

Organizers:

Margaret Buckingham and Giulio Cossu

Venue: Grand Hotel Villa Serbelloni,
Via Roma, 1 - 22021 Bellagio, CO - Italy

**TOWARDS CLINICAL TRIALS
IN MUSCULAR DYSTROPHY**
April 3-4, 2007

Tuesday 3 April

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| 1.00 pm | Lunch |
| | <u>Chair: Olle Lindvall – Lund University, Sweden</u> |
| 2.00-2.20 | Margaret Buckingham – Pasteur Institute, France
<i>From cells to therapy</i> |
| 2.20-2.50 | Jennifer Morgan - Imperial College London, UK
<i>Muscle stem cells as a model system to test antisense oligonucleotides designed to skip dystrophin exons</i> |
| 2.50-3.20 | Yvan Torrente - University of Milan, Italy
<i>Myogenic potential of CD133+ Stem Cells</i> |
| 3.20-3.50 | Giulio Cossu – San Raffaele, Italy
<i>Mesoangioblast cell therapy</i> |
| 3.50-5.00 | General discussion (coffee available) |
| | <u>Chair: Sharaghim Tajbakhsh – Pasteur Institute, France</u> |
| 5.00-5.30 | Terence Partridge - Children's National Medical Center, US
<i>Therapeutic exon skipping in the dystrophin gene</i> |
| 5.30-6.00 | Annemieke Aartsma-Rus - Leiden University Medical Center, Netherlands |
| 6.00-6.30 | Dominic J Wells - Imperial College Faculty of Medicine
<i>Improved delivery of morpholino antisense oligomers to skeletal muscle and heart</i> |
| 6.30-7.30 | General discussion |
| 8.00 | Dinner |

Wednesday 4 April

Chair: Maurilio Sampaolesi

- 9.00-9.30 **Kay Davies** - University of Oxford, UK
Utrophin in the therapy of DMD
- 9.30-10.00 **Lorenzo Puri** - Dulbecco Telethon Institute, Italy
Beneficial effect of deacetylase inhibitors on dystrophic muscles
- 10.00-10.30 **Luis Garcia** - Genethon & CNRS, France
Muscle function recovery in GRMD dystrophic dog after exon skipping gene therapy
- 10.30-11.00 Coffee break
- 11.00-11.30 **Lee Sweeney** - University of Pennsylvania, US
Suppression of premature stop codons as a treatment for a subset of DMD
- 11.30-12.00 **Silvia Brunelli** – Milano University, Italy
Nitric oxide with non-steroidal anti-inflammatory drugs ameliorates muscular dystrophy and enhances stem cell therapy
- 12.00-12.40 General discussion
- 1.00 pm Departure