

**CARE-RND 2024**  
**10-11 October 2024**



**Venue:** University Medical Centre Ljubljana,  
 Zaloška cesta 7, 1000 Ljubljana, Slovenia

**October 10<sup>th</sup> – Day 1**

Time	Topic	Speaker
<i>10:30 Welcome coffee</i>		
11:00 – 11:05	Welcome	Holm Graessner (University Hospital Tübingen)
11:05 – 11:30	Overview of ERN-RND	Holm Graessner (University Hospital Tübingen)
11:30 – 12:00	What is personalized care and how can we make it happen?	ERN-RND patient representatives
<b>Scientific symposium: Translation and Innovation</b>		
<b>Framework</b>		
12:00 – 12:30	European Partnership for Brain Health ( <i>online</i> )	Ulrike Busshoff (Coordination and support action BrainHealth) – CONFIRMED
12:30 – 13:00	Rare diseases regulation	European Medicine Agency ( <i>tbc</i> )
<i>Lunch (60 min)</i>		
<b>Resources</b>		
14:00 – 14:30	ERICA and EJP-RD Innovation Management Toolbox	Agustín Arasanz Duque (Vall d’Hebron Institute of Research) - CONFIRMED
14:30 – 15:00	The Orphan Drug Development Guidebook (ODDG)	Anneliene Jonker (IRDiRC) - CONFIRMED
15:00 – 15:30	Affordable medicine for rare diseases ( <i>online</i> )	Carla Hollak (Amsterdam UMC) – CONFIRMED
<i>Group photo and coffee break (45 min)</i>		
<b>RND use cases</b>		
16:15-16:45	Translational development of emrusolmin for the treatment of MSA	Johannes Levin (LMU Munich) – CONFIRMED
16:45-17:15	Guanabenz (repurposing small molecules) for vanishing white matter ( <i>online</i> )	Marjo van der Knaap (Amsterdam UMC) - CONFIRMED
17:15-17:45	SKYCLARYS, first therapy to treat Friedreich’s ataxia	Biogen - CONFIRMED
17:45-18:00	Wrap-up day 1	
18:00 – 18:30	Registry Data Access Committee meeting (only for DAC members)	
<i>19:30 Dinner</i>		

**October 11<sup>th</sup> – Day 2**

<b>Time</b>	<b>Topic</b>	<b>Speaker</b>
<b>Disease groups parallel session</b>		
08:00 – 09:30	3 DG/WG in parallel: <ul style="list-style-type: none"> <li>- Atypical Parkinsonian Syndromes DG</li> <li>- Leukodystrophies DG</li> <li>- Neurorehabilitation WG</li> </ul>	
09:30 – 11:00	3 DG/WG in parallel: <ul style="list-style-type: none"> <li>- Ataxia and HSP DG</li> <li>- FTD DG</li> <li>- Pediatric issues WG</li> </ul>	
<i>Coffee break (30 min)</i>		
11:30 – 13:00	2 DG/WG in parallel: <ul style="list-style-type: none"> <li>- Dystonia, paroxysmal disorders and NBIA DG</li> <li>- HD and chorea DG</li> </ul>	
<i>Lunch (60 min)</i>		
<b>DG and WG wrap-up on last years' activities and new developments</b>		
14:00 – 14:10	Dystonia, NBIA and paroxysmal disorders	
14:10 – 14:20	Ataxia and HSP	
14:20 – 14:30	FTD	
14:30 – 14:40	Leukodystrophies	
14:40 – 14:50	Choreas and Huntington disease	
14:50 – 15:00	Pediatric Issues	
15:00 – 15:10	Atypical parkinsonian syndromes	
15:10 – 15:30	Wrap-up day 2	
<i>Coffee break (20 min)</i>		
15:50 – 16:50	<b>Board Meeting</b>	Holm Graessner, University Hospital Tübingen
<i>End of the event</i>		