7 September 2021

Dr. Andrzej Rys
Director, Health Systems, Medical Products and Innovation
European Commission Directorate-General for Health and Food Safety (DG SANTE)
1049 Brussels
Belgium

Re: ERN-Cranio; Clinical Practice Guidelines for Pierre Robin Sequence

Dear Dr. Rys,

1. Two weeks ago we pleaded with you to take action on the time sensitive issues we had raised earlier in the summer in our 30 June 2021 letter to the European Commission concerning ERN-Cranio and Erasmus Medical Center in Rotterdam. We do not know what if anything you have done to address the situation, but as a result of the European Commission's silence and inaction, we at Stichting Pierre Robin Europe implored Amsterdam Medical Center's Professor Corstiaan Breugem, and Tübingen University Hospital's Professor Christian Poets, to urgently review the draft EU Clinical Practice Guidelines which ERN-Cranio and Erasmus Rotterdam produced without them.

2. We just received feedback from Professor Poets, which we have attached: 17 pages of detailed feedback from this top EU Pierre Robin Sequence expert exposing serious flaws, medical inaccuracies, misinterpreted research, unsupported conclusions, overlooked medical studies, and professional bias, in ERN-Cranio's EU Clinical Practice Guidelines.

3. The substandard material which ERN-Cranio and Erasmus Rotterdam produced for EU babies suffering from this life-threatening rare disease is not at all surprising. On the contrary, this was predictable, and it is exactly what we repeatedly warned you about Dr. Rys. This is what happens, this is the inevitable result, when an extremely sensitive EU-funded ERN rare disease Clinical Practice Guideline project is carried out without the top EU experts in the Guideline Development Group. Any outside observer would have no choice but to conclude that Erasmus Rotterdam ran this EU-funded ERN Clinical Practice Guideline development project based on relationships and politics, rather than on experience and expertise.

4. It is unmistakably clear that this ERN Clinical Practice Guideline project for Pierre Robin Sequence must now be restarted from the very beginning. This EU-funded initiative must be relaunched and carried out as it should be carried out, in accordance with the EU rules, the official EU ERN Methodology – which the EU paid for, and distributed, to every single ERN in the entire ERN system. As ERN-Ithaca rightfully points out, the EU Methodology:

> "is an important step forward of the ERN CPG programme and a key element and tool for the work of the ERNs in the production, adaptation, adoption and appraisal of the different guidelines and technical sub-products, to be used for now on by all the ERNs. The methodology will ensure the quality and standardisation of the CPG and CDST produced and used by the ERNs, the backbone of the process of the diagnosis and treatment of patients suffering of rare or low prevalence and complex diseases."
> https://ern-ithaca.eu/handbook-and-toolkit-of-the-erns-clinical-practice-guideline-programme/

If ERN-Cranio's gross mismanagement of this EU-funded project and defiant rejection of the EU Methodology is not thoroughly corrected, then the EU's ERN system will face a

devastating loss of international credibility. The European Commission's Directorate-General for Health and Food Safety (DG SANTE) will be held accountable, not only for the failures, but also for the misspent EU funds. We ask you Dr. Rys to kindly contact us to discuss this matter at your earliest convenience, for the benefit of all parties concerned. Thank you.

Kind regards,

Philippe Pakter
Stichting Pierre Robin Europe, voorzitter
Member, EURORDIS, The European Organisation for Rare Diseases
Member, VSOP, Vereniging Samenwerkende Ouder- en Patiëntenorganisaties, The Dutch Patient Alliance for Rare and Genetic Diseases
PhD candidate, law: "Access to healthcare in Europe: the effectiveness of EU legislation in the context of rare disease patients"