ECTRIMS
EUROPEAN COMMITTEE FOR TREATMENT AND RESEARCH IN MULTIPLE SCLEROSIS

1st ECTRIMS Summer School

Phase IV studies: New methods in pharmaco-vigilance and monitoring drug effectiveness in Multiple Sclerosis

25 - 27 June 2013
Bari, Italy

The ECTRIMS Summer School in *Phase IV studies: New methods in pharmaco-vigilance and monitoring drug effectiveness in Multiple Sclerosis* is directed at young MS investigators with an interest in registries and their use in post-marketing studies.

The immediate objective is to equip clinicians with the knowledge and expertise they need to conduct phase IV studies aimed to evaluate the effectiveness and safety of drugs in MS in clinical practice.

The course will provide a detailed introduction to:

1. Concepts of pharmaco-epidemiology and pharmaco-vigilance
2. Study designs of phase IV studies in MS
3. Potential applications of existing large MS patient databases and registries to study important questions regarding clinical risk factors, treatments and outcomes
4. Fundamentals of different types of measures (clinical, MRI and laboratory) used for demonstrating improvement/worsening in patient outcomes and risk/benefit and cost-effectiveness effects of drugs in clinical practice
5. The theory and application of statistical techniques that are commonly used in phase IV studies

The faculty will be asked to select papers which will be sent to the participants with a “reading guide” in order for them to exercise their ability to spot in the current literature what is important and reliable. This self-profile will be confronted during didactical lectures and workshops with the notions proposed by the faculty.

By the end of the course, participants should be able to face practical issues in obtaining, linking and analyzing large databases, to understand all of the key statistical tests, recognize the assumptions behind their analyses, interpret the results and develop project proposals.

ECTRIMS Summer School – Preliminary Programme

24 June 2013: Arrival Day

Arrival in the afternoon.

25 June 2013: Summer School Day 1

08.30-08.45 Opening Session

Welcome and Introduction
M. Trojano (Bari, IT)

08.45-11.10 Didactic lectures 1

M. Clanet (Toulouse, FR), G. Comi (Milan, IT)

08.45 Introduction to Pharmacoepidemiology and Pharmacovigilance: differences in monitoring the steps of clinical development
G. Tognoni (Chieti, IT)

09.25 Discussion

09.40 Efficacy and Risks associated with new DMDs in Multiple Sclerosis
L. Kappos (Basel, CH)
X. Montalban (Barcelona, ES)

10.20 Discussion

10.35 Safety of medical products: pre- and post-marketing strategies
H.P. Hartung (Düsseldorf, DE)

10.55 Discussion

11.10-11.30 Coffee break

11.30-13.15 Didactic lectures 2

M.P. Amato (Florence, IT), P. Soelberg-Sorensen (Copenhagen, DK)

Registries

11.30 Use of disease registries in drug effectiveness assessment
J. Hillert (Stockholm, SE)

11.50 Use of disease registries in drug safety assessment
M. Trojano (Bari, IT)

12.10 Use of administrative registries
V. Lepore (Chieti, IT)

12.30 Discussion

12.45 Observational studies and Registries: how to use the data to fulfill regulatory obligations
M. Sturkenboom (Rotterdam, NL)

13.05 Discussion

13.15-15.00 Lunch break

15.00-17.45 Didactic lectures 3

C. Lubetzki (Paris, FR), D. Miller (London, UK)

15.00 Phase IV study: Essential clinical outcomes
M.P. Amato (Florence, IT)
P. Soelberg-Sorensen (Copenhagen, DK)

15.40 Discussion

15.55 Phase IV study: Essential MRI outcomes
M. Filippi (Milan, IT)
F. Fazekas (Graz, AU)

16.35 Discussion

16.50 Phase IV study: Essential lab outcomes
G. Giovannoni (London UK)
R. Gold (Bochum, DE)

17.30 Discussion

17.45-18.00 Coffee break

18.00-19.55 Didactic lectures 4

G. Cutter (Birmingham, US), X. Montalban (Barcelona, ES)

18.00 Introduction to Biostatistics
M.P. Sormani (Genoa, IT)
F. Pellegrini (Chieti, IT)

18.40 Discussion

18.55 Risk / Benefit assessment: post marketing strategies
J.D. Seeger (Boston, US)

19.15 Discussion

19.25 Cost / Benefit assessment: post marketing strategies
G. Kobelt (Mulhouse, FR)

19.45 Discussion

19.55 End of programme

20.30 Dinner

26 June 2013: Summer School Day 2

08.30-10.30 **Workshops 1 – 3**
See timetable below
10.30-11.00 **Coffee break**

11.00-13.00 **Workshops 1 – 3**
See timetable below
13.00-14.00 **Lunch break**

14.00-16.00 **Workshops 1 – 3**
See timetable below

	Workshop 1	***Workshop 2***	***Workshop 3***
	Phase IV study: Essential clinical, MRI and lab data **Leaders:** **M. Filippi** (Milan, IT), **G. Giovannoni** (London, UK) **J. Hillert** (Stockholm, SE) **Discussants:** F. Fazekas (Graz, AT) H.P. Hartung (Düsseldorf, DE) C. Lubetzki (Paris, FR), D. Miller (London, UK) M. Trojano (Bari, IT)	***Phase IV study: Statistical techniques risk and cost / benefit assessment*** **Leaders:** **G. Cutter** (Birmingham, US) **G. Kobelt** (Mulhouse, FR) **F. Pellegrini** (Chieti, IT) **Discussants:** H. Butzkueven (Melbourne, AU) S. Reingold (Salisbury, US) J.D. Seeger (Boston, US) M.P. Sormani (Genoa, IT) V. Lepore (Chieti, IT)	***Phase IV study: Study designs and interpretation of results*** **Leaders:** **J. Cohen** (Cleveland, US) **L. Kappos** (Basel, CH) **G. Tognoni** (Chieti, IT) **Discussants:** M. Clanet (Toulouse, FR) R. Gold (Bochum, DE) X. Montalban (Barcelona, ES) P. Soelberg-Sorensen (Copenhagen, DK) M. Sturkenboom (Rotterdam, NL)
08.30-10.30	**Group A** (Coordinator: H Tremlett, Vancouver, CA)	**Group B** (Coordinator: E. Waubant, San Francisco, US)	**Group C** (Coordinator: M Pugliatti, Sassari, IT)
11.00-13.00	**Group B** (Coordinator: E. Waubant, San Francisco, US)	**Group C** (Coordinator:M Pugliatti, Sassari, IT)	**Group A** (Coordinator: H Tremlett, Vancouver, CA)
14.00-16.00	**Group C** (Coordinator: M Pugliatti. Sassari, IT)	**Group A** (Coordinator: H Tremlett)	**Group B** (Coordinator: E.Waubant San Francisco, US)

Each workshop will be led by 3 leaders. Additional faculty will be invited as discussants to support the practical discussions during the sessions.

16.00-16.30 Coffee break

17.00-19.00 Plenary Session 1

Facilitators M. Filippi (Milan, IT), G. Cutter (Birmingham, US,) L. Kappos (Basel, CH), C. Lubetzki (Paris, FR)

Proposals and choice of research plans on specific open questions

The session will be led by 4 facilitators. Proposals will be briefly presented and discussed both by WG coordinators/ participants (as a result of their "exposure" to the didactic lectures and the Workshops) and by the facilitators / faculty in order to select 3 themes which will be translated into projects by working groups on Day 3 of the Summer School.

20.00 Dinner

27 June 2013: Summer School Day 3

09.00-12.30 Group work

*Participants will work in 3 small groups (**A ,B, C**) and prepare 3 draft projects (i.e. clinical, MRI and biomarkers projects). Participants will receive feedback from senior investigators.*

13.00-15.00 Lunch break

15.00-17.00 Plenary Session 2

Facilitators M. Clanet (Toulouse, FR), S. Reingold (Salisbury, US), G. Tognoni (Chieti, IT)

Participants' presentations of their acquired knowledge

Participants will make proposals of research plans. Each project will be presented by WG coordinators and discussed. Ideally, these proposals will provide the foundation for future research projects. The session will be lead by 3 facilitators.

17.00-18.00 Closing Session

Summing up
G. Tognoni (Chieti, IT) M. Trojano (Bari, IT)

19.00 Summer School Dinner

28 June 2013: Departure Day

Departure in the morning.