

Annual Congress on

Rare Diseases & Orphan Drugs

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AFM-Telethon, France

How patient organizations can change the game in innovation and drug marketing: Example of AFMTelethon

Partnerships between industry, public institutions and charity organizations may lead to win-win situations and new models for advanced therapeutic medicinal products development. The example of AFMTelethon, a patient organization dedicated to rare diseases, will show why the caricatural vision of patient associations in an activism, awareness and emotional support holding pattern, should slip our mind. AFMTelethon has raised >\$100M/year for 29 years, which helped to reframe the whole field, bringing it from a rather remote scientific and commercially unattractive area to a very active and innovative ground. A striking demonstration is the comprehensive genetic map of the human genome published in 1992 and 1996 by its non-for-profit biotech Genethon; a quantum leaps in the human genetics field. Not only patient organizations can serve as disease-specific experts, they more importantly work on reducing bottlenecks/barriers for all levels of the process of product development, initiate and drive research projects, provide tools (e.g., databases, registries, large animal facilities, high-throughput screening and even GMP biotherapeutics platforms) and represent a voice for all aspects (ethical, regulatory, technical) of innovative drug developments. Through AFMTelethon initiatives, examples will be given of successful ecosystems, which also serve as models for frequent disorders. This includes shared scientific and financial risks in translational research by directly funding and supporting drug development programs in industry or in academia, putting in place its own research and development facilities, generating innovative drugs that are now either in the clinic or approved, founding start-up companies out of its own portfolio, taking equity shares in biotech companies, launching a venture capital fund and a new pharmaceutical entity together with the French Public Investment Bank and launching a new type of private company dedicated to cell and gene therapy. The different actions presented are also driven by the growing challenge of fair price of the therapeutics will be addressed.

Biography

Serge Braun is a Scientific Director of AFM-Telethon since 2005, the French Muscular Dystrophy Association and President of Genosafe, a CRO company dedicate to QC of biotherapeutic products. He has obtained his degree in Pharmacy, PhD in Pharmacology from the Louis Pasteur University of Strasbourg, France and was a Postdoctoral Fellow at the Neuromuscular Center, School of Medicine of the University of Southern California in Los Angeles, USA. In 1995, he has joined Transgene SA (Strasbourg, France), the largest French biotech company, where he became Vice-President Research and developed his career in the field of gene therapy of genetic diseases and of immunotherapy of cancer. He was also Co-Founder of Neurofit; a contract research organization specialized in preclinical testing of both the central and the peripheral nervous system. He is a Member of the French National Academy of Pharmacy.

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