



A Ferrara University
Spin off

RARESPLICE s.r.l.

RNA BASED THERAPEUTICS,
A R&D CUTTING EDGE

MISSION: to discover, patent and develop innovative biotechnological drugs (Gene Therapy Medicinal Products) for the cure of Rare Diseases of genetic origin, characterized by high unmet medical needs.



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THE IDEA:

RareSplice srl is a Spin off of the University of Ferrara.

The business idea is based on the development of a highly advanced biotechnological platform permitting the identification, characterization and production of new molecules (new molecular entities/NME), covered by patent, for the therapy of Rare Human Diseases of genetic origin.

This has been possible thanks to the significant wealth of knowledge and complementary expertise of founders, highly skilled in the fields of the molecular bases of genetic disorders and of the Rare Disease market.



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HISTORY:

The project started in 2008 from the synergistic collaboration between Researchers of the University of Ferrara and of the International Centre for Genetic Engineering and Biotechnology (ICGEB) in Trieste.

Rare Splice Srl was founded in 2012 with the financial support of the Italian Ministry of the for Instruction, University and Research (MIUR) and Ministry of Economic Development (MSE)



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INNOVATIVE FEATURES:

The therapeutic approach consists of “new molecular entities (NMEs) for «RNA-based therapies», highly innovative, patented, for the cure of Rare Diseases of Genetic origin in humans.

The biological target is the messenger RNA (mRNA) in the cell nucleus, and its processing (splicing).

From the regulatory perspective (EMA) these molecules fit within the **Gene Therapy Medicinal Products (GTMPs)**

SHORT-MEDIUM TERM OBJECTIVES:

Strategic partnerships with Pharmaceutical Companies, Business Angel /Venture Capital , etc., interested in supporting the pre-clinical and clinical development of the identified NMEs



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FIELD:
Pharmaceutic / Biotechnology

- TARGET:**
- **International Pharmaceutical Company with a strong R&D organization**
 - **Foundations**
 - **Private Equity**
 - **Venture Capital**



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THE CURRENT RESEARCH IS FOCUS ON THERAPIES FOR
NEUROLOGICAL DISORDERS AFFECTING NEWBORNS, LETHAL
AND WITH NO CURE:

- **Spinal Muscular Atrophy (SMA)**
- **Familial Dysautonomia (FD)**



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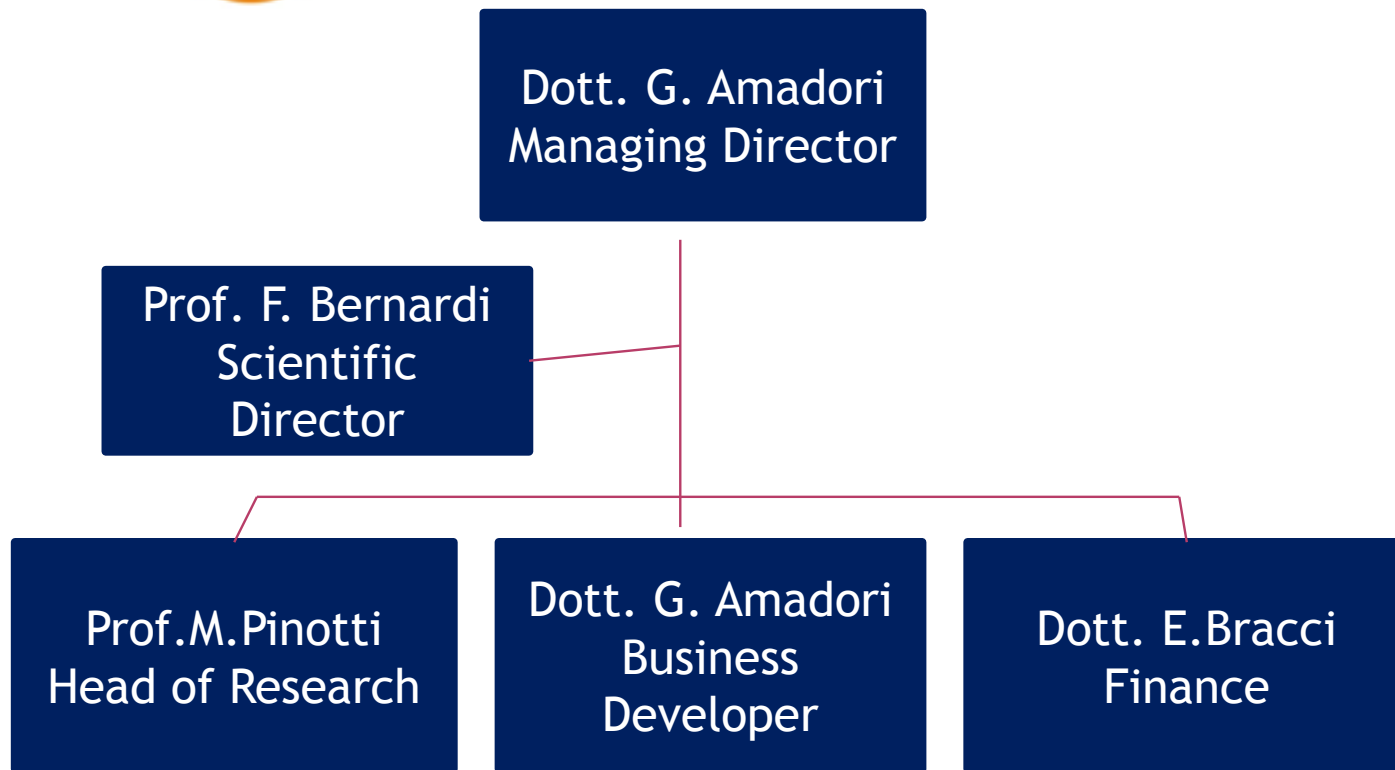


➤ **FOUNDING PARTNERS**

- **Francesco Bernardi**, Full-Professor of Biochemistry at the University of Ferrara.
- **Mirko Pinotti**, Associate Professor of Biochemistry at the University of Ferrara.
- **Leonardo Guerra**, expert in the pharmaceutical market of rare diseases.
- **University of Ferrara (UniFE)**



ORGANIZATION CHART





LATEST NEWS

Strategic agreement between **RareSplice srl**, spin-off of the Ferrara University, and **UniQure B.V**, pharmaceutical company leader in the field of Gene Therapy

Ferrara, July 1st, 2015

RareSplice has signed an important scientific collaboration agreement with UniQure to conduct pre-clinical studies aimed at developing an innovative gene therapy for Spinal Muscular Atrophy (SMA), a rare neuromuscular disorder of genetic origin that affects one over 6.000 newborns. There is currently no cure and the outcome is often fatal.

The agreement implies the strategic collaboration with the research group headed by Prof. **Franco Pagani** at the **International Centre for Genetic Engineering and Biotechnology (ICGEB)** in Trieste (Italy).

ICGEB is an international, nonprofit research organization dedicated to advanced research and training in molecular biology and biotechnology and holds out the prospect of advancing knowledge and applying the latest techniques in the fields of biomedicine, crop improvement, environmental protection/remediation, biopharmaceuticals and biopesticide. Prof. Pagani leads the Human Molecular Genetics group and is internationally recognized for his studies in the modulation of RNA processing. For more info: <http://www.ICGEB.org>.

uniQure is a pharmaceutical company based in Amsterdam (NL) and leader in the field of gene therapy. For more information: <http://www.uniquire.com>