The Symptoms, Challenges, and Issues of Prader-Willi Syndrome: The Development of a Conceptual Model

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▶ Background

Prader-Willi Syndrome (PWS) affects 1 in 25,000 individuals[1] and there is no treatment[2] except for one approved drug by the Food and Drug Administration (FDA). This therapy only partially addresses some symptoms of the disease, leaving many others such as constant hunger (hyperphagia)[3], repetitive questioning, resistance to change, and hoarding[1,4,5] unaddressed.

Many pharmaceutical companies have clinical development programs that hold promise. However, the limitations in clinical outcomes assessments to capture meaningful change pose a significant barrier to their success.

▶ Purpose

Here, we aim to improve the success of these programs by filling the gap between the patient/caregiver experience and the medical model of the disease.

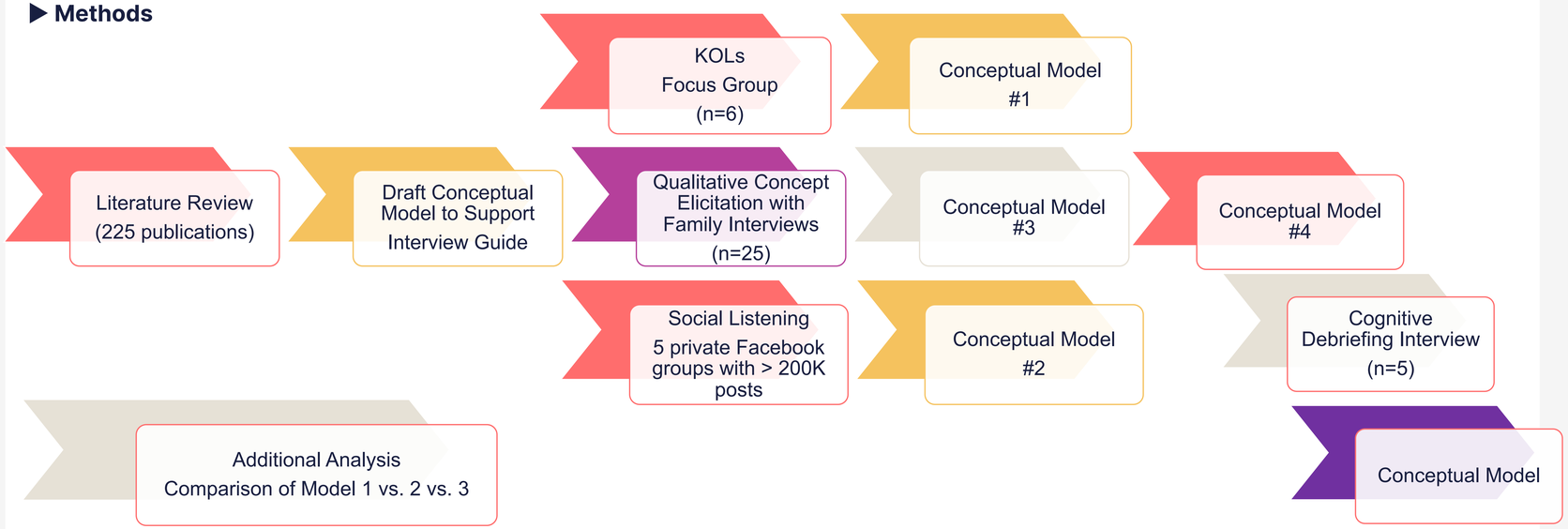
To achieve this, we designed a study to develop a conceptual model of PWS using:

1-) social listening, a novel method that leverages anonymized real-world conversations in social media,

2-) input from expert clinicians through group interviews, and

3-) one-on-one interviews with persons living with PWS and their caregivers.

▶ Methods



▶ Methods

We first developed a draft model based on a non-systematic literature review (n= 225 publications).

This draft model informed the interview guides for the qualitative concept elicitation interviews and will be updated after the interviews with caregivers and people with PWS (n=25).

This model will be updated with input from clinician experts (n=6) and social listening data derived from 5 private Facebook groups, including more than 200,000 posts/comments.

Finally, cognitive debriefing interviews with people living with PWS and their caregivers (n=5) will assess and improve the validity of the combined model.

▶ Results

Critically, we will compare and contrast draft conceptual models developed based on three different data sources (social listening vs. patient/caregiver vs. expert clinicians) and assess the strengths and weaknesses of various methods utilized in developing conceptual models.

This study will result in a conceptual model of PWS that accurately reflects patient/caregiver experiences.

It will act as a proof-of-concept study to introduce a novel method (social listening) in conceptual model development, and eventually will help guide future conceptual model development strategies.

▶ Conclusions

Novel methodologies utilized in this research study may enhance the accuracy of the conceptual models and increase their feasibility.

The developed conceptual model will enhance the development and adaptation of novel clinical outcome assessments such as goal attainment scaling - a personalized outcome assessment that quantifies the impact of an intervention on individualized goals, and ultimately improve the success of clinical development programs.

▶ Acknowledgements & References

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Presenting Author: Maurice Flurie

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Background: Prader-Willi Syndrome (PWS) affects 1 in 25,000 individuals[1] and there is no treatment[2] except for one approved drug by the Food and Drug Administration (FDA) that only partially addresses some symptoms of the disease, leaving many others such as constant hunger (hyperphagia)[3], repetitive questioning, resistance to change, and hoarding[1,4,5] unaddressed. Many pharmaceutical companies have clinical development programs which hold promise, however the limitations in clinical outcomes assessments to capture meaningful change poses a significant barrier to their success.

Purpose: Here, we aim to improve the success of these programs by filling the gap between patient/caregiver experience and medical model of the disease. To achieve this, we designed a study to develop a conceptual model of PWS using 1-) social listening, a novel method that leverages anonymized real-world conversations in social media, 2-) input from expert clinicians through group interviews, and 3-) one-on-one interviews with persons living with PWS and their caregivers.

Methods: We first developed a draft model based on a non-systematic literature review (n= 225 publications). This draft model informed the interview guides for the qualitative concept elicitation interviews and will be updated after the interviews with caregivers and people with PWS (n=25). This model will then be combined with input from clinician experts (n=6), and social listening data derived from 5 private Facebook groups including more than 200,000 posts/comments. Finally cognitive debriefing interviews with people with PWS and their caregivers (n=5) will assess and improve validity of the combined model. Critically, we will compare, and contrast draft conceptual models developed based on three different data sources (social listening vs. patient/caregiver vs. expert clinicians) and assess the strengths and weaknesses of various methods utilized in the development of conceptual models.

Results: This study will result in a conceptual model of PWS that accurately reflects patient/caregiver experiences. It will act as a proof-of-concept study to introduce a novel method (social listening) in conceptual model development; and eventually will help guide future conceptual model development strategies.

Conclusions: Novel methodologies utilized in this research study may enhance the accuracy of the conceptual models; and increase their feasibility. The developed conceptual model will enhance the development and adaptation of novel clinical outcome assessments such as goal attainment scaling - a personalized outcome assessment that quantifies the impact of an intervention on individualized goals.; and ultimately improve the success of clinical development programs.

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