



# RNAi - A new chance for gene therapy?

Public panel discussion on ethical aspects  
of RNA interference based gene therapy

3rd December 2007  
Berlin-Brandenburg Academy  
of Sciences and Humanities  
Gendarmenmarkt, Berlin  
16.00 - 19.00

"Introduction to RNA interference and the European research project RIGHT"  
Thomas F. Meyer (Berlin)

## Panel participants

**Marina Cavazzana-Calvo** (Paris)  
"Gene therapy for primary immunodeficiencies"

**Luigi Naldini** (Milan)  
"How to use RNA interference for gene therapy of inherited diseases"

**Christoph Rehmann-Sutter** (Basel)  
"A new twist for gene therapy ethics or the same reloaded?"

**Dietmar Mieth** (Tuebingen)  
"Gene Therapy - Progress and Responsibilities"

**Moderation:**  
**Volker Stollorz** (Cologne)

### BENEFITS and RISKS of Silencing Gene Therapy A critical appraisal

**Contemporary medicines** used for the treatment of devastating human diseases, including cancer, neuro-degeneration, chronic inflammation and autoimmune processes rely on the modulation of aberrant molecular processes inside the cells of our bodies. Many conventional treatments, however, are connected with severe side effects or hampered by the occurrence of treatment resistances.

**RNA interference** (RNAi) is a naturally occurring mechanism that leads to the "silencing" of a gene. In consequence, the respective protein is no longer synthesised. New trials explore the feasibility of RNAi for research and therapy, but there are hints that the technique might induce immune reactions or silence the wrong gene or genes. Furthermore, the targeted delivery of the RNA inhibitors only to the affected organ or cells is still a challenge.

**Future treatment** options for gene therapy can build upon this new approach, which promises to be more specific and more versatile compared to conventional treatments. RNAi could be applied to turn off genes that are overactive in diseases such as cancer or to disable genes needed by an invading virus, e.g. HIV. It is the goal of the European RIGHT consortium to explore this new strategy and to elucidate the pros and cons.