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Examining Hyperglycemia 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[1]
- PWS is most often caused by **genes** on a specific **chromosome** (chromosome 15) that are missing or do not work properly[2]
- PWS affects all races, ethnicities, and sexes equally[4]
- People with PWS have different kinds of signs and symptoms, which change with age[4]
- 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[3]

Learn more about genes and chromosomes

Chromosomes are packages of DNA inside human cells[5]
Each human cell (except for sperm and egg cells) has 23 pairs of chromosomes[5]

DNA within chromosomes is made up of sections called genes[5]
Genes carry the instructions needed for the body to function[6]

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 hyperglycemia and why is it important to monitor blood sugar levels during diazoxide choline treatment?

- In almost a quarter of people with PWS, their body processes the sugar they eat differently than people without PWS[3]
- Increased blood sugar levels, or **hyperglycemia**, can cause symptoms such as feeling very thirsty and having to urinate often[3]
  - Hyperglycemia can cause serious health problems if it is not treated and goes on for a long time[4]
- Hyperglycemia and symptoms related to it are **known side effects** that people with PWS may experience when taking diazoxide choline[7]

What did this analysis look at?

- The goal of this analysis was to understand **how often hyperglycemia 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 experienced hyperglycemia?
  - **How severe** was the hyperglycemia event?
  - **How** was hyperglycemia **managed**?
- In the clinical studies, the researchers identified participants who experienced hyperglycemia and monitored blood sugar levels in 2 different ways:
  - Reports of when participants in the study experienced hyperglycemia from the clinical study doctors
  - Measurement of **HbA1c** levels over time in the blood of participants throughout the studies

Learn more about HbA1c

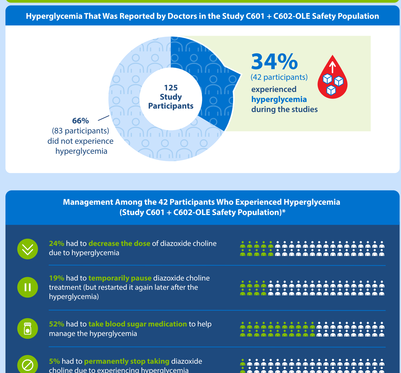
An **HbA1c (glycated hemoglobin) test** is a blood test that shows how much sugar is in a person's blood on average over the past 2 to 3 months[8]

Doctors can use HbA1c levels to check for long-term hyperglycemia and diabetes risk[1]

An HbA1c level of less than 5.7% is considered **normal blood sugar**[8]

An HbA1c level of 5.7% or higher indicates long-term **hyperglycemia** and possible risk for diabetes[8]

Who participated in these studies?



- In Study C601, people 4 years of age and older who had hyperphagia with PWS took either **diazoxide choline** or a "placebo" (a tablet that looked like **diazoxide choline** but contained no medicine)
  - C601 was a randomized, double-blind study, which means that participants took **diazoxide choline** or placebo by chance ("randomized"), and neither the participants nor the researchers knew which medicine they took until the study was over ("double-blind")

- At the end of 3 months, people could decide to enter a longer follow-up study called "C602-OLE" where everyone was given **diazoxide choline** for up to 4.5 years
  - "OLE" stands for "open-label extension," which means that both the study participants and the researchers knew that the study participants were taking **diazoxide choline** ("open-label") for a long time

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
- At the start of this analysis
  - About 8% of participants were taking medications to lower their blood sugar, like metformin
  - The average HbA1c value of participants was 5.6%

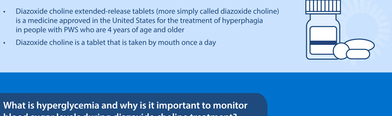
Study C601 and Study C602-OLE Analysis

Hyperglycemia That Was Reported by Doctors in the Study C601 + C602-OLE Safety Population

Management Among the 42 Participants Who Experienced Hyperglycemia (Study C601 + C602-OLE Safety Population)*

*The percentages shown in this figure do not add up to 100% because some participants who experienced hyperglycemia may have required multiple management strategies

Average HbA1c Levels Over 3 Years



What are the main conclusions of this analysis?

- This analysis shows that across all the participants with PWS included in the Study C601 + C602-OLE Safety Population
  - **Most participants did not experience hyperglycemia** during long-term treatment with diazoxide choline
  - In participants who did experience hyperglycemia during treatment, **hyperglycemia was generally manageable**, and **most participants were able to continue** taking diazoxide choline
  - The **average HbA1c level increased slightly above normal** levels over 3 years of treatment with 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

The full title of this presentation is:
The Glycemic Outcomes of Diazoxide Choline Extended-Release (DCCR) Tablets Administered for Hyperphagia in Individuals with Prader-Willi Syndrome Over 4 Years

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

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