



IPWSO
International
Prader-Willi Syndrome
Organisation

IPWSO Leadership ECHO summary

February 8, 2022: The Future of Research in PWS

“Every presentation that I watched so far in ECHO really informed me more about Prader-Willi syndrome. And I thought that as we had the challenges that kind of connected us, and it enabled us to share information that otherwise we wouldn't have shared with each other.”

~Leadership ECHO participant

Our last planned IPWSO ECHO of the 2021/2022 Leadership Programme took place on Tuesday February 8th, 2022. In this session Maithé Tauber Professor of Pediatrics, University of Toulouse, France presented *“New Data in PWS and Perspectives”*.

Maithé first gave a brief summary of previously known data, then went on to present new data of her research centre in Toulouse. They are understanding more and more the various genes that are responsible for the features of PWS.

Her presentation included an Overview of PWS; Mental Health; Endocrine and Metabolic Troubles; Scoliosis, COVID 19; and Therapeutic Perspectives.

In the presentation, Maithé outlined a definition of hyperphagia then went on to demonstrate that we can consider hyperphagia to be an addictive disorder, giving examples of commonalities with other addictive disorders. This was a topic for group discussion, with some finding the addiction model helpful and wondered if the addiction approach may be used by psychologists as a method of management, while others said we all need food, and the issue with hyperphagia is that inability to switch off the desire to eat.

“Don't be shy to talk about PWS! It can lead to great conversations.”

We hope you enjoyed the presentation; if you would like to revisit it or if you weren't able to join us on the day, here is the video link so you can view in your own time.

Maithé Tauber: [New Data in PWS and Perspectives](#)

The PDF is available [here](#).

We also heard from Nathalie Kayadjanian, Independent Consultant and Expert in Translational Biomedical Research for Rare Diseases, who sparked discussion on how advances in research are then disseminated. Such as, once a drug is approved, what next? What is the process to patient access to new drugs? What are the barriers to be overcome? And especially, what is/could be the role of national associations and families in that?

Drug regulatory approval is really just the beginning when bringing a drug to the market. Next comes the Post Market Period.

Nathalie outlined the three post approval steps to market:

1. Post Marketing Surveillance. More detailed observation of the drug ethics in real market setting is closely monitored. This process can lead to changes or even drug withdrawal.
2. Drug Price and Reimbursement. One challenge for new drug development is that there is often no correlation between the trial cost and final price. A Health Technology Assessment usually plays a role in assessing the final price. This assessment can be clinical, economic and budgetary. Ideally there will be an evidence-based cost versus benefit analysis. The outcome varies considerably from country to country.
3. Developing access to the new drugs. There can be a regulatory delay between initial approval and user access.

There can be a lack of transparency in the process. Patients are often involved, but often only in an advisory capacity. Nathalie posed the question, "How can patients be more involved in the Post Market Process?"

The group discussion related to whether data could be collected to, for example, understand the cost to community or more importantly government, of hyperphagia so that cost benefit could be more accurately highlighted once hyperphagia drugs reach the market.

Thank you very much to everyone who attended the ECHO sessions and participated. IPWSO hopes to announce new ECHO programmes later in 2022 as they are decided.

Ends.