CLINICAL STUDY RESULTS

A study to learn about the efficacy and safety of triptorelin 6-month formulation in Chinese children with Central Precocious Puberty (CPP)

This study showed that triptorelin 6-month formulation was effective in suppressing early puberty in children with CPP.

The results shown in this summary are from one clinical study. Other clinical studies may produce different results.

This lay summary has been produced by Ipsen with the assistance of a third party writing service provider.

D-CN-52014-244
January 2024

What was the study about?

The study is to learn about the efficacy and safety of a 6month formulation of triptorelin on central precocious puberty (CPP).

Children with CPP start to mature sexually too early. This means that they start to develop secondary sexual characteristics generally before 8 years of age in girls and before 9 years of age in boys. Secondary sexual characteristics in girls include growth of breasts and first menstrual period. In boys, there are changes in voice, beard hair starts to appear, and release of sperm happens at night. In both sexes, there are changes in external genitalia and appearance of pubic and armpit hair.

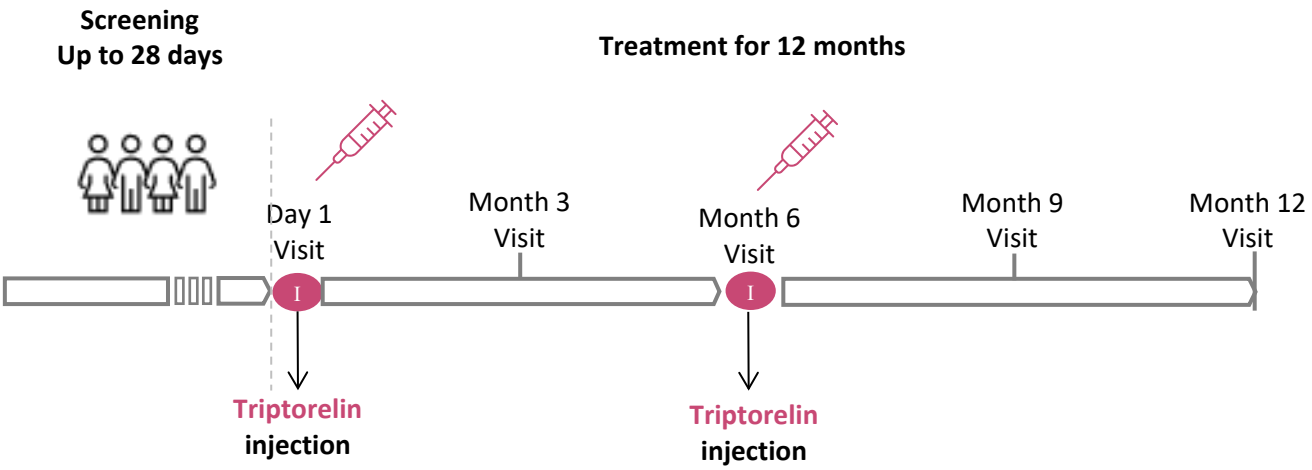
In CPP, the part of the brain that releases sex hormones develops and becomes active before the normal time and starts releasing these hormones. Hormones are messengers which tell the body what to do and when to do it. In CPP, sex hormones called luteinizing hormone (LH) and follicle stimulating hormone (FSH) are released too early. This causes puberty to happen early.

Triptorelin when given continuously and in regular doses can control the release of sex hormones. Currently in China, triptorelin 1-month formulation (1 injection once a month) and 3-month formulation (1 injection every 3 months) are used to treat children with CPP at the time of this study report. The aim of this study was to learn if triptorelin 6-month formulation (1 injection every 6 months) was effective in reducing LH to pre-puberty levels in children with CPP. This triptorelin 6-month formulation may help reduce the number of times the injection is given and increase comfort in the management of CPP.

The aim of this study was to learn about the efficacy of triptorelin 6-month formulation in reducing LH levels to pre-puberty levels in children with CPP.

This study took place between August 2021 and February 2023 at 15 sites in China.

This was an “open label” study, which means that both researchers and participants knew what treatment participants were receiving.

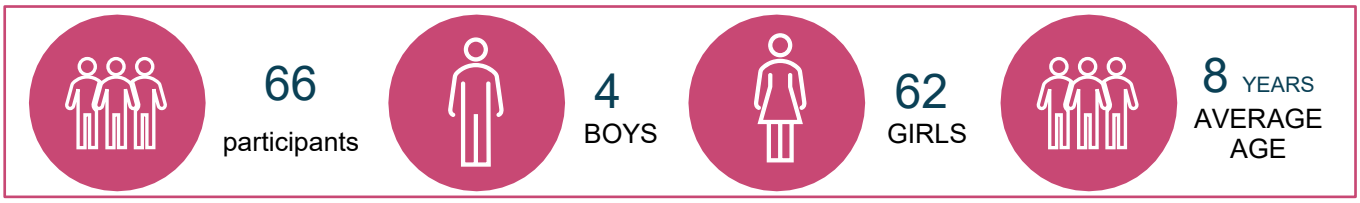


The study consisted of a Screening period, during which the study doctor assessed if the participants were eligible to enter the study. Participants had study visits at screening, Day 1, and Months 3, 6, 9, and 12. Their health was monitored throughout the study.

Each participant was enrolled in this study for around 1 year.

Who took part in this study?

A total of 66 participants were included and treated in the study.



To be eligible to take part in the study, participants:

- had to be less than 9 years old for girls and less than 10 years old for boys, and
- had to be diagnosed with CPP.

Participants were not eligible to take part in the study if they had any health condition making them unfit for the study, according to the study doctor.

What treatments were used?

Participants received triptorelin injections into the muscles on Day 1 and Month 6 of the study.

How many children had LH levels back to pre-puberty levels at Month 6?

LH levels in the blood were checked after giving the child a "stimulation test", which is done to test for CPP. The test involves taking a blood sample, then giving the child an injection containing a hormone that increases the release of LH. More blood samples are then taken over timepoints decided beforehand by the researchers to show how hormones in the child's body react. In children with CPP, this test causes the levels of LH to raise.

In this study, 100% (all 66) of children treated with injection of triptorelin, and tested at Month 6 with the stimulation test, had LH levels return to pre-puberty levels.

How did the treatment make participants feel?

During clinical studies, participants are asked to report if they feel unwell, experience any kind of medical event, or notice anything different about their bodies. These are called 'adverse events'. Researchers record *all* adverse events reported by participants, whatever the cause.

If the study doctor thinks an adverse event may be related to the study treatment, it is called a 'side effect'. A side effect is considered 'serious' when it is life-threatening, causes lasting problems, or leads to hospitalization.

- Side effects that are *life-threatening*, cause lasting problems or require an individual to go to the *hospital* are considered s*erious*.
- No participants experienced serious side effects.
- There were no deaths related to study treatment.

During the study 13 out of 66 participants (20%) experienced side effects.

Triptorelin was generally well tolerated by participants. No participant stopped taking part in the study because of a side effect.

The most commonly reported side effects that occurred in at least 3% (3 out of 100) of participants are shown in the table below. The table shows a percentage (%) followed by the actual number of participants in the group (example: 9%, 6 out of 66).

Side Effects (at least 3% of participants)	Triptorelin (66 Participants)	
Weight gain	9% (6 out of 66)	
Obesity	3% (2 out of 66)	
Vaginal bleeding	3% (2 out of 66)	
Fatty liver disease	3% (2 out of 66)	

More information

To learn more about this study, please visit the clinicaltrials.gov website and search for study NCT05029622.

For more information about current treatments available, please speak to your healthcare provider. If you have any questions about this study, please contact the sponsor, Ipsen at:

clinical.trials@ipsen.com

Future research

There is no future research planned on this topic.

Study identification and other information

Full study title: A phase III, open-label, multicentre, single arm study to assess the efficacy and safety of the triptorelin 6-month formulation in Chinese paediatric participants with central precocious puberty

Study number: United States: NCT05029622

Protocol: D-CN-52014-244

Other information: Phase 3 studies can take several months to years to complete and look at the effects and safety of a potential new treatment.

We thank all the volunteers who took part in this study. Without their support, advances in treatments for medical conditions would not be possible.

We would also like to thank the people who took the time to review this document to make it easier for a general audience to read.