29th Workshop of the EURORDIS Round Table of Companies

How to teach an old medicine new tricks

The importance of repurposing medicines for patients

Wednesday, 19 February 2020 (09:00 to 17:30)

The Crowne Plaza – Le Palace, Brussels

DRAFT PROGRAMME

Morning Session Co-Chairs: **Serge Braun,** PharmD, PhD, Scientific director, AFMTelethon	
09.00 - 09.20	**Welcome introduction, setting the scene & goals for the day** **Yann Le Cam,** Chief Executive Officer, EURORDIS-Rare Diseases Europe
09.20 – 09.40	**EU initiatives on timely access to innovative medicines** **Helen Lee,** Administrator, DG Health and Food Safety - European Commission
Stakeholders' interests for repurposed medicines: why do we need them and why is it difficult to obtain?	
09.50 – 10.05	**Professor Marc Dooms,** Leuven University Hospitals
10.05 – 10.20	**Ciska Verbaanderd,** PhD Student, Anticancer Fund
10.20 – 10.35	**Karine Proulx,** Drug and Discovery Alliance Manager, Healx
10.35 – 11.00	***Discussion***
11.00 – 11.30	*Coffee break*
The absence of a regulatory framework is one of the difficulties. How can the STAMP initiative help and how does it work?	
11.30 - 11.50	**The STAMP proposal/discussion**: the framework, how it works, pilots, RepOG **César Hernández García,** Head of the Department of Medicines for Human Use, AEMPS
11.55 – 13.00	**Facilitated panel discussion + Q&A from audience** Panellist representatives: **Christelle Bouygues,** Regulatory Affairs, European Medicines Agency **Sini Eskola,** Director, Team Leader Regulatory, Drug development and Manufacturing, EFPIA **Beate Stepniewska,** Deputy Director General & Head of Regulatory Affairs, Medicines for Europe **Professor Ranganath,** Royal Liverpool University Hospital, AKU Society Trustee
13.00 – 14.00	*Lunch*
Afternoon Session Co-Chairs: **Pablo Lapunzina,** Scientific Director, CIBERER	
How can we support academics and non-profits in re-purposing medicines? Incentives, intelligence, funding, etc.	

14.00 – 14.15	**Ferenc Marofka,** Policy Officer – Health Research & Innovation at European Commission
14.15 – 14.30	**Barbara Goodman,** President & COO, Cures Within Reach
14.30 – 15.00	**Q&A from audience**
15.00 – 15.20	***Coffee break***
What do you think of this initiative? Initial reactions and discussion	
15.20 – 16.20	**3 simultaneous Breakout Sessions, all sessions will address:** ✓ Initial reactions: given current legal framework, does scientific advice respond to industry's questions when deciding whether or not to repurpose a medicine? ✓ Are relations between champions, regulators and industry clear or are there aspects that require simplifying or alternatives? ✓ Pricing of repurposed medicines: how to keep prices reasonable / reactions/concerns/questions from industry
	Breakout session 1 • **Moderator: François Houÿez,** Treatment Information and Access Director, Health Policy Advisor, EURORDIS-Rare Diseases Europe • **Rapporteur: Russell Wheeler,** LHON Society
	Breakout session 2 • **Moderator: Simone Boselli,** Public Affairs Director, EURORDIS-Rare Diseases Europe • **Rapporteur: Vesna Aleksovska,** Chair & BoD of the International Gaucher Alliance, BoD of Macedonia National Alliance, Chairman of the association of citizens for rare diseases
	Breakout Session 3 • **Moderator: Elizabeth Vroom,** Founder & President of the Duchenne Parent Project Netherlands • **Rapporteur: Leonardo Scapozza,** Pharmaceutical Biochemistry/Chemistry, School of Pharmaceutical Sciences, University of Geneva
International Initiatives	
16.30 – 16.45	**Noel Southall,** IRDiRC
16.45 – 17.00	**Noel Southall,** National Centre for Advancing Translational Sciences, NIH
17.00 – 17.20	***Discussion***
17.20 – 17.30	**Toolkit overview: next steps on how to implement what you have heard today** **Concluding remarks** **François Houÿez,** Treatment Information and Access Director, Health Policy Advisor, EURORDIS-Rare Diseases Europe
17.30	***Meeting ends***