



Association
Monégasque
Contre les
Myopathies

International Collaborative Effort (ICE)

Towards clinical trials for DMD using gene and cell therapies combined with exon skipping

exon skipping:

an innovative technique called RNA surgery,
that allows cellular mechanisms “to ignore” the
affected part of the gene carrying the anomaly
responsible for the disease.

2009

€ 1.7 million

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1 goal

Overcome enduring hurdles
in order to initiate clinical trials using exon skipping strategies

2 promoters

Association Monégasque contre les Myopathies
Duchenne Parent Project France

4 countries

France, Italy, United Kingdom, U.S.A

12 institutions

INSERM
University of Milan
University College London
King's College London
Royal Holloway University London
University of Rome
University of Oxford
University of Pennsylvania
National Institute of Health Bethesda
Institut de Myologie Paris
Imperial College London
INSERM-Transfer

15 partners

Thomas Voit, Myology Institute,
Pitié-Salpêtrière Hospital, Paris
Jamel Chelly, Cochin Institute, Cochin Hospital, Paris
Luis Garcia, Pierre & Marie Curie University,
Myology Institute, Paris
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Myology Institute, Paris
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Paris, & University College, London
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Great Ormond Street Hospital, London
Kay Davies, Oxford University
George Dickson, Royal Holloway University, London
Dominic Wells, Imperial College, London
Simon Hughes, King's College, London
Irene Bozzoni, Sapienza University of Rome

Yvan Torrente, University of Milan,
Maggiore Policlinico Hospital, Milan
Lee Sweeney, University of Pennsylvania, Philadelphia
Robert Kotin, NHBL, National Institutes of Health, Bethesda
Anton Ottavi, Inserm-Transfert, Lyon, France

90 researchers and clinicians

6 work packages

- 1 Evaluation of the therapeutic value of truncated dystrophins completed by exon skipping
- 2 Design and upgrading optimized-snRNAs for dystrophin rescue
- 3 Autologous stem cell therapy using LV vectors encoding opt-snRNAs
- 4 Widespread delivery of AAV vectors encoding opt-snRNAs:
Clinical relevance in GRMD
- 5 Design of the future clinical trials
- 6 Management

42 deliverables

4 scientific advisors

Jean-Claude Kaplan (France)
Terry Partridge (UK)
Adrian Trnhaser (UK)
Berch Griggs (USA)

1 scientific coordinator

Luis Garcia (France)

Associated research laboratories

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€ 350 000

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