



UE 2.1. Current concepts in Precision Health

15 ECTS

Parcours « Precision Health »

ST6: Novel Mode of actions for personalized drugs

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Thanks to omics, the causal molecular chains underlying diseases and their clinical manifestations are within reach. The transition from symptomatic treatments to "disease modifying" treatments, that directly attack these causal chains, has been carried out recently for several monogenic pathologies or several types of related cancers. For multifactorial diseases, the stratification of patients by pathophysiological mechanism rather than by clinical presentation, makes it possible to search for the most appropriate modes of action, to design targeted therapies and to define precisely responders in clinical trials known as "basket trials". The objective of this seminar is to illustrate the current transition to precision drugs with several concrete examples of novel mode of actions and approaches.

Duration: 3 days

Program

Provisional schedule

<u>Underlined = industrial speakers;</u> 3 days (incl. 1/2day for industry visit) Introductory talk

- Personalized Drug innovation through novel mode of action: Concepts and case studies & High-Throughput Compound Screens (Pr Benoit Deprez, Univ. Lille, Inserm, Institut Pasteur de Lille, U1177) Oncology Session
- Two development strategies in oncology : PI3K inhibitors & TRK inhibitors (Pascal Dao Phan, Bayer)
- Molecules targeting ERAPs towards precision treatments for immune diseases and cancer immunotherapy (Pr R. Deprez-Poulain, IUF, Univ. Lille, Inserm, Institut Pasteur de Lille, U1177
- Targeting the Hippo pathway for precision therapies in cancers and regenerative medicine (Pr P. Cotelle Univ. Lille, CHU Lille, UMR-S-1172)
 - Neurology Session
- Venglustat in GBA related Parkinson disease (Pablo Sardi & Laurent Pradier, Sanofi US & FR
- Innovative and personalized therapeutic strategies for neurodegenerative diseases (Pr David Devos, Univ. Lille, CHU Lille, INSERM U1171 projet I-SITE & Matthieu Fisichella <u>InBrain Pharma</u>).
 Infection Session
- Narrow spectrum antibiotics for tailored treatment of infections (Pr Nicolas Willand Univ. Lille, Inserm, Institut Pasteur de Lille, U1177 & Alain Baulard Univ. Lille, CNRS, Inserm, CHU Lille, Institut Pasteur de Lille, U1019-UMR8204 & Marilyn Bourotte, Bioversys
- Ultrafast Tb strain diagnostic tools for precision TB drugs presciptions Deeplex[®]-Myc-TB, (P Supply CIIL U1019-UMR8204 & <u>Genoscreen</u>)

Metabolic & rare diseases Session

- In-born errors of metabolism: how to address the medical need of ultra-orphan patients (Terence Beghyn, Apteeus)
- MicroRNA-based therapies to target peripheral adipose tissue for obesity treatments. (JS Annicotte, Inserm, Univ. Lille, Institut Pasteur de Lille & EGID, U8199 & <u>Marc Thibonnier Aptamir therapeutics, FR</u>
- Tailored drugs for rare or common mutations in Cystic fibrosis patients (Steven Van Der Plas, Galapagos, NL)