i3du

PhD Programme in Medicines
and Pharmaceutical Innovation

PhD Advanced Course
MOLECULAR BIOMARKERS AND TECHNOLOGIES
July 8 - 12, 2019
Faculty of Pharmacy
Amphitheatre B
Universidade de Lisboa, Portugal

Course Coordinator: Cecília Rodrigues
Course Organizers:
Cecília Rodrigues
Adelaide Fernandes
Elsa Rodrigues
Paula Leandro
Rui Castro
Susana Solá

Teaching staff: Invited speakers

The [FCT PhD Programme in Medicines and Pharmaceutical Innovation](#) (i3DU) trains students in target discovery, drug design, pre-clinical development, and drug safety, bridging the translational gap from discoveries on disease targets and mechanisms into novel diagnostic and therapeutic agents.

The Course on Molecular Biomarkers and Technologies is part of the PhD Programme in Medicines and Pharmaceutical Innovation (i3DU), trains students in the development of advanced medicinal products, covering crucial aspects that determine the fate of drugs in the human or animal body, from their fundamentals to the advanced strategies to overcome the physiological barriers, including innovative technological and therapeutic applications.

The course will be held at the Research Institute for Medicines (iMed.ULisboa), Faculdade de Farmácia, Universidade de Lisboa, in Lisbon, 8- 12 July2019. The training program is aimed at PhD students, but welcomes the participation of external academic and scientific community members. [Registration](#) is free but mandatory.

Short Introduction

Biomarkers are now an integral part of the drug discovery and development process,

acting as indicators of mechanism of action, efficacy, safety and disease progression, as well as assisting in disease diagnosis, patient selection and clinical trial design. Biomarkers also offer the potential to inform treatment decisions and bring personalized medicine into clinical practice.

In early stage drug discovery, biomarkers are used to validate target engagement and modulation. As projects progress, biomarker assays are developed for pharmacokinetic/pharmacodynamic (PK/PD) models, profiling molecules prior to testing in disease models as initial proof of concept. PK/PD models can also assist in dose-to-man scaling predictions for use in clinical trials. Importantly, the biomarker assays developed during the *in vitro* discovery phases are frequently used as efficacy or toxicity endpoints in the clinic. Clinical trials, particularly in oncology, are frequently designed around these biomarkers, in addition to biomarkers of disease progression.

Special focus is also given to the development, robust validation and advancement in technologies towards biomarkers that diagnose, risk stratify and/or monitor disease progression.

Goals and Learning Outcomes

This course is designed to cover principles and applications of biomarkers and assay development, from identification to validation, to impact in drug discovery, and disease diagnosis, prognosis and treatment.

The following learning outcomes are expected:

- Apply on-target cellular readouts to support drug discovery and development programmes;
- Understand efficacy and safety biomarker assays in model systems;
- Explore biomarker formats ranging from nucleic acids, proteins and signaling pathways to cellular phenotypic changes;
- Comprehend technological and methodological platforms for identification and validation of candidate biomarkers for translational and back-translational studies.

PROGRAMME

The course is divided into a balanced blend of lectures on theoretical, practical and laboratory case-based discussions presented during a dedicated course with limited attendance. The training programme has specific slots allocated to seminars and workshops, including informal discussions with lecturers.

Early discovery science requires broad expertise in biomarker identification and assay development across many therapeutic areas. We will focus on oncology, central nervous system, and metabolic diseases and address the development and validation of quantitative biomarker assays in diverse model systems. Ultimately, these biomarkers will progress from the discovery phase to the clinic. A range of technological and methodological platforms will be discussed that can support

biomarker selection, including genomic, proteomic, metabolomic and epigenetic markers for effective translational and back-translational science.

8 July 2019

9h00 **Welcome address**
Cecilia Rodrigues

BIOMARKERS IN NEURODEGENERATIVE DISEASES
Moderators: Susana Solá, Adelaide Fernandes (iMed.ULisboa, FFULisboa)

9h30 **Alzheimer's without dementia**
Alexandre Mendonça
iMM, Faculty of Medicine, University of Lisbon
Lisbon, Portugal

10h30 **Molecular and functional imaging biomarkers in neurodevelopmental and neurodegenerative disorders**
Miguel Castelo Branco
CIBIT, Faculty of Medicine, University of Coimbra
Coimbra, Portugal

11h30 **Break**

12h00 **Biosensing technologies for neurodegenerative disease diagnostics and monitoring**
Inês Pinto
International Iberian Nanotechnology Laboratory (INL)
Braga, Portugal

13h00 **Lunch break**

14h30 **Workshop**
PhD Students ONLY
Imaging tools in biomarker discovery
Liana Silva and Adelaide Fernandes
iMed.ULisboa, Faculty of Pharmacy, University of Lisbon
Lisbon, Portugal

17h00 **End of day**

9 July 2019

BIOMARKERS IN CANCER
Moderators: Rui Castro, Susana Solá (iMed.ULisboa, FFULisboa)

9h30   **Extracellular vesicles: biomarkers and beyond**
Bruno Costa-Silva
Champalimaud Centre for the Unknown
Lisbon, Portugal

10h30   **Exploiting epigenetic-based cancer biomarkers in liquid biopsies**
Carmen Jerónimo
Instituto Português de Oncologia do Porto
Instituto de Ciências Biomédicas Abel Salazar, University of Porto
Porto, Portugal

11h30   **Break**

12h00   **Key cellular and molecular regulators of malignant brain tumors**
Bruno Costa
University of Minho
Braga, Portugal

13h00   **Lunch break**

14h30   **Workshop**
        Personalised medicine in cancer
Pedro Borralho
Novartis, Portugal

17h00   **End of day**

10 July 2019

BIOMARKERS IN METABOLIC LIVER DISEASE

In co-organization with the III ETN Foie Gras Summer School
Biomarkers for NAFLD Staging, Prognosis and Treatment

Session I: Clinical burden, invasive and non-invasive diagnosis
Moderators: Cecília Rodrigues, Carina Prip-Buus

9h30   **Global perspectives on NAFLD and non-alcoholic steatohepatitis**
Helena Cortez-Pinto
Hospital de Santa Maria, Faculty of Medicine, University of Lisbon
Lisbon, Portugal

10h30   **The pathologist's view of fatty liver**
Carolin Lackner
Medical University of Graz
Graz, Austria

11h30 **Break**

12h00 **Technology advancement in biomarker research using laser capture microdissection and proteomics**
Frédéric Saltel
INSERM, Université Bordeaux
Bordeaux, France

13h00 **Lunch break**

14h00 **Bioinformatics in biomarker and assay development**
José Pereira Leal
Ophiomics – Precision Medicine
Lisbon, Portugal

15h00 **Workshop 1: How to write a research project?**
Cecília Rodrigues
iMed.ULisboa, Faculty of Pharmacy, University of Lisbon
Lisbon, Portugal

15h45 **Break**

16h00 **Workshop I: Group discussions**
Cecília Rodrigues, Rui Castro, Marta Afonso, André Santos
iMed.ULisboa, Faculty of Pharmacy, University of Lisbon
Lisbon, Portugal

18h00 **End of day**

11 July 2019

BIOMARKERS IN METABOLIC LIVER DISEASE

In co-organization with the III ETN Foie Gras Summer School
Biomarkers for NAFLD Staging, Prognosis and Treatment

Session II: Experimental biomarkers and therapeutic opportunities
Moderators: Carina Prip-Buus, Rui Castro

9h30 **Metabolic targets as potential biomarkers of NAFLD**
Catherine Postic
Institut Cochin, INSERM, Université Paris Descartes
Paris, France

10h30 **Microbiota and nutritional hepatopathies (ALD/NAFLD): pathophysiology and biomarkers**
Gabriel Perlemuter
Hôpital Antoine-Béclère, INSERM, Université Paris-Sud

Paris, France

11h30   **Break**

12h00   **Gut microbiota and liver disease – basic science opportunities**
André Santos
iMed.ULisboa, Faculty of Pharmacy, University of Lisbon
Lisbon, Portugal

13h00   **Lunch break**

14h00   **A spotlight on NAFLD pathogenesis, diagnosis and novel therapies**
Cecilia Rodrigues
iMed.ULisboa, Faculty of Pharmacy, University of Lisbon
Lisbon, Portugal

15h00   **Workshop II: miRNAs and potential as biomarkers**
Rui Castro
iMed.ULisboa, Faculty of Pharmacy, University of Lisbon
Lisbon, Portugal

16h00   **Workshop II: Method development for novel non-invasive biomarker profiling**
Andrea Normann and Vanda Serra Marques
Mediagnost, Reutlingen, Germany; iMed.ULisboa, Faculty of Pharmacy, University of Lisbon, Lisbon, Portugal

16h45   **Break**

17h00   **Liver bioengineering and other alchemies for the treatment of liver disease**
Pedro Baptista
Centro de Investigación Biomédica Aragón
Zaragoza, Spain

18h00   **End of day**
End of Summer School

12 July 2019

BIOMARKER TECHNOLOGIES
Moderators: Elsa Rodrigues, Paula Leandro (iMed.ULisboa, FFULisboa)

9h30   **Transcriptomics and bioinformatic approaches for biomarker discovery**
Margarida Gama Carvalho
BioISI, Faculty of Sciences, University of Lisbon
Lisbon, Portugal

10h30	**Computational Biology: bridging computer science and bioengineering for modeling multiscale systems**
	Susana Vinga
	INESC-ID/IDMEC, IST, University of Lisbon
	Lisbon, Portugal

| 11h30 | **Break** |

12h00	**Paper-based, label-free, colorimetric assays of health markers using plasmonic nanoprobes**
	Elvira Fortunato
	CENIMAT, Faculty of Science and Technology, New University of Lisbon
	Caparica, Portugal

| 13h00 | **Lunch break** |

14h30	**Research Project: group discussions**
	Elsa Rodrigues, Susana Solá, Paula Leandro, Adelaide Fernandes
	iMed.ULisboa, Faculty of Pharmacy, University of Lisbon
	Lisbon, Portugal

| 17h00 | **End of course** |

Assessment

Assessment of the course consists in the preparation and submission of a research project, 10,000 characters long (including spaces). Students are grouped to build multidisciplinary teams. Each group works throughout the week on a research project that should reflect the topic of the course, including methodologies and strategies to solve an innovative research question. The project is expected to adhere to the following general structure: a) Title; b) Conceptual hurdle and innovative idea to be tested; c) Plan and methods: d) Relevance of the project (scientific and social impact).

The students will select a broad topic of research and are expected to propose a specific project. This project will be evaluated according to the following criteria and weight: a) Novelty and relevance (30%); b) approach to the problem (30%); c) multidisciplinary angle of the research plan (40%).

Students will attend a workshop on "How to write a research project". Specific slots are allocated in the course programme for group discussions, which will take place in prebooked rooms.