



COST Action CA17103

Action Title: Delivery of Antisense RNA Therapeutics

Training School:

The guide to antisense therapy development

Draft programme

IS3, Porto, 3-7 Feb 2020

Duration: 1 week

Scientific Organizers

Shalini Andersson

Willeke van Roon-Mom

Annemieke Aartsma-Rus

ECl co-organizers

Ines Potocnjak and Liliana Matos

Local Organizers

Training school
The guide to antisense therapy development
3-7 Feb 2020
i3S, Porto

Alexandra Moreira, i3S/IBMC, ICBAS, Porto
Sandra Alves, INSA, Porto, Portugal

PROGRAM



Funded by the Horizon 2020 Framework Programme
of the European Union



Topics

1. Which modality to choose? (Monday)

Purpose of this session:

- Outline different types of oligonucleotide modalities
- Explain the need for chemical modifications
- Outline the consideration for biodistribution

2. How to design your antisense oligonucleotide? (Tuesday)

Purpose of this session

- Understand considerations for oligo design for different modalities
- Explain the basics of preclinical studies for pharmaceuticals
- Apply what you have learnt

3. How to test your antisense oligonucleotide? (Wednesday-Thursday)

Purpose of this session

- Understand how to perform in vitro and in vivo tests with oligonucleotides
- Understand different model systems and which to select
- Understand outcome measures and which to select
- Understand the need for proper controls
- Understand the relevance of preclinical studies
- Best practice for Industry Academic collaborations

4. Towards the clinic (Friday)

Purpose of this session

- Understand the stages of clinical trial and trial design
- Understand ethical considerations for trials
- Understand how drugs are approved
- Understand the data needed for drug approval

Schedule (tentative)

Monday, February 3 (Chair Willeke van Roon-Mom)

10.30 Welcome, outline of training school and brief round of introduction (Annemieke Aartsma-Rus and Shalini Andersson)

11.00 Brief introduction to all things oligonucleotide (Annemieke Aartsma-Rus)

12.00 Considerations for biodistribution (Shalini Andersson)

13.00 Lunch

14.00 Workshop : 4 use cases: which approach to use best?

15.00 Feedback from use cases (5 minutes per group)

15.30 Coffee/tea break

16.00 Bring your own use case: apply what was learnt to your own disease/target gene

17.00 End of day

Tuesday February 4 (Chair Alexandra Moreira)

9.00 How to design and exon skipping oligo (Annemieke Aartsma-Rus)

10.30 Coffee

11.00 How to design siRNA (Michela Denti)

12.00 How to design RNase H AONs (Anders Dahlen)

13.00 Lunch

14.00 Do it yourself: design your own oligo (either for your target gene or using a target from the use cases from yesterday)

15.30 Coffee/tea break

16.00 Pharmacology 101 (ADME/DMPK/Safety) (Carl Amillon)

17.00 End of day

Wednesday February 5 (Chair Annemieke Aartsma-Rus)

In preparation for this day: Watch OTS webinar on using proper controls

10.00 What is needed to provide proof-of-concept? (Willeke van Roon-Mom)

10.30 Coffee

11.30 How to select the best model system (Carl Amilon)

12.30 Model systems for neuronal diseases (Willeke van Roon-Mom)

13.00 Lunch

14.30 How to select the best outcome measure (Shalini Andersson)

15.30 Coffee/tea break

16.30 Interactive panel session on experience working and career perspectives in Industry/Academia (Shalini Andersson, Annemieke, Carl Amilon, Willeke van Roon-Mom)

17.30 End of day

Thursday February 6 (Chair Shalini Andersson)

9.00 Considerations for preclinical studies and the TACT model (Annemieke Aartsma-Rus)

10.30 Coffee

11.00 Academic/Industry collaborations (Willeke van Roon-Mom and Shalini Andersson)

12.30 Interactive Q&A session on how to collaborate best with Industry

13.00 Lunch

14.30 Mock TACT session

15.30 Clinical trials (Ines Potocnjak)

16.30 End of day (free until networking dinner)

20.00 Networking dinner



Friday February 7 (Chair Sandra Alves)

9.00 Workshop on ethical considerations for clinical trials

9.45 How are drugs approved? (tbc)

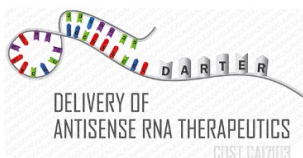
10.30 Coffee

11.00 What do you need to know to prepare for drug approval (**Marie Elebring**)

12.00 Case report: what if you did not prepare well for drug approval (Annemieke Aartsma-Rus)

13.00 Evaluation/feedback

14.00 Lunch and departure



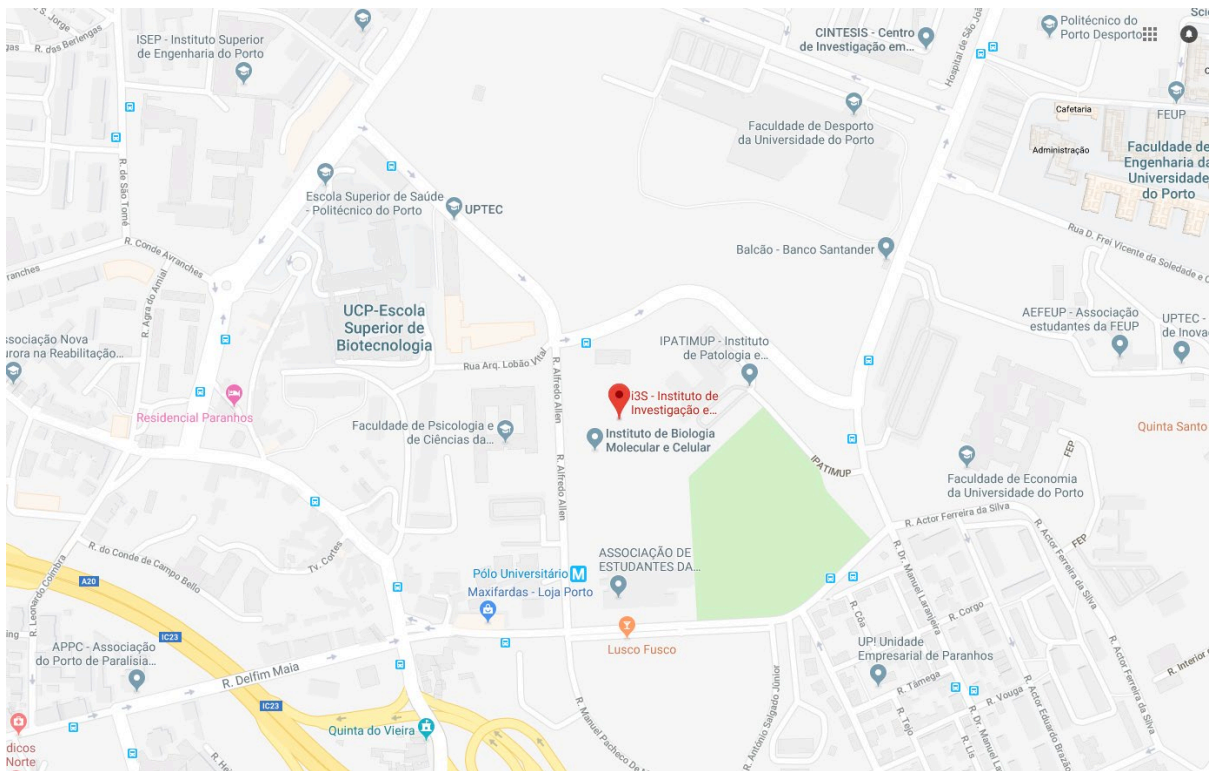
Venue

i3S - Instituto de Investigação e Inovação da Universidade do Porto

Rua Alfredo Allen, 208; 4200-135 Porto, Portugal

Tel: +351 220 408 800 | Site: www.i3s.up.pt | E-mail: events@i3s.up.pt

GPS coordinates: 41° 10' 30.008" N, 8° 36' 12.488" W.



Travel Info

A. Arriving at Porto by train

Take the train at Campanhã train station or São Bento train station. Metro or bus are available from both stations to the venue. However, the metro is the easiest way to reach i3S.

From Campanhã train station:

Metro: All lines are possible (A, B, C, E and F) | Destination Station: Trindade and then change to line D - yellow (destination Hospital São João) – and leave at station: **Pólo Universitário** | Average time: 16 minutes | For more information: <http://www.metrodoporto.pt/en/>

From São Bento train station:

Metro: Line D - yellow | Destination Station: Hospital São João; leave at station: **Pólo Universitário** (no connections – direct line) | Average time: 10 minutes.
For more information: <http://www.metrodoporto.pt/en/>



B. Arriving at Porto by car

The i3S Institute - GPS coordinates are: 41° 10' 30.008" N, 8° 36' 12.488" W.

Please note that i3S **does not offer parking space**.

Important Information

Registration

Certificate of attendance

The certificate of attendance will be sent by email at the end of the meeting.

Internet Access

Wireless Internet is available for free.

The network is i3S_Temp and the password is Password2015.

Sponsors

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