Timetable of the **INFORMATION/NETWORKING EVENT** (January 25th 2021, 3pm – 6:30 pm):	
3:00 pm – 3:15 pm	*(10 min. presentation + 5 min. QA)* Presentation of the call on "Internal call for innovation project in clinical trials methodology" (use as possible reference for motivation: Rees, Plos Medicine 2019) • modalities and main goals
3:15 pm – 5:15 pm	*(10 min. per speaker)* Various perspectives of the remaining challenges that the present call will target. • <u>Registries:</u> A underused data source in RD (PAG? ERN?) • <u>Innovative methodologies:</u> what was achieved and how it can change the future of CT in limited population (C4C/Ideal) • <u>PCOMS</u> (patients centered outcome measures: primary or secondary end points (Zogenix, PAG, IRDIRC) • <u>Pharmacogenetic biomarkers and Pharmacokinetic:</u> How to develop Pharmacogenetic biomarkers in the future CT in RD and how to modelize differences between age groups in small population(TBD, CPATH?) • <u>EMA on the future of RD Trials:</u> should we innovate in the CT models (EMA representative) • <u>AREB:</u> How ethical guidance should be guiding the whole process (Viviana or another person from AREB) • <u>Gene therapy:</u> A model of innovating therapy in rare and ultra rare diseases (Stoke or Pfizer) *(30 min. discussion)*
5:15 pm - 5:30 pm	15 min. break
5:30 pm – 6:30 pm	*(60 min. in total; 2 to 3 min. per speaker; to be confirmed after registration)* Speed presentations of applicant ideas to initiate suitable partnerships between interested parties. (Clinical researchers could present their major gaps which are unsolvable by present methodologies.)

For the registration form:

- Name
- Institution
- Possible topic : Tick one of the pre-specified main topics. If "Other" leave the applicant space for 1-2 sentences to describe the topic.
- The registration will be closed on 18th of January 2021.
- Speed presentation session: Please tick, if you are willing to present your methodological problem related to a RD area during the "speed presentation" session in 2-3 min. using 2-3 slides. If the applicant clicks yes, the transparencies must be submitted to the call secretariat 3 days prior to event.
- Confirm that the organizer of the event are allowed to share your contact info with the other participants, so that partnerships between interested parties can be initiated.