Advocating for New Treatments for Prader-Willi Syndrome
Carbetocin (LV101): New Drug Application (NDA) Journey

Carbetocin Phase 3 Clinical Trial

Sponsor submits NDA

FDA files & reviews the application

FDA actively reviews data and works with Sponsor over a 6-month (Priority Review)

OPTIONAL
FDA holds advisory committee meeting

A public hearing will be held Nov 4, 2021, seeking advice regarding the approvability of carbetocin

Sponsor opens dialog with FDA

FDA conducted a high-level review of data to determine sufficiency to warrant a full review

FDA issues a Complete Response Letter

FDA approves the application

Members of the PWS community can provide written or oral testimony.
21st Century Cures Act Mandates the Consideration of the Patient Voice:

*Patient Focused Drug Development*
What is an Advisory Committee?

The FDA may choose to hold an Advisory Committee meeting when reviewing a new drug application.

Not all drug reviews have an advisory committee meeting!

This meeting is taking place because the FDA has questions regarding the application.
A committee normally consists of 9 “standing” members including the chairperson.

- Statistician
- Consumer Rep
- Industry Rep
- Psychiatrists
- Endocrinologist
- Patient Rep
What might the meeting agenda look like??

Call to order and introduction of the committee
Recitation of the conflicts of interest statement (this is required by the Federal Advisory Committee Act)
Brief opening remarks from FDA
Sponsor presentation
Clarifying questions from the committee to the sponsor
FDA presentation
Clarifying questions from the committee to FDA
Open public hearing (OPH)
Committee charge (where FDA explicitly recites the questions asked of the committee)
Discussion by the committee (almost always includes questions to FDA and the sponsor)
Committee answering the questions and/or voting and explaining their answers/vote
Adjournment of the meeting
### FDA Benefit-Risk Framework

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How to Make an Impact – Points for Comments

• Unmet need: PWS has a tremendous impact on the person with PWS and their family. It is a serious, life-threatening disorder and currently, we have NOTHING to address the greatest challenges of PWS

• What would improvement mean to you? Modest improvements in key behaviors (hyperphagia, anxiety) will have a meaningful impact on the ability of the person with PWS to ...

• Risk tolerance - Our kids live with risk every day – families struggle to manage behaviors using medications that have NOT been tested in the PWS population.

• Willingness to accept uncertainty of BENEFIT. We expect that not every drug will help EVERY person with PWS. Carbetocin has a favorable safety profile, and we'd like to be able to work with our doctors to see how it works in our children.

• Additional trials are not realistic. The challenges of PWS, as well as our small patient population, makes participating in and completing a clinical trial incredibly difficult.
Carbetocin Study Participants May Want to Elaborate on One or More of the Following:

- How has carbetocin changed your child’s relationship with food? Describe what your child’s food interest and hunger looked like before treatment. Could they delay meals or snacks, could they be a part of events that food was available? Did they talk or ask about food? How is it different now with treatment?

- Describe your child’s anxiousness before beginning treatment. What did it look like? Tearful, angry, irritable, aggressive? What triggered it? How are things different now with treatment?

- Was your child rigid prior to beginning treatment? What about now?

- What is the most important way carbetocin has changed in your life? In your child’s?
How To Submit Your Written Comments

• Visit: https://www.regulations.gov/commenton/FDA-2021-N-0860-0001

• For the best readability, submit comments by uploading word or PDF document, rather than submitting through the text field.

• ‘What is your comment about?’ - select none of the above or simply leave blank)

• ‘Tell us about yourself, I am....’ – select ‘individual’ and complete form

• NOTE: If you are requesting speaking time, do not submit written comments until AFTER oral comments close on October 13th!
Dates to Remember

13 Oct.
Deadline to submit oral comments (recommended only for trial participants)

21 Oct.
Deadline to submit written comments to Advisory Committee

3 Nov.
Deadline to submit electronic comments for FDA only

4 Nov.
Advisory Committee Meeting – Open to Public!!
10 am – 4pm ET
Ready to submit your comment? Submit your written comments here.

Comments for the committee must be submitted by Oct 21.

To attend the meeting and download meeting materials, click here.

Items will be available at least 2 days prior to the event.

Looking for feedback or a second set of eyes for your comments?

Email Susan.Hedstrom@fpwr.org or Privard@pwsusa.org