



## 30th Workshop of the EURORDIS Round Table of Companies (ERTC)

# Newborn Screening: Shaping the future

15-16 October 2020 ONLINE

### FINAL PROGRAMME

<p><b>Thursday 15<sup>th</sup> OCTOBER</b>  <b>13:00-18:00</b>  <b>Session chaired by:</b>  <b>Dr. Gulcin Gumus</b>, Research and Policy Project Manager, EURORDIS-Rare Diseases Europe</p>	
<p><b>Understanding the landscape and diverse national approaches</b></p>	
13:00 – 13:10	<p><b>Welcome introduction, setting the scene &amp; goals for the workshop</b>  <b>Yann Le Cam</b>, Chief Executive Officer, EURORDIS-Rare Diseases Europe</p>
13:10-13:15	<p><b>Programme of the day</b>  <b>Dr. Gulcin Gumus</b>, Research and Policy Project Manager, EURORDIS-Rare Diseases Europe</p>
13:15 – 13:30	<p><b>Newborn Screening Landscape</b>  EU perspective- <b>Dr. Martina Cornel</b>, Professor of Community Genetics &amp; Public Health Genomics, Amsterdam University Medical Center</p>
13:30 – 15:15	<p><b>Session 1: Learning from experiences and pilots (Comfort break after 45 min)</b>  Panel discussion with representatives from different stakeholder groups in newborn screening.</p> <ul style="list-style-type: none"> <li>• UK Newborn Screening programme – <b>Sara Hunt</b>, Chief Executive Officer, Alex, The Leukodystrophy Charity</li> <li>• Italian Extended Newborn Screening programme – <b>Prof. Giancarlo La Marca</b>, Head, Newborn Screening, Clinical Chemistry and Pharmacology Lab, Meyer Children's Hospital</li> <li>• French Newborn Screening programme - <b>Marie-Christine Ouillade</b>, President, SMA NBS Alliance</li> <li>• World Duchenne Organisation Newborn Screening programme: Wales, Antwerp, Australia, US – <b>Elizabeth Vroom</b>, Chair and Co-Founder, World Duchenne Organisation, EURORDIS Board of Directors</li> <li>• SMA newborn screening: perspectives and learnings from pilots - <b>Wildon Farwell</b>, Global Medical Head of Neuromuscular Diseases, Biogen</li> <li>• <b>Q&amp;A</b></li> </ul>
15:15 – 15 :30	<i>Break</i>
<p><b>Ethical, social and economic ramifications</b></p>	



15:30 – 16:30	<b>Session 2: Ethical, social and economic considerations</b> <ul style="list-style-type: none"> <li>• <b>Ethical &amp; Social implications of Newborn Screening (15 min)</b> Prof. Pascal Borry, Interfaculty Centre for Biomedical Ethics and Law, University of Leuven</li> <li>• <b>Economic implications of Newborn Screening (15 min)</b> Prof. Jim Chilcott, Professor of Healthcare Decision Modelling, School of Health and Related Research, University of Sheffield</li> <li>• <b>Q&amp;A</b></li> </ul>
16:30 – 18:00	<b>Meet the participants</b> (Online facilitated networking session)

## Friday 16<sup>th</sup> OCTOBER

**13:00-17:00**

Session chaired by:

Dr. Virginie Bros Facer, Scientific Director, EURORDIS-Rare Diseases Europe

### Refining the Newborn Screening Position Statements

13:00 – 15:40	<p><b>Workshop session 1: Newborn Screening as a whole system, not just a test (Comfort Break 14:30-14:45)</b></p> <p>Introduction on Newborn Screening- Working Group &amp; EURORDIS Draft position statements (10 minute video) – Dr. Gulcin Gumus, Research and Policy Project Manager, EURORDIS-Rare Diseases Europe</p> <p><b>Moderator: Nick Meade</b>, Director of Policy, Genetic Alliance UK</p> <p><b>Rapporteur: Sarah Creviston</b>, Global Head, Patient Advocacy and Government Affairs, Novartis Gene Therapies</p> <hr/> <p><b>Workshop session 2: Empowerment &amp; follow up support (Comfort Break 14:30-14:45)</b></p> <p>Introduction on Newborn Screening- Working Group &amp; EURORDIS Draft position statements (10 minute video) – Dr. Gulcin Gumus, Research and Policy Project Manager, EURORDIS-Rare Diseases Europe</p> <p><b>Moderator: Simona Bellagambi</b>, UNIAMO, EURORDIS Board of Directors</p> <p><b>Rapporteur: Charlotte Chanson</b>, Director, Diagnostics EMEA, Orchard Therapeutics</p> <hr/> <p><b>Workshop session 3: From shaping the future to concrete implementation (Comfort Break 14:30-14:45)</b></p> <p>Introduction on Newborn Screening- Working Group &amp; EURORDIS Draft position statements (10 minute video) – Dr. Gulcin Gumus, Research and Policy Project Manager, EURORDIS-Rare Diseases Europe</p> <p><b>Moderator: Valentina Bottarelli</b>, Public Affairs Director, Head of European and International Advocacy, EURORDIS</p> <p><b>Rapporteur: Gabriella Pasciullo</b>, European Regional Medical Lead, Bluebird Bio</p>
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	<p><b>Workshop session 4: Maximising the potential (Comfort Break 14:30-14:45)</b></p> <p>Introduction on Newborn Screening- Working Group &amp; EURORDIS Draft position statements (10 minute video) – <b>Dr. Gulcin Gumus</b>, Research and Policy Project Manager, EURORDIS-Rare Diseases Europe</p> <p><b>Moderator: Dr. Cor Oosterwijk</b>, Director, VSOP – Dutch National Patient Alliance for Rare and Genetic Diseases</p> <p><b>Rapporteur: Shirlene Badger</b>, Patient Advocacy Manager, Illumina Cambridge Ltd.</p>
15:40-16:00	<b>Break</b>
16:00 – 16:45	<p><b>Feedback from breakout sessions</b></p> <p><b>Moderator: Dr. Virginie Bros Facer</b>, Scientific Director, EURORDIS-Rare Diseases Europe</p> <p><b>Rapporteurs:</b> Refer to breakout sessions above</p>
16:45 – 16:50	<p><b>Key take-home messages:</b> The importance for industry to support the advocacy of Newborn Screening</p> <p><b>Sian O'Neill</b>, Senior Director, Patient Engagement EMEA, PTC Therapeutics</p>
16:50 – 17:00	<p><b>Concluding remarks</b></p> <p><b>Yann Le Cam</b>, EURORDIS-Rare Diseases Europe, Chief Executive Officer</p>
17:00	<b>Meeting ends</b>