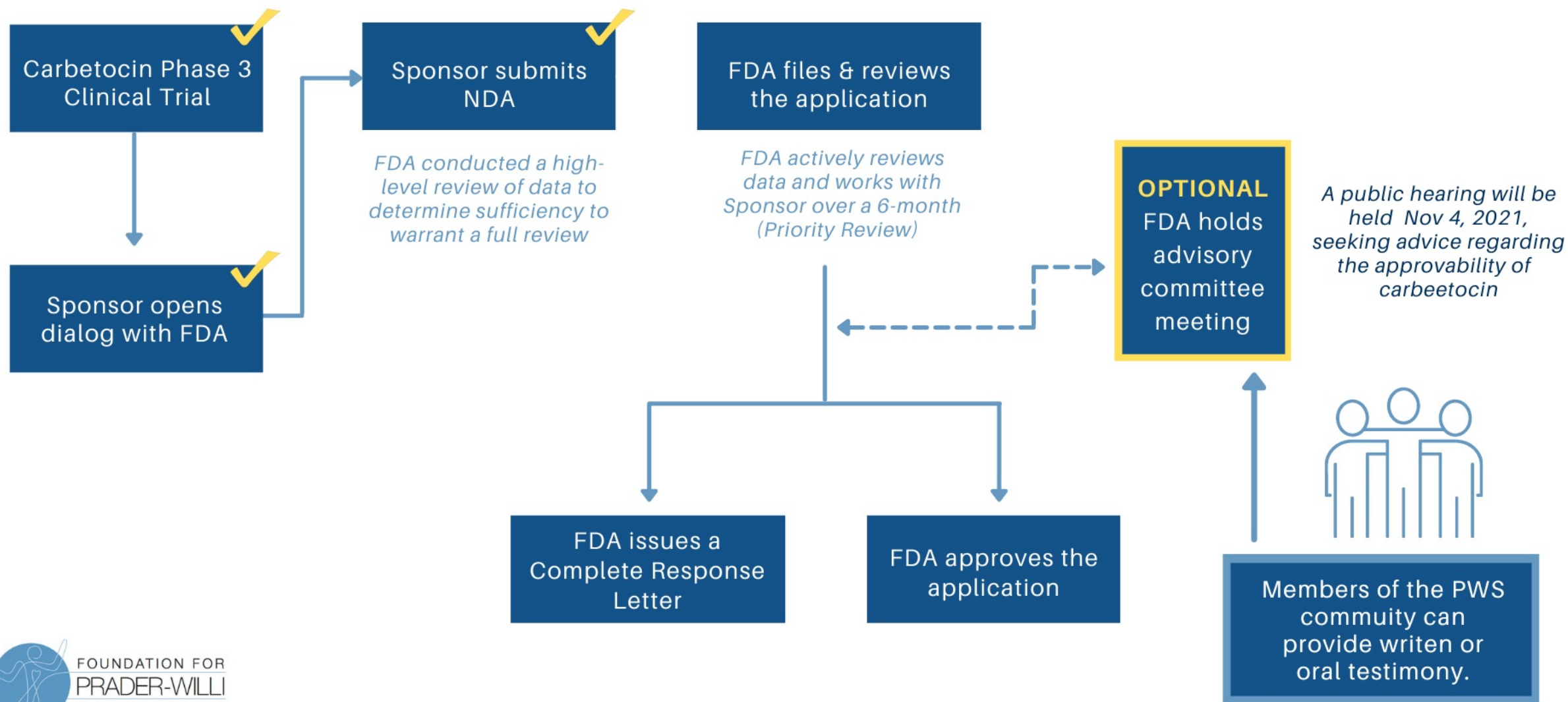


Advocating for New Treatments

for Prader-Willi Syndrome



Carbetocin (LV101): New Drug Application (NDA) Journey



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.

Who is on the Advisory Committee?



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??

FOOD AND DRUG ADMINISTRATION (FDA)
Center for Drug Evaluation and Research (CDER)

LEVO Carbetocin (LV101)

November 4th, 2021, 10:00 am - 4:00 pm

Open Public Hearing from 1:30 - 2:30 pm

SAMPLE AGENDA

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)

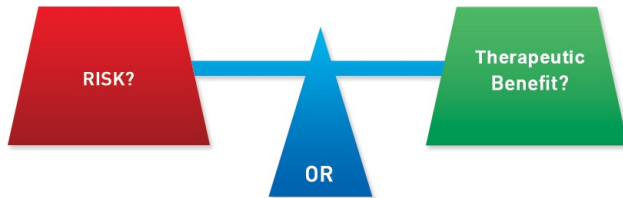
Committee charge (where FDA explicitly recites the questions asked of the committee)

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



Dimension	Evidence and Uncertainties	Conclusions and Reasons
Analysis of Condition		
Current Treatment Options		
Benefit		
Risk and Risk Management		

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!

Regulations.gov
Your Voice in Federal Decision Making

SUPPORT

You are commenting on a Notice by the Food and Drug Administration
Psychopharmacologic Drugs Advisory Committee; Notice of Meeting; Establishment of a Public Docket; Request for Comments

Comment Period Ends: 44 Days

Write a Comment

Commenter's Checklist

Comment*

Start typing comment here...

5000

What is your comment about?

Select a Comment Category

Attach Files

You can attach up to 20 files, but each file cannot exceed 10MB. Valid file types include: bmp, docx, gif, jpeg, pdf, png, pptx, rtf, sgml, tif, tiff, txt, wpd, xlsx, xml.

Drop files here or [Browse...](#)


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Tell us about yourself! I am...*

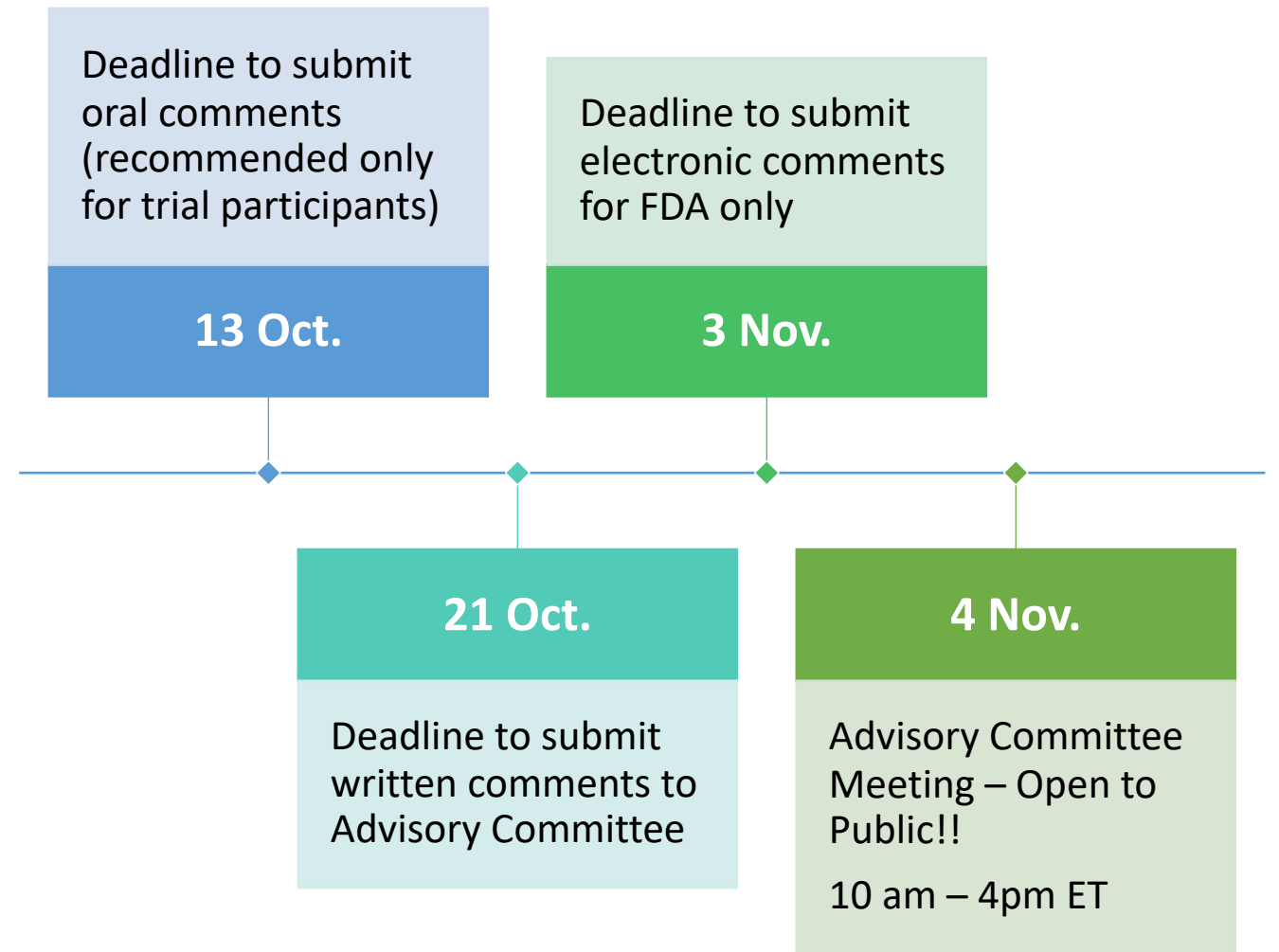
(Select an identity below)


An Individual


An Organization


Anonymous

Dates to Remember



Links and Resources

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@pwsausa.org