

## ERN-RND annual meeting on 17-19 June in Siena

### Agenda

#### 17 June – ERN-RND scientific symposium

13:00-14:00	Registration and welcome coffee	
	<b>Topic</b>	<b>Person</b>
14:00-14:30	Genetic therapy in SMA	Enrico Bertini (Rome)
14:30-14:50	Rare Diseases: Natural History Studies for Drug Development Guidance for Industry (FDA)	Baltazar Gomez Mancilla (Novartis)
14:50-15:10	SCA Global - trial readiness in RND	Thomas Klockgether (Bonn)
15:10-15:40	Coffee Break	
<b>Genetic therapy in RND</b>		
15:40-16:05	SCA3 and HD program of uniQure	Astrid Valles-Sanchez (uniQure)
16:05-16:30	HD	Bernhard Landwehrmeyer (Ulm)
16:30-16:55	FA	Massimo Pandolfo (Brussels)
16:55-17:20	Leukodystrophies	Caroline Sevin (Paris)
17:20-17:45	PSP (Tau as target)	Thomas Rösler (Munich)
17:45-18:10	MSA/GBA/genetic PD (Alpha synuclein as target)	Leonidas Stefanis (Athens)
18:10-18:30	Wrap up	Ludger Schöls (Tübingen)

19:00	<ROOM>	<b>Poster session with food and drinks</b>
19:00	<ROOM>	<b>ePAG meeting</b>
20:00	<ROOM>	<b>Dinner</b>

#### 18 June – ERN-RND annual meeting

08:00-8:20	<ROOM>	<b>ERN-RND overview</b> - Holm Graessner <ul style="list-style-type: none"> <li>• <i>DG and WG results</i></li> <li>• <i>Monitoring</i></li> <li>• <i>Aims of this meeting</i></li> </ul>
08:20-08:40		<b>CPMS live case discussion</b> – Judit Molnar
08:45-10:10	<ROOM>	<b>Results, work for year 3 and start of brain storming process for second 5 years</b> <b>Session 1:</b> <ul style="list-style-type: none"> <li>• <i>Care Coordination (Care need surveys – summary Marina de Koning-Tijssen)</i></li> <li>• <i>Diagnostic Pathways (EQA NGS pilot – Sophie Bernichtein, access to genetic testing in Europe - tbd)</i></li> <li>• <i>Registries and Research (ERN-RND registry – Ludger Schöls, Trial Readiness and registries workshop – Holm Graessner)</i></li> <li>• <i>Work plan</i></li> <li>• <i>Brain storming: success categories for ERN-RND</i></li> </ul> <b>Session 2:</b> <ul style="list-style-type: none"> <li>• <i>Training and Education (Winter schools, ERN-RND/Euro-NMD-EAN webinar series, fellow exchange programs – Judit Molnar)</i></li> <li>• <i>Guidelines and Pathways (Value of Treatment project – Paola Guinti,</i></li> </ul>



		<p><i>EAN RND guideline task force – Antonio Federico</i></p> <ul style="list-style-type: none"> <li>• <i>Information sharing and disease resources (Languages, ERN-RND standards for patients and non-experts – Alicia Brunelle)</i></li> <li>• <i>Work plan</i></li> <li>• <i>Brain storming: success categories for ERN-RND</i></li> </ul>
10:10-10:30	<ROOM>	Coffee Break / CPMS hands-on
10:30–12:00	<ROOM>	<p><b>Parallel disease group sessions:</b></p> <ul style="list-style-type: none"> <li>• <i>Frontotemporal Dementia</i></li> <li>• <i>Choreas and Huntington's Disease</i></li> </ul>
12:00-13:00	<ROOM>	Lunch
13:00-14:30	<ROOM>	<p><b>Parallel disease group sessions:</b></p> <ul style="list-style-type: none"> <li>• <i>Atypical Parkinsonian Syndromes</i></li> <li>• <i>Leukodystrophies</i></li> </ul>
14:30-15:00	<ROOM>	Coffee Break / CPMS hands-on
15:00-16:30	<ROOM>	<p><b>Parallel disease group sessions:</b></p> <ul style="list-style-type: none"> <li>• <i>Dystonias, paroxysmal disorders (non-epileptical ones), Neurodegeneration with Brain Iron Accumulation</i></li> <li>• <i>Cerebellar Ataxias and Spastic Paraplegias</i></li> </ul>
16:30 – 17:30		<b>Plenum session for reporting back from Disease and Working group sessions and decisions for next steps</b>
17:45		Sightseeing and Dinner in Siena

#### 19 June

09:00- 10:30	<ROOM>	<p><b>Board meeting</b></p> <ul style="list-style-type: none"> <li>• ePAGs feed-back</li> <li>• Feed-back from participants</li> <li>• Feed-back WP leads</li> <li>• European Joint Programming for Rare Diseases – Update</li> </ul>
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#### **Points to be included in all disease group sessions:**

- *Election of DG coordinators*
- *CPMS, Peer-experience*
- *DG-specific points*
- *New developments in patient care that should be considered within DG*
- *ePAG perspective*
- *Start of Brain Storming for second 5 years*