

WP20 FINAL MEETING AGENDA

WP20 Demonstration, Innovation, and Dissemination: Innovative Methodologies in Rare Diseases Clinical Trials (RD CTs)

June 14th - 15th, 2024

Location: Imagine Institute, 24 Bd du Montparnasse, 75015 Paris
6th floor, Amphitheatre

Time	Friday June 14 th , 2024
Day 1: Demonstration and Innovation Projects presentations	
12:00 PM – 1:00 PM	Lunch
1:00 PM - 1:15 PM	Welcoming by EJP RD coordination and by WP 20 leaders
1:15 PM - 4:15 PM	Presentations of WP 20 Demonstration projects <ul style="list-style-type: none"> • EPISTOP-IDEAL: <i>R. Nabbout, RD. Hilgers</i> (Coordinator: Rima Nabbout, Imagine Institute, Necker Hospital, France) • EBStatMax: <i>G. Zimmermann</i> (Coordinator: Johann W Bauer, Paracelsus Medical University, Austria) • Improve-PSP: <i>G. Höglinger</i> (Coordinator: Günter Höglinger, Hannover Medical School, Germany)
4:15 PM – 4:45 PM	Coffee break
4:45 PM – 6:45 PM	Presentations of WP 20 Innovation projects <ul style="list-style-type: none"> • i-STORE: <i>R. Nabbout, G. Molenberghs</i> (Coordinator: Rima Nabbout, Imagine Institute, Necker Hospital, France) • Evidence-RND: <i>M. Karlsson</i> (Coordinator: Matthis Synofzik, Center for Neurology & Hertie-Institute for Clinical Brain Research, Germany)
7:30 PM	Dinner

Time	Saturday June 15 th , 2024
Day 2: Course on innovative statistical methods to efficiently obtain and deliver empirical evidence in rare diseases	
9:00 AM - 9:45 AM	Lecture 1: Natural history studies – Is my registry data rich enough? Is it not too messy? <i>G. Molenberghs</i> (Hasselt University and KU Leuven, Belgium)
9:45 AM - 10:30 AM	Lecture 2: Multiple Endpoints - Formulation and Implementation. <i>J. Verbeeck</i> (Hasselt University, Belgium)
10:30 AM -11:00 AM	Coffee Break
11:00 AM - 11:45 AM	Lecture 3: Item Response Theory – How can it aid the development and use of clinical outcome assessments? <i>M. Karlsson</i> (Uppsala University, Sweden)
11:45 AM - 12:30 PM	Lecture 4: Historical control data - When the ethics of a control arm is called into question? <i>TBC</i>
12:30 PM - 1:30 PM	Lunch
1:30 PM - 2:15 PM	Lecture 5: Similarity – Do I need another study? <i>L.Koletzko</i> (Ruhr University Bochum, Germany)
2:15 PM - 3:00 PM	Lecture 6: Random Allocation - Is Randomization always successful? And how to harness randomization for success. <i>RD. Hilgers</i> (RWTH Aachen University, Germany)
3:00 PM - 3:15 PM	Coffee Break
3:15 PM - 4:00 PM	Lecture 7: Repeated measures and missing data. <i>G. Molenberghs</i> (Hasselt University and KU Leuven, Belgium)