



# Examining Peripheral Edema in People With Prader-Willi Syndrome Who Took Diazoxide Choline Extended-Release Tablets Long Term

## What is Prader-Willi syndrome?

- Prader-Willi syndrome (**PWS**) is a rare disease that is found in about 1 out of every 15,000 babies born in the United States<sup>1</sup>
- PWS is most often caused by **genes** on a specific **chromosome** (chromosome 15) that are missing or do not work properly<sup>2</sup>
- PWS affects all races, ethnicities, and sexes equally<sup>4</sup>
- People with PWS have different kinds of signs and symptoms, which change with age<sup>2</sup>
- One of the most challenging parts of having PWS is “hyperphagia,” which is extreme hunger or an overwhelming urge to eat and having constant thoughts about food even when the body does not need more food<sup>4</sup>

### Learn more about genes and chromosomes

-  **Chromosomes** are packages of DNA inside human cells<sup>3</sup>  
Each human cell (except for sperm and egg cells) has 23 pairs of chromosomes<sup>3</sup>
-  **DNA** within chromosomes is made up of sections called genes<sup>3</sup>  
**Genes** carry the instructions needed for the body to function<sup>3</sup>

## What is diazoxide choline?

- Diazoxide choline extended-release tablets (more simply called diazoxide choline) is a medicine approved in the United States for the treatment of hyperphagia in people with PWS who are 4 years of age and older
- Diazoxide choline is a tablet that is taken by mouth once a day



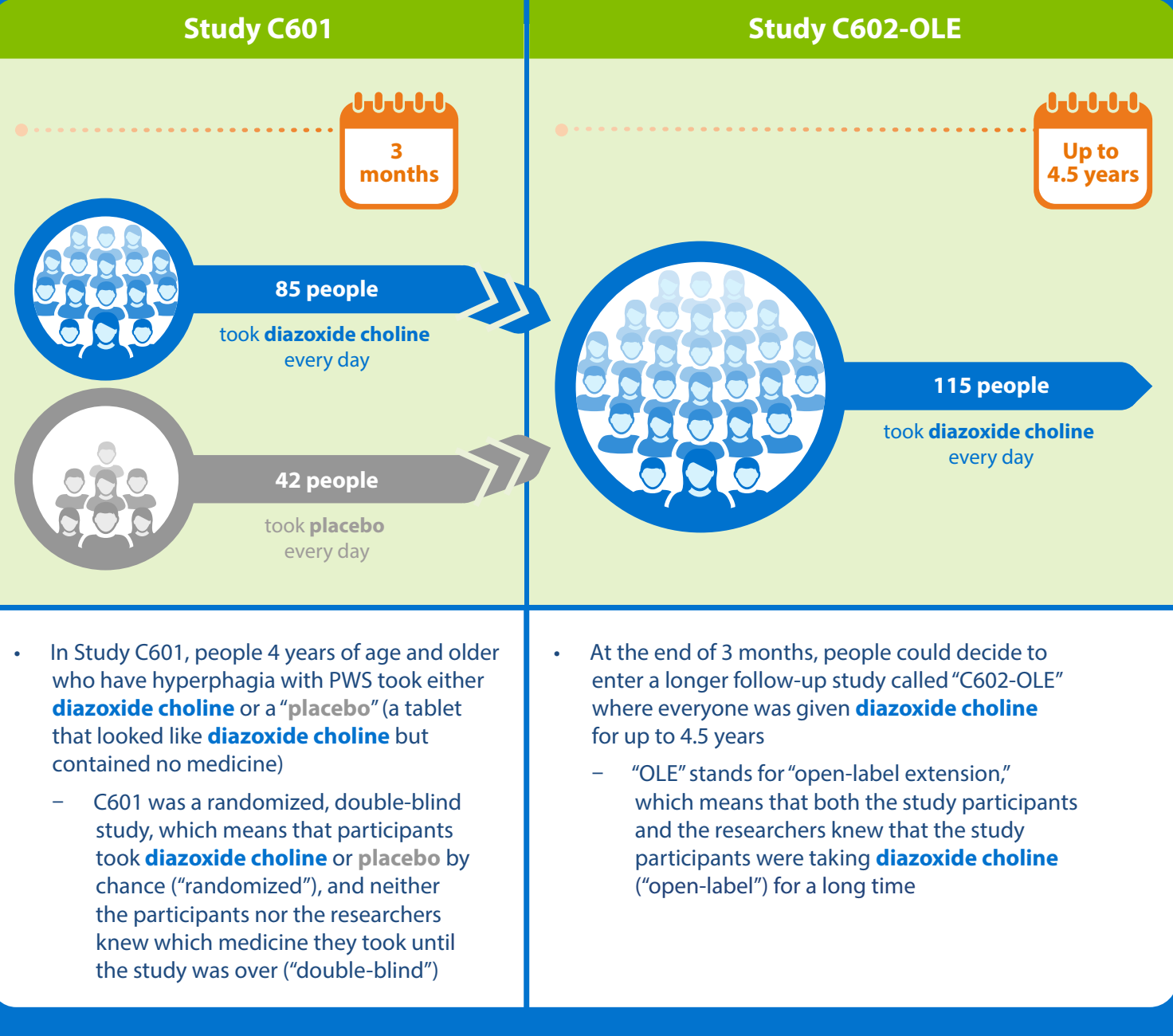
## What is peripheral edema and why is it important to monitor during diazoxide choline treatment?

- A build-up of fluid in the body can cause swelling in the lower legs, feet, and ankles (**peripheral edema**)<sup>5</sup>
- Peripheral edema can cause pain, itchiness, stiffness, and problems with walking<sup>5</sup>
- Peripheral edema is a **known side effect** that some people with PWS experience when taking diazoxide choline<sup>6</sup>

## What did this analysis look at?

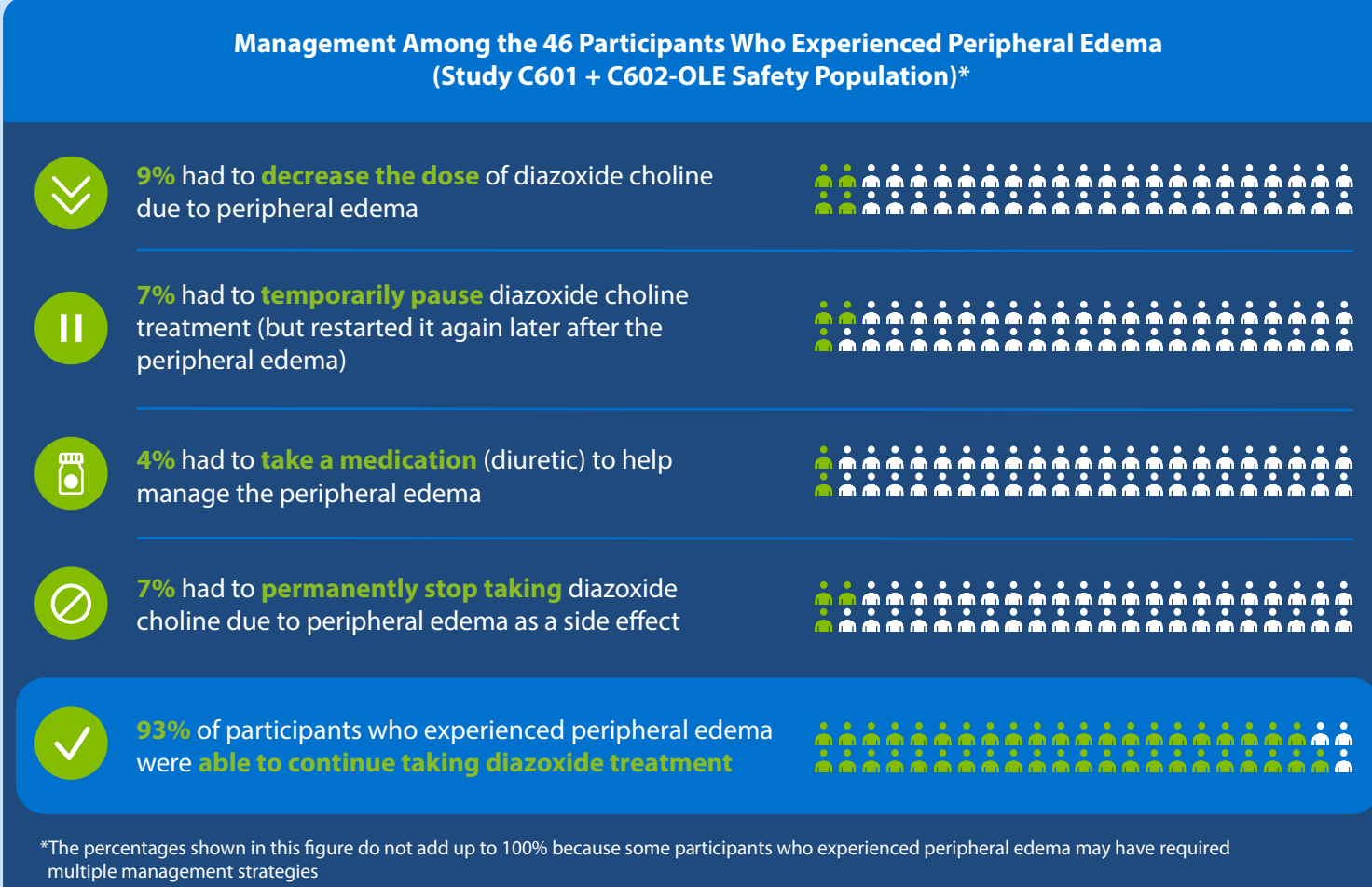
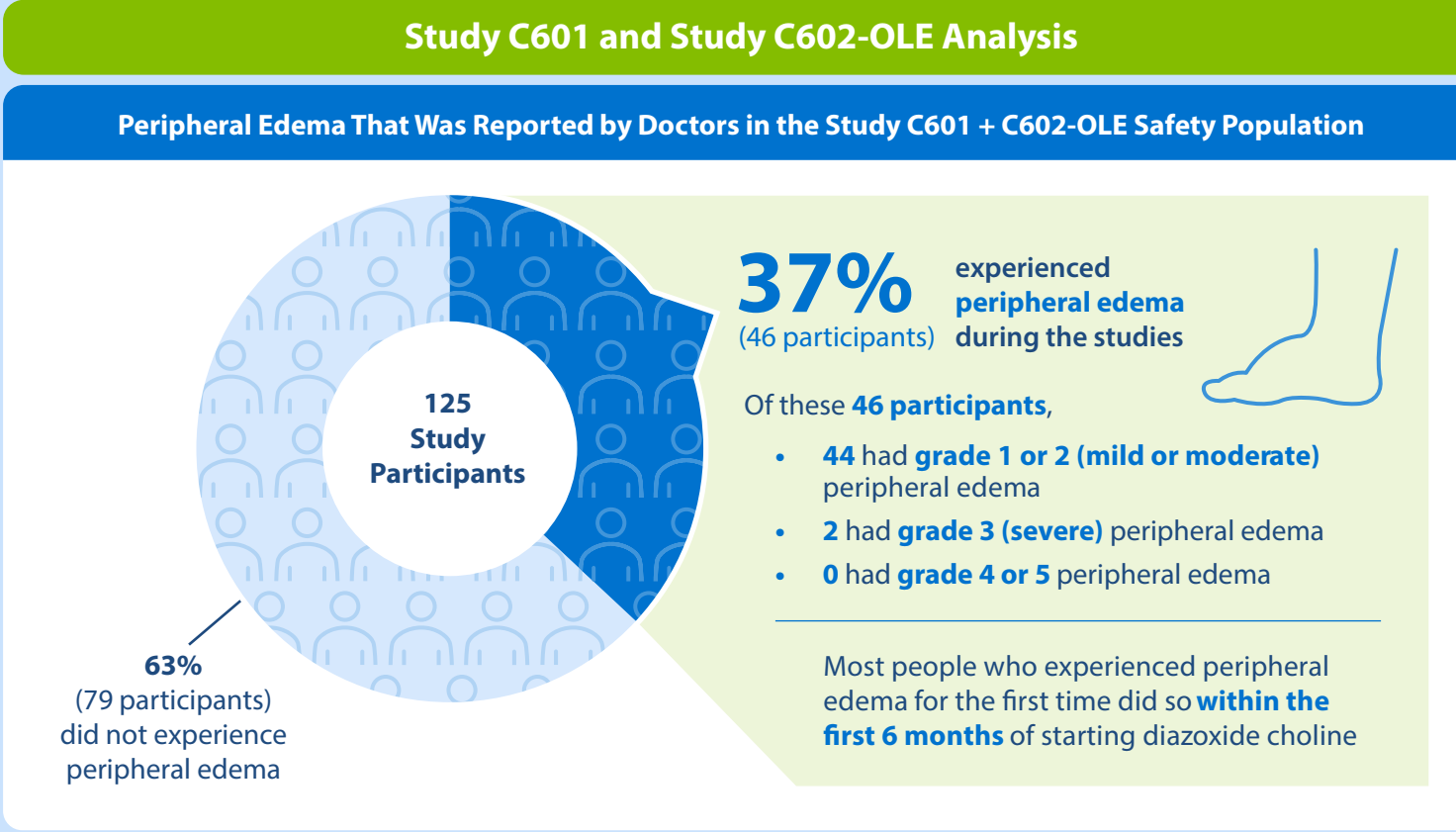
- The goal of this analysis was to understand **how often peripheral edema was experienced and how it was managed in people with PWS** who were taking diazoxide choline in clinical studies for up to 4.5 years
- Specifically, researchers wanted to answer:
  - **How many** participants developed peripheral edema and how severe was peripheral edema in these participants?
  - **When** did the peripheral edema occur after they started taking diazoxide choline?
  - **How** was peripheral edema **managed**?
- In the study, peripheral edema was identified by clinical study doctors. It was measured by gently pressing on the skin to see if it left a temporary dent (“**pitting**”), which suggests fluid had built up in that area<sup>5</sup>
  - Pitting was graded by the doctor on a scale ranging from 1+ to 4+ depending on how deep the dent was and how long it took for the skin to return to normal<sup>5</sup>

## Who participated in these studies?



## What did this analysis find?

- This analysis included 125 participants who took at least 1 dose of diazoxide choline in Study C601 or Study C602-OLE (the **Study C601 + C602-OLE Safety Population**)
- On average, participants took diazoxide choline for about 2.5 years
  - Over 50% of the participants took diazoxide choline for over 3 years
- About 15% of participants had peripheral edema before they started taking diazoxide choline in these studies



## What are the main conclusions of this analysis?

- This analysis shows that **most participants with PWS did not experience peripheral edema** during long-term treatment with diazoxide choline
  - When participants taking diazoxide choline did experience peripheral edema for the first time, it usually occurred soon after treatment started and was mostly considered mild
  - In participants who did experience peripheral edema during treatment, **peripheral edema was generally manageable**, and **most participants were able to continue** taking diazoxide choline

## Who sponsored this study?

- This study was sponsored by Soleno Therapeutics, Inc.
- This summary reports findings from 2 studies that occurred one after another
- The results shown here are from this specific analysis and may differ from results of other studies and analyses
- Health professionals should make treatment decisions based on all available evidence and on each individual patient's needs, not on the results of a single study alone

## Where can I find more information?

Read more about the C601 and C602-OLE studies

NCT03440814 (Study C601)

NCT03714373 (Study C602-OLE)



For more information about general clinical studies

[www.ClinicalTrials.gov](https://www.ClinicalTrials.gov)

### The full title of this presentation is:

Characterization of Peripheral Edema in Individuals with Prader-Willi Syndrome During Long-term Administration of Diazoxide Choline Extended-Release Tablet (DCCR) Over 4.5 Years

### Researchers

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To access a copy of this PLS and the associated poster presentation, please click here

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