

# Genetic therapies and the main trends in haematology: Dr. Marina Cavazzana

27 June 2017



Recent scientific breakthroughs hold promises for the development of innovative therapies says Dr. Marina Cavazzana, Head of the Department of Biotherapy at the Necker Hospital, Paris.

**Dr. Cavazzana, what are the main trends in haematology?**

One of the main trends is the development of therapies for hereditary diseases and blood diseases. Another interesting development concerns the use of CAR T-cell therapy in patients with advanced blood cancers.

A CAR-T therapy consists in the infusion of engineered T-cells that express a Chimeric Antigen Receptor (CAR). This receptor has external target-binding domain designed to recognize a specific tumor antigen. The most common procedure for CAR-T therapy consists in the extraction of T-cells from the own patient, which are then genetically modified and expanded in vitro. Finally, they are reinfused into the patient, ready to fight the tumor.

**Do you think that new biotechnologies could be important for patients?**

Yes. Although they were tested only on a few patients, the recent successes of biotechnologies applied to medicine demonstrate that they can be extremely powerful tools in several pathologies identified as unmet medical needs.

**In your opinion, what are the most promising biotechnologies in your sector?**

Firstly, the monoclonal antibody therapy is giving excellent results in the cure of several diseases such as auto-immune diseases and cancers. Next to that, there is the ever-growing field of genetic therapies, both ex-vivo and in vivo. They delivered promising results in the case of hemophilia, blindness and hereditary diseases of the hematopoietic system, e.g. thalassemia, sickle cell anemia, but also leukemia, etc.

**According to your experience, are patients concerned with the use of genetic therapies?**

The position of the patients towards gene therapy is strictly dependent on the balance benefit/risk in comparison with existing treatments. The severity of the diseases and the absence of satisfying treatments allow us (physicians and patients) to cautiously choose for alternatives as in the case of gene correction of the somatic cells. So far, the clinical results obtained fulfil our expectancies in terms of benefits for the patients and since 2006 we didn't observe any severe adverse event.

...Following the severity of the disease and after a balanced appreciation of the benefits and risks, patients and physicians take a cautious decision in favor of gene therapy...

*Dr. Marina Cavazzana, Necker Hospital.*

*Dr. Marina Cavazzana is Head of the Department of Biotherapy at the Necker Hospital, Paris.*

**What is plant breeding?**

Plant breeding is the art and science of changing the traits of plants in order to produce desired characteristics to improve the overall function of various plants and crop systems.

With the predicted growth in the global population and the effects of climate change, varieties with increased yields and resistance to drought and disease are critical if we are to provide enough food for future generations. Plant breeding is one of the tools that will help us achieve sustainable crop production in the long term.

**About the NBT Platform**

The NBT Platform is a coalition of SMEs, large industry representatives and members of prominent academic and research institutes. Its aim is to provide policy makers and stakeholders with clear and precise information on NBTs and to generate awareness about their benefits for the European economy and society.

More information on [www.nbtplatform.org](http://www.nbtplatform.org), or contact us via [info@nbtplatform.org](mailto:info@nbtplatform.org).