



European Joint Program on Rare Diseases

Training Workshop

**Pluripotent stem cells for rare disease research:
banking, data, application**

**25 - 27 October 2021
Online**

As a part of the work in EJP RD Pillar 3: Capacity Building & Empowerment
Organised by project partners Fraunhofer Institute for Biomedical Engineering (Berlin, Germany) and Fondazione Telethon (Milan, Italy)

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Pluripotent stem cells for rare disease research: banking, data, application

25 - 27 October 2021, online training workshop (times in CEST)

25 October 2021 DAY 1

13:30 - 14:00	Online system, connection and sound testing for participants	
	Opening Session: Chair: Nancy Mah	
14:00 - 14:15	Welcome address	FH-IBMT - TBC
14:15 - 14:25	Introduction - Presentation of the EJP RD Project	Mary Wang Fondazione Telethon, Italy
14:25 - 14:45	Introduction to human pluripotent stem cells (hPSCs) How they are made, differences in iPSC vs ESC	Julia Neubauer FH-IBMT, Germany
14:45 - 15:00	Patient Perspective on biobanking and iPSCs	TBC
15:00 - 15:10	<i>Discussion, Q&A</i>	
15:10 - 15:20	Break	
	Session 1: Introduction to hPSC stem cells and banking Chair: Julia Neubauer, FH-IBMT	
15:20 - 15:40	The human pluripotent stem cell registry (hPSCreg)	Andreas Kurtz, FH-IBMT, Germany
15:40 - 16:00	The European Bank for induced pluripotent Stem Cells (EBiSC)	Sabine Müller, FH-IBMT, Germany
16:00 - 16:20	Reprogramming; best practices & protocols overview	Glyn Stacey, International Stem Cell Banking Initiative, UK
16:20 - 16:40	Applications of iPSC in RD research Researcher experience, JTC project, High Throughput Screening	Alessandro Prigione, Department of General Pediatrics, Neonatology, and Pediatric Cardiology, University Clinic Düsseldorf (UKD), Dusseldorf, Germany
16:40 - 17:00	<i>Discussion, Q&A</i>	



26 October 2021 DAY 2

10:00 – 10:10	Goals of Day 2	
	Session 2: Quality of biological samples: from source to end products Chair: JTC2020 Scientist, Ilaria Meloni	
10:10 – 10:30	iPSC quality control	Davide Cacchiarelli Telethon Institute of Genetics and Medicine – TIGEM, Italy
10:30 – 10:50	Cryopreservation of iPSCs	Ina Meiser, FH-IBMT, Germany
10:50 – 11:00	Discussion	
11:00 – 11:20	Gene editing approaches overview	TBC
11:20 – 11:40	Differentiation of hPSC lines General info; specific case – cardiac cells	Ralf Kettenhofen, FH-IBMT, Germany
11:40 – 12:00	<i>Discussion and Q&A</i>	
12:00 – 14:00	Lunch Break	
	Session 3: Quality of data: interoperability and access Chair: Andreas Kurtz	
14:00 – 14:20	Biosample data management: FAIR data	Pillar 2 FAIR Steward, TBC
14:20 – 14:40	Interoperability of biobank sample data (examples of a federated approach) Relevance to rare disease (RD connect) and other non rare disease-specific resources	Esther van Enckevort UMCG, Netherlands
14:40 – 15:00	Discussion	
15:00 – 15:20	Informed consent for RD research and GDPR: Practical lessons from ERNs	Annalisa Landi Benzi Foundation, Italy
15:20 – 15:40	Ethics, specific terms in the consent form: for hPSC	Rosario Isasi Dr. John T. Macdonald Foundation Department of Human Genetics, John P. Hussman Institute for Human Genomics, Interdisciplinary Stem Cell Institute, University of Miami, US
15:40 – 16:00	<i>Discussion and Q&A</i>	

27 October 2021 DAY 3

10:00 – 10:05	Goals of Day 3	
	Session 4: Future proofing of iPSCs biobanks Chair: Eva Bermejo, ISCIII	
10:05 – 10:20	iPSCs in translational research	TBC
10:20 – 10:40	ELSI issues (future proofing, handling unforeseen uses in consent) Dynamic consent, Broad consent	TBC
10:40 – 11:00	Sustainability of biobanks: accessibility and consent, including MTA and transfer of iPSC, freedom to operate	TBC



11:00 – 11:20	Perspective from Industry	TBC
11:20 – 11:40	<i>Discussion and Q&A</i>	
11:40 – 11:45	Closing remarks	

