FONDAZIONE
PER LA RICERCA FARMACOLOGICA
GIANNI BENZI
ONLUS

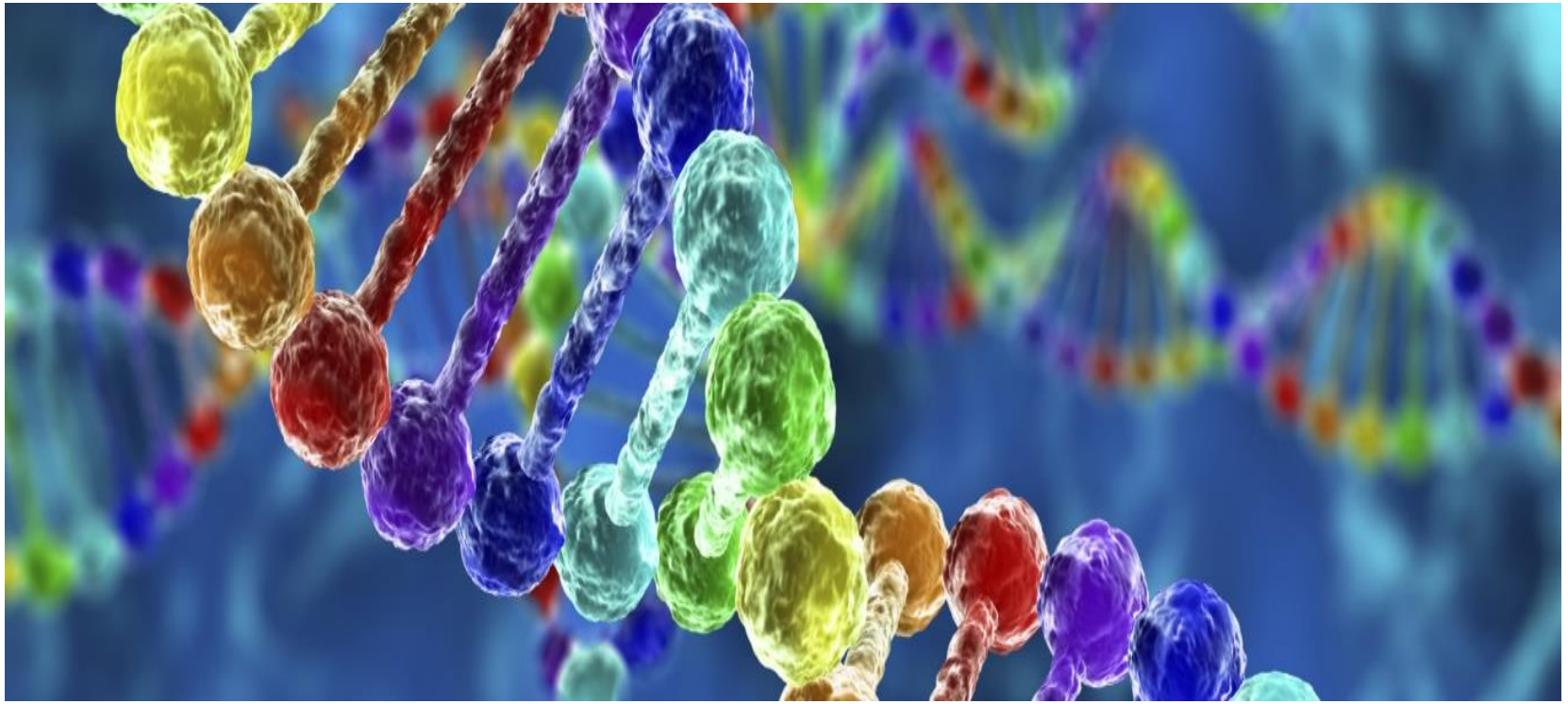
XII Foresight Training Course

Innovative medicine and research: ethical, legal and regulatory issues

Toulouse – 27/09/2019

Fondazione per la Ricerca Farmacologica Gianni Benzi onlus

Via Abate Eustasio, 30 – 70010 Valenzano (BA) Tel.: +39 080 2052499
www.benzifoundation.org



7th Conference of the European Association of Health Law

INNOVATIVE MEDICINE AND RESEARCH: ETHICAL, LEGAL AND REGULATORY ISSUES

Exploring solutions to foster ATMP's development and access to patients in Europe

Vincenzo Salvatore
Full Professor of European Union Law

Of Counsel – Focus Team Leader Healthcare
Healthcare and Life Sciences –BonelliErede, Milan

Department of Economics
University of Insubria – Varese

***Toulouse* 27 September 2019**

Outline

- The current scenario
- Critical factors that have influenced ATMP success rates
- Possible ways to foster development and expand patients' access
- The European Commission and EMA Action Plan on ATMPs
- The outstanding challenges

The current scenario

- ATMPs comprise gene therapies, tissue engineered products and somatic cell therapies
- Not a success story
- Only 10 ATMPs authorised in the EU since Regulation 1397/2007 entered into force
- 3 subsequently withdrawn and 1 suspended
- mainly developed in academic environment, where industrial regulatory expertise and marketing knowledge may lack

Why?

- Complexity of clinical trials
- Supply chain management
- Lack of standardisation in manufacturing procedures
- Unavailability of enough evidence of comparable clinical effectiveness
- Commercialisation hurdles
- High range costs of reimbursement for the national health systems

How to foster ATMP development

- Engage pharmaceutical industry and venture capital investors (*e.g.*: CAR-T)
- Apply for SMEs status whenever eligible
- Creation of a data base of accredited establishments in Europe
- Implementation across the EU of a Cell History/Master File
- Non-authorisation of hospital exemption when authorised ATMP is available

Accelerating patients' access

- More frequent interaction, informal and real-time dialogue between ATMPs developers and regulatory agencies (*e.g.*: ITF)
- Free scientific advice
- Exploit early access options
- Anticipate discussion with HTA bodies (parallel advice)
- Encourage funding and investments through financial incentives at EU and national level

The EC/EMA Action plan on ATMPs

- Strengthen multi-stakeholder cooperation (EMA, EC, NCAs, HTAs)
- The EC/EMA action plan
- List of proposed actions
- Issuing of ATMP specific guidelines (GMP, GCP) and Q&A documents (GLP)
- Application of a risk-based approach
- Development of targeted communication/training material

Thank you!

VINCENZO SALVATORE

Full Professor of European Union Law

Department of Economics – University of Insubria

Of Counsel, Focus Team leader Healthcare and Life Sciences – **BonelliErede**

vincenzo.salvatore@belex.com

vincenzo.salvatore@uninsubria.it