldini and Bernhard Gentner (Hematologist and Physician Scientist at OSR and SR-TIGET). In March 2015, Genenta Science banked a Euro 10 million (USD 11 million) Series A round.

## About Ospedale San Raffaele

Ospedale San Raffaele (OSR) is a clinical-research-university hospital established in 1971 to provide international-level specialized care for the most complex and difficult health conditions. Since 2012 it is part of Gruppo Ospedaliero San Donato, the leading hospital group in Italy. The hospital is a multi-specialty center with over 50 clinical specialties and has over 1,300 beds. Research at OSR focuses on integrating basic, translational and clinical activities to provide the most advanced care to patients. The hospital counts on over 1,800 medical doctors, scientists and technicians and on state-of-the-art facilities and technology platforms. The institute is recognized as a global authority in molecular medicine and gene therapy, and is at the forefront of research in many other fields.

Ospedale San Raffaele stands out for the deep interaction between clinical and scientific area. This makes the transfer of scientific results from the laboratories to the patient's bed easier.

Its mission is to improve knowledge of diseases, identify new therapies and encourage young scientists and doctors to grow professionally. For further information, visit: <a href="https://www.hsr.it">www.hsr.it</a>