26th Workshop of the EURORDIS Round Table of Companies (ERTC)

Rare Disease Therapies: do we get what we incentivise?

Wednesday, 21 February, 2018 (09:30 to 17:00)
Hotel Le Plaza – Brussels, Belgium

PROGRAMME

Morning Session Co-Chaired by: **Avril Daly**, Vice-President, Board of Directors, EURORDIS-Rare Diseases Europe; Chief Executive Officer, Retina International **David King,** Co-Chair, EFPIA-EuropaBio Joint Task Force on Rare Diseases and Orphan Medicines; Director, Regulatory Policy and Intelligence, Shire	
Morning Session: ***Assessing the current incentives framework for rare disease therapies development***	
09:30 – 09:35	**Welcome & Introduction** **David King,** Co-Chair, EFPIA-EuropaBio Joint Task Force on Rare Diseases and Orphan Medicines; Director, Regulatory Policy and Intelligence, Shire
09:35 – 09:45	**Setting the scene & goals for the day** **Avril Daly**, Vice-President, Board of Directors, EURORDIS-Rare Diseases Europe; Chief Executive Officer, Retina International
09:45 – 10:00	**Assessing the tools in place to foster therapy development for rare diseases** **Kaja Kantorska**, Policy Officer, Unit B5 Medicines: policy, authorisation and monitoring, Directorate General Health and Consumers, European Commission
10:00 – 10:15	**The long road to therapy development: the key role of incentives** **Emmanuel Chantelot**, Chair, EUCOPE Working Group on Incentives; Executive Director, Head of Government Relations and Policy Europe, Celgene
10:15 – 10:30	**The patient's perspective: are we fulfilling real unmet needs?** **Julian Isla**, EURORDIS-Rare Diseases Europe; Founder and Chairman, Dravet Syndrome European Federation
10:30 – 11:15	**Cross-fire panel debate:** Moderator: **Yann Le Cam,** Chief Executive Officer, EURORDIS-Rare Diseases Europe **Panellists:**

	• **Kaja Kantorska**, Policy Officer, Unit B5 Medicines: policy, authorisation and monitoring, Directorate General Health and Consumers, European Commission • **Emmanuel Chantelot**, Chair of Therapies, EUCOPE Working Group on Incentives; Executive Director, Head of Government Relations and Policy Europe, Celgene • **Julian Isla**, Dravet Syndrome European Federation • **Michela Gabaldo,** Head Alliance & Regulatory Affairs, Fondazione Telethon • **Stephen Moran,** Global Head of Strategy, Novartis
11:15 – 11:20	**Introduction to the breakout sessions** Morning Chairpersons
11:20 – 11:50	Coffee break
11:50 – 13.30	**<u>Breakout session 1</u>: Repurposing of existing therapies to fulfil rare disease needs: what framework is needed?** Moderator: **Daniel O'Connor**, Medical Assessor at the Medicines and Healthcare Products Regulatory Agency (MHRA) Rapporteur: **Diego Ardigo**, Chair Therapies Scientific Committee of IRDiRC; Project Lead, Chiesi
	<u>Breakout session 2:</u> Is R&D sufficiently incentivised to address real unmet medical needs (orphan and paediatrics)? Moderator: **Fabio D'Atri**, Policy Officer, Unit B5 Medicines: policy, authorisation and monitoring, Directorate General for Health and Consumers, European Commission Rapporteur: **Chay Morgan,** Head of Europe/MEA/CIS Regulatory Affairs, BioMarin
	<u>Breakout session 3</u>: Are we backing up advanced therapies enough? Moderator: **Rocio Salvador Roldan**, Policy Officer, U nit B5 Medicines: policy, authorisation and monitoring, Directorate General Health and Consumers, European Commission Rapporteur: **Tresja Bolt**, Head of Public Affairs, Europe, Bluebird Bio
13:30 - 14:30	Lunch

Afternoon Session Co-Chaired by:

Lieven Bauwens, EURORDIS-Rare Diseases Europe, Board of Directors; Secretary General of the International Spina Bifida Federation

Marlene Haffner, Former Director of the Office of Orphan Products Development at the Food and Drug Administration (FDA)

Afternoon Session:

Ensuring the right ecosystem for rare disease therapies development

14:30 – 15:30	**Feedback from breakout sessions with panel of rapporteurs** Moderated by **Marlene Haffner**, Former Director of the Office of Orphan Products Development at the Food and Drug Administration (FDA) 10' each + 30' Q&A Rapporteurs: Breakout session 1: **Diego Ardigo**, Chair Therapies Scientific Committee of IRDiRC; Project Lead, Chiesi Breakout session 2: **Chay Morgan,** Head of Europe/MEA/CIS Regulatory Affairs, BioMarin Breakout session 3: **Tresja Bolt**, Head of Public Affairs, Europe, Bluebird Bio Moderators to join for 30' Q&A
15:30 – 16:35	**The quest for effective incentives for rare disease therapies development - a global outlook** **Moderator: Lieven Bauwens**, EURORDIS-Rare Diseases Europe, Board of Directors; Secretary General of the International Spina Bifida Federation **Keynote speech (setting the scene)** • **Sarah Garner**, Co-ordinator Innovation, Access and Use, Essential Medicines and Health Products, World Health Organization **Panel discussion between panellists:** • **François Houyez,** Treatment Information and Access Director, Health Policy Advisor, EURORDIS-Rare Diseases Europe • **Anthony Humphreys**, Head of Sector Regulatory Affairs Committee Support and Community Procedures, European Medicines Agency • **Sarah Garner**, Co-ordinator Innovation, Access and Use, Essential Medicines and Health Products, World Health Organization • **Frans De Loos**, Director, Foundation Fair Medicine
16:35 – 17:00	**Take-home messages & concluding remarks** • **Martin Seychell**, Deputy Director General, Directorate-General for Health and Food Safety, European Commission • **Nathalie Moll**, Director General, EFPIA • **Yann Le Cam**, Chief Executive Officer, EURORDIS Rare Diseases Europe
17:00	Meeting ends