Pediatric Endocrine Society Annual Meeting; May 15-18, 2025; National Harbor, Maryland, USA

Taking Diazoxide Choline Extended-Release Tablets for 1 Year or Longer Improved Body Composition in People With Prader-Willi Syndrome

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 commonly 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[2]
- Compared to people of the same age without PWS, people with PWS have a:
  - Higher percentage of fat in their bodies[5]
  - Lower percentage of "**lean body mass**," which includes muscles and organs (basically everything except fat)[5]
- A previous research study in boys with PWS who were treated with growth hormone showed that the average lean mass index increased by 0.28 kg/m^2 per year before puberty and 0.74 kg/m^2 per year during puberty[7]
  - Lean mass index is a way of calculating lean body mass relative to a person's height; it is measured in kilograms per meter squared ("kg/m^2")

Learn more about genes and chromosomes

Learn more about lean body mass

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 did this analysis look at?

- This analysis looked at if taking diazoxide choline long term (for up to 3 years) could continue to increase the **amount of lean body mass** in people with PWS
  - In the study, researchers measured study participants' lean body mass using a special type of X-ray test (called a "DEXA scan")
- Researchers also calculated the participants' **lean mass index**
  - Because it took height into account, the lean mass index factored in any height growth that occurred over the time of the study in young participants

Who participated in these studies?

Study C601

- In Study C601, people 4 years of age and older who have 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")

Study C602-OLE

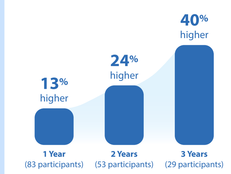
- At the end of 3 months, people could decide to enter a longer follow-up study called "C602-OLE" where everyone was given **diazoxide choline** for up to 4.3 years
  - "OLE" stands for "open-label extension," which means that both the study participants and the researchers knew that the study participants were taking **diazoxide choline** ("open-label") for a long time

What did this analysis find?

- A total of 125 participants who took at least 1 dose of diazoxide choline in Study C601 and Study C602-OLE were included in this analysis
- At the start of this analysis:
  - Participants were on average 13.4 years old
  - Average weight of participants was 62.1 kg and average height was 146.7 cm
  - Average lean body mass of participants was 29.0 kg

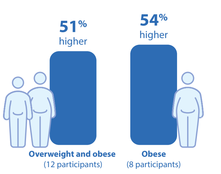
Study C601 and Study C602-OLE Analysis

Change in Lean Body Mass in All Participants Over Time



The percentage of lean body mass increased in study participants who took diazoxide choline for 1 year, and lean body mass continued to increase over 3 years

Change in Lean Body Mass After 3 Years in Participants Who Were Overweight and/or Obese



In study participants with PWS who were overweight or obese before they started diazoxide choline, **lean body mass increased by more than 50% after 3 years of treatment**

Change in Lean Mass Index After 1 Year in Participants of Different Ages

After 1 year of diazoxide choline treatment, **the lean mass index of study participants increased across all age groups**

What are the main conclusions of this analysis?

- This analysis shows that treatment with **diazoxide choline increased lean body mass at all time points over 3 years** in people with PWS
- The improvements in **lean body mass** were largest in:
  - **Younger children** who were less than 12 years of age
  - People who were **overweight or obese** at the start of the study
- While gaining more lean body mass might lead to improved strength, metabolism, and physical abilities, these potential effects have not yet been studied in people with PWS who received 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 of this analysis may differ from those of other studies. Health professionals should make treatment decisions based on all available evidence, not on the results of a single study

Where can I find more information?

Read more about the C601 and C602-OLE studies

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For more information about general clinical studies

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Researchers
Jennifer Miller, Jack Yanovski, Michael Huang, Jing Gong, Neil Cowen, and Evelien Gevers

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Learn more about genes and chromosomes ✕

Chromosomes are packages of DNA inside human cells[3]
Each human cell (except for sperm and egg cells) has 23 pairs of chromosomes[3]

DNA within chromosomes is made up of sections called genes[3]
Genes carry the instructions needed for the body to function[3]

- PWS affects all races, ethnicities, and sexes equally[4]
- People with PWS have different kinds of signs and symptoms, which change with age[2]
- Compared to people of the same age without PWS, people with PWS have a:
  - Higher percentage of fat in their bodies[5]
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  - Lean mass index is a way of calculating lean body mass relative to a person's height; it is measured in kilograms per meter squared ("kg/m^2")

Learn more about lean body mass

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 did this analysis look at?

- This analysis looked at if taking diazoxide choline long term (for up to 3 years) could continue to increase the **amount of lean body mass** in people with PWS
  - In the study, researchers measured study participants' lean body mass using a special type of X-ray test (called a "DEXA scan")
- Researchers also calculated the participants' **lean mass index**
  - Because it took height into account, the lean mass index factored in any height growth that occurred over the time of the study in young participants

Who participated in these studies?

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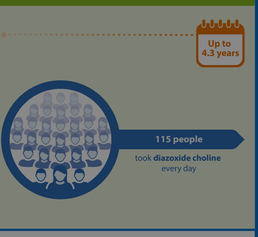
- In Study C601, people 4 years of age and older who have hyperphagia with PWS took either **diazoxide choline** or a "placebo" (a tablet that looked like **diazoxide choline** but contained no medicine)
  - C601 was a randomized, double-blind study, which means that participants took **diazoxide choline**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

Study C602-OLE



- 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 4.3 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?

- A total of 125 participants who took at least 1 dose of diazoxide choline in Study C601 and Study C602-OLE were included in this analysis
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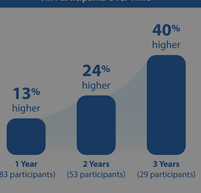

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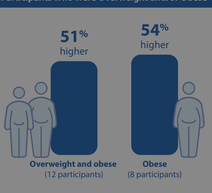
Change in Lean Body Mass After 3 Years in Participants Who Were Overweight and/or Obese



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After 1 year of diazoxide choline treatment, **the lean mass index of study participants increased across all age groups**

What are the main conclusions of this analysis?

- This analysis shows that treatment with **diazoxide choline increased lean body mass at all time points over 3 years** in people with PWS
- The improvements in **lean body mass** were largest in:
  - **Younger children** who were less than 12 years of age
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Learn more about lean body mass

- People with a high level of body fat and a low level of lean body mass are more likely to **develop heart disease, breathing difficulties, and become unwell** compared to people with lower body fat and higher lean body mass[6]
- For people with PWS, increasing lean body mass may lead to other health benefits, such as **improved strength and energy use** (also called **metabolism**)[7]

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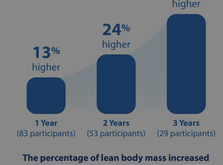

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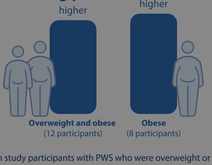

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