



## NANTES 2026 GENE & CELL-BASED APPROACHES IN NEUROMUSCULAR DISEASE

14-16 January 2026  
IRS2 Nantes Biotech, Nantes, France

### ACADEMY PROGRAMME

#### Wednesday 14 January

11:30-12:00

##### Welcome

Introduction round and selection of the order of the student presentation

12:00-12:45

##### Lunch

12:45-13:30

##### Hildegard Büning, Hannover Medical School

Adeno-Associated Virus (AAV) vectors as delivery tools in neuromuscular diseases

13:30-14:00

##### Caroline Le Guiner, Target, Nantes

Gene therapy for Duchenne Muscular Dystrophy: The journey of an rAAV- $\mu$ Dystrophin product

14:00-15:30 Student talks 1, 2 & 3

15:30-16:00

##### Coffee Break

16:00-16:30

##### Mario Amendola, Genethon

Genome Editing and AAV Delivery for Utrophin-mediated Duchenne Muscular Dystrophy Therapy

16:30-18:00 Student talks 4, 5, 6

18:30

##### Surprise Networking evening



## Thursday 15 January

08:30-09:00

**Isabelle Richard, Genethon**

Therapeutic Challenges in Muscular Dystrophies

09:00-09:30

**Federic Relaix, UPEC Health Faculty, INSERM, ENVA, EFS & APHP**

Rat Duchenne muscular dystrophy models for preclinical studies and deciphering tissue repair mechanisms

09:30-10:30 Student talks 6 & 7

10:30-11:00

**Coffee Break**

11:00-11:30

**Sonia Albini, Genethon**

Profibrotic iPSC-derived MYOrganoids as a translational model for gene therapy evaluation in Duchenne Muscular Dystrophy

11:30-12:30 Student talks 8 & 9

12:30-14:00

**Lunch**

14:00-14:30

**Sumitava Dastidar, UCL**

Human Multi-Organoid Platform for Preclinical Evaluation of Neuromuscular Gene Therapies

14:30-16:00 Student talks 10, 11

16:00-16:30

**Coffee Break**

16:30-17:30

**Gloria González-Aseguinolaza, CIMA, University of Navarra, Pamplona**

Between academia and industry: juggling roles to deliver solutions to patients

17:30-18:30

**Visit TARGET Lab**

19:30-

**Dinner**

## Friday 16 January

09:15-10:00

**Yann Pereon, Rare Diseases Reference Center at Nantes University Hospital**

Gene therapy treatments for patients with neuromuscular diseases

10:00-10:45

Discussion with parent of 2 young patients

10:45-11:15

**Coffee Break**

11:15-12:00

**Capucine Trollet, Institut de Myologie**

Cell and Gene Therapy Approaches for Oculopharyngeal Muscular Dystrophy

12:00-12:15

**Closing**