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EURORDIS HEALTHCARE WEBINAR SERIES

Approaches to Evaluate Evidence on Rare Disease Guidelines

29 April 2024 at 14.00-15.30 CET

Agenda

Please use this link to register:

https://uso2web.zoom.us/meeting/register/tZEvcemrrTwsHgcs-6bTdkBmqeSqzA8wjfV4

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Wednesday, 29 April 2024 at 14.30-16.00 CET

For many rare and complex conditions, the volume of peer-reviewed evidence available to consider for this guideline is and always will be small, which is the result of small affected populations. The volume of evidence is also unlikely to ever reach even a fraction of that for a more common disease. This creates a difficulty when considering the grading of the strength of evidence. If the evidence for rare and complex conditions is categorised and then graded using standard approaches, then the small volume of papers means it would be graded as weak. This is not an accurate reflection of the combination of the expert's experience and clinical consensus with the available evidence. This is further compounded by the low likelihood of additional volumes of evidence that could change the recommendation. In addition, there are other challenges in using standard approaches to evaluate evidence for rare disease guidelines, specifically the questions including in the scope of a rare disease guideline does not easily 'fit' into a PICO approach. This can result in many rare disease guidelines having gaps in recommendations which does not support clinical decision making to address the complex and multi-system needs which people living with a rare disease present with.

It is well acknowledged that a different approach is needed to evaluate the evidence for new Orphan Medicines compared to standard medicine and vaccines, due to the challenges of small populations that are affected by rare diseases. However, in guideline development one model for the evaluation of evidence is held as good practice up as best practice and that is using GRADE.

How can we benefit from all the different types of evidence and review this systematically? How can we take all evidence for a rare and complex condition to inform robust decisions and recommendations, that is the best that can be made within the timeframe for any possible of addition evidence to be published? This webinar brings together experts and methodologists experienced to successfully navigate the challenges of evaluating the evidence for small populations to agree on the underlying issues and, drawing on their real world experience, proposing solutions to developing effective guidelines for rare diseases.

The outcome of the webinar will be a prospective article that presents both the challenges and solutions to evaluating the evidence for rare and complex conditions guidelines.

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Agenda

Time	Topics	Speaker
14.30 – 14.35	Welcome, introductions and setting the scene	• Matt Bolz-Johnson, Healthcare Advisor, EURORDIS
14.35 – 15.00	Part 1: Experiences of the challenges in synthesis high quality evidence gathered in rare and complex conditions Presentations (5 mins each): 1. European Urology Association 2. ERN ERNICA 3. ERN GENTURIS 4. ERN ITHACA	• Imran Omar, EAU • Willemijn Irvine & Iris den Uijl, ERN ERNICA • Tom Kenny & Manon Engels, ERN GENTURIS • Agnies van Eeghen & Charlotte Gaasterland, ERN ITHACA
15.00 – 15.15	Questions & Answers	All
15.15 – 15.40	Part 2: Approaches taken and potential solutions Presentations (5 mins each): 1. European Urology Association 2. ERN ERNICA 3. ERN GENTURIS 4. ERN ITHACA	• Imran Omar, EAU • Willemijn Irvine & Iris den Uijl, ERN ERNICA • Tom Kenny & Manon Engels, ERN GENTURIS • Agnies van Eeghen & Charlotte Gaasterland, ERN ITHACA
15.40 – 15.55	Questions & Answers	All
15.55 – 16.00	Main Conclusions & Next Steps	• Matt Bolz-Johnson, Healthcare Advisor, EURORDIS