



# **European Reference Networks**

# Research Working Group (ERN RWG)

State-of-play





### **ERN RWG representation**

- a) All 24 ERNs are represented by 2 members each
- b) 2 Representatives of the ERN Board of Member States (France and Lithuania)

#### **ERN RWG state-of-play**

- RD-ACTION Workshop on ERNs & Research, held at EMA premises on 29-30 May 2018
- Workshop on 24 January 2019: brief presentation by Jonathan Olsson Consulting of the survey on ERN Research capabilities (Save-the-date, 1 place per ERN)







### **ERN RWG plans**

- Based on the results of the survey on the status of research of ERNs a more
  defined structure and objectives of ERN RWG
- Definition of sub-groups to target specific objectives, on specific topics of interests (some possible topics of interests Clinical Trials, Registries, Regulatory aspects- EMA, HTA)
- Organization of a meeting with the ERICs in the life science field (BBMRI;
  EATRIS; ECRIN; ELIXIR) to define collaboration with ERNs, inviting other research projects such Solve-RD, STARS etc.



### **ERN Research WG**





### Contact persons:

- On ERN side:
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  - Co-chairs:
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- Members on BoMS side: Birute Tumiene (LT)

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# **Update on EU-funded research**





## Clinical trials on rare diseases



Immunotherapy for multiple myeloma



2-Hydroxyoleic acid for treatment of malignant glioma



Gene Therapy for Crigler-Najjar syndrome



Disease modifying therapies for cystic fibrosis (ultra-rare CFTR mutations)



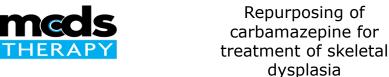
Stem-cell based gene therapy for recombination deficient SCID

OligoG Pivotal CF

Inhaled alginate oligosaccharide for cystic fibrosis



T-cell treatment for refractory viral infection post-HSCT



**UshTher** 

Gene therapy for retinitis pigmentosa in Usher syndrome

**TUDCA-ALS** 

Tauroursodeoxycholic acid as add-on treatment for ALS



New therapies for rare diseases (SC1-PM-08-2017)





# Solving the unsolved rare diseases

ERN-RND, ERN-ITHACA, ERN-Euro-NMD and ERN-GENTURIS form core ERNs, will reach out to all ERNs

More information available on Cordis Project Search: http://cordis.europa.eu/projects/home\_en.html

### **ImmunAid**

#### Immunome project for AutoInflammatory Disorders

Several recruiting centres are members of the ERN on Rare immunological and autoinflammatory diseases (RITA)



Diagnostic characterisation of rare diseases (SC1-PM-03-2017)



### Rare Disease European Joint Programme Cofund

Create research and innovation pipeline "from bench to bedside" ensuring rapid translation of research results into clinical applications and uptake in healthcare for the benefit of patients.

Implement joint programme of activities including research, coordination, networking, training, demonstration and dissemination:

- 1. Research and innovation programme to be funded through transnational calls
- 2. Virtual platform for RD information, research data, samples, tools and standards; pilots to ensure usefulness to be followed by upscaling in progressive manner
- 3. Capacity building include training and support activities to improve R&I potential
- 4. Strategic coordination and management through annual programming

Establish connections across RD community in particular with **ERNs** and patient organisations. Follow the policies and contribute to objectives of IRDiRC.

