



Press Release Evry, October 4, 19h00

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2012 Prix Galien France Genethon receives the 2012 Prix Galien France for its Innovative Treatments in Gene Therapy

The 43rd Prix Galien France today recognized Genethon for its innovative treatments of rare diseases using gene therapy and awarded it the prize in the category of "Medicines destined for rare diseases – cell therapy and gene therapy." Created in 1990 by a patient association, the AFM-Téléthon, and 90% financed by donations from the French Téléthon, Genethon is dedicated to the development of gene therapy treatments for rare diseases - from their discovery through production and through their preclinical and clinical development. This is the first time that a non-profit organization, created by a patient association, has received the PRIX GALIEN FRANCE.

Frédéric Revah, Chief Executive Officer of Genethon, said "This prestigious prize, awarded to a laboratory created by a patient association and funded by public generosity, confirms the incontestable role of Genethon as world leader in the field of gene therapy, and recognizes its major contribution to medicine in general and to the treatment of rare diseases specifically."

Laurence Tiennot-Herment, Chairman of the AFM-Téléthon and of Genethon, said "I would like to dedicate this prize to the donors and partners of the Téléthon who have supported Genethon since its inception and who have made possible its development. Without them this incredible adventure never could have happened. Thanks to their continued support we have tackled the most audacious challenges, from producing the first maps of the human genome to the production today of medicines using gene therapy."

Following its inception in the 90's, Genethon was recognized for creating the first maps of the human genome which immediately were made available to the world scientific community. Genethon also made history in gene therapy with major breakthroughs which included exon-skipping by AAV, the first gene therapy treatment of a neuromuscular disease (gamma sarcoglycanopathy) and the first trans-Atlantic (France/England/USA) multicenter, gene therapy clinical trial in immunodeficiency. To respond to the needs of production, Genethon has evolved and now has the largest bio-production center in the world for gene therapy products suitable for use in humans, Genethon-Bioprod. This facility is unique and essential to respond to the needs of researchers conducting human trials for rare diseases. The creation of Genethon-Bioprod has transformed Genethon which now has moved from the age of research into the age of pre-industrialization.

Invitation to the Press Thursday 18 October from 10h00 à 13h30, in Evry France Come discover Genethon and Genethon Bioprod

- More than 200 researchers, doctors, pharmacists, engineers and medical and regulatory specialists,
- 10,000 m² of laboratories.
- R&D teams dedicated to the discovery, preclinical and clinical development of gene therapy treatments for rare diseases, as well as bio-manufacturing and process development.
- The world's largest-capacity manufacturing center for gene therapy products under GMP (good manufacturing practices): **5,000 m²** dedicated to bio-manufacturing and quality control.
- The largest collection of DNA and cells in Europe for rare genetic diseases: 236,000 samples from 73,000 patients covering 413 maladies.

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