CTEXLI is the **only** FDA-approved treatment for cerebrotendinous xanthomatosis (CTX) in adults. It is designed to help stop the buildup of CTX toxins and prevent them from being stored throughout the body where they can cause symptoms. Talk to a health care provider to learn more about CTEXLI.

CTX leaves critical gaps in bile acid production.
CTEXLI helps replenish bile acid levels.

INDICATION

CTEXLI is indicated for the treatment of cerebrotendinous xanthomatosis (CTX) in adults.

IMPORTANT SAFETY INFORMATION

What are the possible side effects of CTEXLI?

- **Serious Side Effects—Hepatotoxicity (Liver Injury):** You will need to undergo laboratory testing before starting and periodically while on treatment with CTEXLI to assess liver function. Changes in certain liver tests may occur during treatment and may be a sign of liver injury. People with preexisting liver disease or bile duct abnormalities may be at higher risk for liver injury during treatment.

Please see Important Safety Information throughout and accompanying full Prescribing Information.

Ctexli™
(chenodiol) tablets 250mg

What is CTX?

Cerebrotendinous xanthomatosis ***(suh-ree-bro-ten-din-us zan-tho-ma-toe-sis)***, or CTX, is a **rare, inherited lipid storage disorder**. Lipids are a type of fat. Lipid storage disorders happen when the body is not able to break down lipids. When this happens, lipids and other toxic substances build up in the body. The buildup can interfere with how the body works.

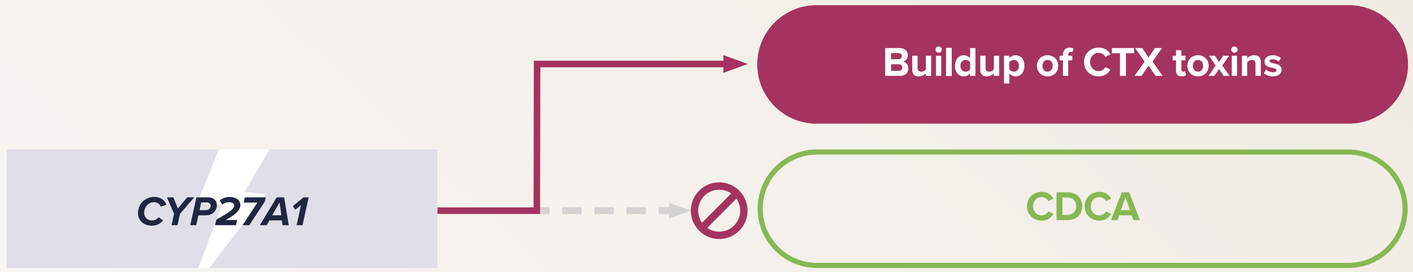
People with CTX have **a mutation in the *CYP27A1* gene** that prevents an important enzyme (sterol 27-hydroxylase) from working properly. An enzyme is a protein that speeds up certain processes in the body.

When this particular enzyme malfunctions, it prevents the body from breaking down fats and making certain bile acids needed to help digest food. As a result, **CTX toxins build up in the bloodstream, eyes, tendons, brain, and other tissues over time**. This buildup then leads to a range of signs and symptoms.

Because CTX is genetic, it can run in families. Get family members tested for CTX, too.

CTX prevents the body from making a bile acid called chenodeoxycholic acid (CDCA)

CDCA plays an important role in regulating the bile acid production process. When there is not enough CDCA, it can cause the buildup of CTX toxins.



CTX is progressive, meaning it may get worse over time. Early diagnosis and treatment may help prevent further damage.

IMPORTANT SAFETY INFORMATION (cont'd)

- **Serious Side Effects—Hepatotoxicity (Liver Injury):** Stop taking CTEXLI immediately and tell your healthcare provider right away if you get any signs or symptoms of liver problems, including:
  - Stomach (abdomen) pain
  - Bruising
  - Dark-colored urine
  - Feeling tired (fatigue)
  - Bleeding
  - Yellowing of the skin and eyes
  - Nausea
  - Itching

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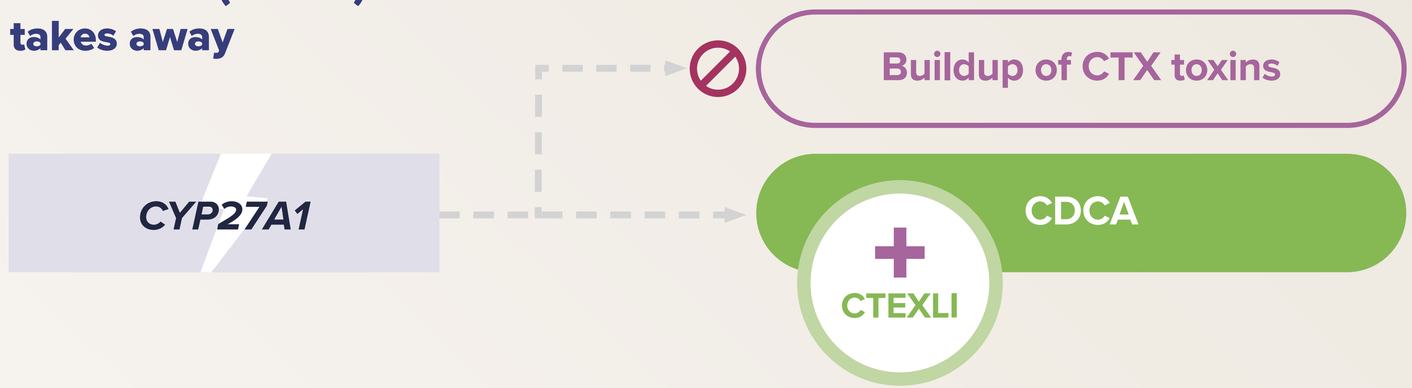
How does CTEXLI work?

CTEXLI helps put back the chenodeoxycholic acid (CDCA) that cerebrotendinous xanthomatosis (CTX) takes away

CTEXLI is designed to help stop the buildup of CTX toxins. It does this by replacing the CDCA missing in adults with CTX.



CTEXLI helps to:

Replenish

CDCA, an important bile acid the body has trouble making because of CTX

Restart

the cycle that prevents more harmful toxins from being made

Reduce

levels of harmful toxins in the blood and urine that may lead to CTX signs and symptoms

IMPORTANT SAFETY INFORMATION (cont'd)

- **Most Common Side Effects:** Diarrhea, headache, stomach pain, constipation, high blood pressure, muscular weakness, and upper respiratory tract infection.
- Tell your healthcare provider about all the medications that you take, as CTEXLI may interact with other medicines. Do not take bile acid sequestering agents or aluminum-based antacids with CTEXLI. If you take coumarin, your doctor will need to monitor your prothrombin time (PT) while taking CTEXLI and may adjust your coumarin dose.

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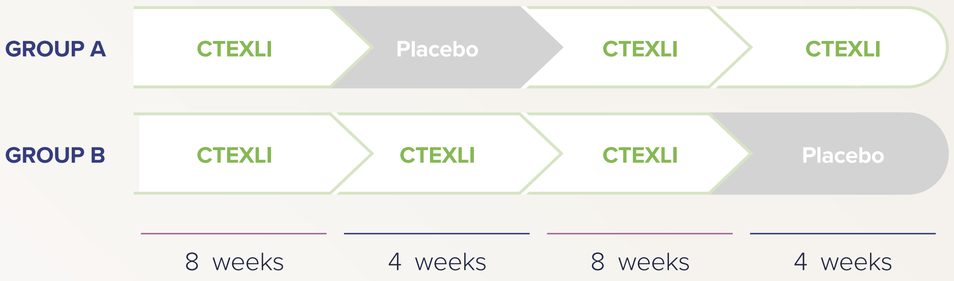
How was CTEXLI studied?

CTEXLI was evaluated in a 24-week clinical study

The purpose of the study was to see if CTEXLI helps reduce the production of 2 key cerebrotendinous xanthomatosis (CTX) toxins.



13* people aged 16 and older participated in the study. It was a randomized, double-blind study, meaning neither the participants nor the doctors knew who received CTEXLI or a placebo. Placebo looked like "real" medicine, but did not have any active medicine.

- All 13 participants received CTEXLI for the first 8 weeks
- Participants were split into 2 groups for the next 4 weeks:
  - One group received CTEXLI
  - The other group received a placebo
- Both groups received CTEXLI again for 8 weeks
- In the final 4 weeks, the groups switched treatments:
  - The group that had CTEXLI received placebo
  - The group that had placebo received CTEXLI

*14 participants signed up for the study, but only 13 were ultimately included.

Improvements were seen within 4 weeks

Lower levels of toxins were achieved in participants taking CTEXLI vs those who took placebo

Patients taking CTEXLI saw lower levels of toxins in the blood and urine after 4 weeks on treatment compared with those on placebo.

With ongoing CTEXLI treatment, the reduction in CTX toxins lasts over time

Toxin levels in the blood and urine stayed consistently low with continued CTEXLI use.

When participants stopped taking CTEXLI, CTX toxin levels increased

When patients switched from CTEXLI to placebo, CTX toxins in the blood and urine increased within 2 weeks.

Since stopping CTEXLI may cause CTX toxins in the blood and urine to go back up, it's important to keep taking CTEXLI as prescribed by your health care provider.

IMPORTANT SAFETY INFORMATION (cont'd)

- **Most Common Side Effects:** Your healthcare provider may temporarily or permanently stop treatment if you have certain side effects.

CTEXLI is taken by mouth three (3) times each day and can be taken with or without food. Swallow tablets whole.

These are not all the possible side effects of CTEXLI. For more information, ask your healthcare provider or pharmacist. Tell your doctor if you have any side effect that bothers you or that does not go away.

Please see Important Safety Information throughout and accompanying full Prescribing Information.

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What are the possible side effects?

CTEXLI may cause side effects. Talk to your doctor about what to expect and how to manage any side effects

The most common side effects with CTEXLI include:

- Frequent, loose, or watery stools (diarrhea)
- Headache
- Stomach (abdominal) pain
- Constipation
- High blood pressure (hypertension)
- Weak or tired muscles
- Infection in the nose, throat, or sinuses (upper respiratory tract infection)

Hepatotoxicity (liver injury) is a potential serious side effect of CTEXLI. You will need to undergo laboratory (blood) testing before starting and periodically while on treatment with CTEXLI to assess liver function. Changes in certain liver tests may occur during treatment and may be a sign of liver injury. People with preexisting liver disease or bile acid abnormalities may be at a higher risk for liver injury during treatment. Stop taking CTEXLI immediately and tell your health care provider right away if you get any signs or symptoms of liver problems, including:

- Stomach (abdominal) pain
- Bruising
- Dark-colored urine
- Feeling tired
- Bleeding
- Yellowing of the skin and eyes
- Nausea
- Itching

Your doctor may change your dose or temporarily or permanently stop treatment with CTEXLI if you have certain side effects. These are not all the possible side effects of CTEXLI. For more information, ask your health care provider or pharmacist.

You may report side effects to the FDA at 1-800-FDA-1088 or www.fda.gov/medwatch. You may also report side effects to Mirum Pharmaceuticals at 1-855-MRM-4YOU (1-855-676-4968).

How is CTEXLI taken?

Take CTEXLI as prescribed. The recommended CTEXLI dose is **one 250-mg oral tablet 3 times per day**, with or without food. Swallow tablets whole.

What happens if a dose is missed?

Take CTEXLI as prescribed by your doctor. If you forget to take one of your scheduled CTEXLI tablets, skip the missed tablet. Take your next tablet at the normal, scheduled time. **Do not take 2 tablets at once.**

Will CTEXLI affect or be affected by other medications?

Tell your health care provider about all the medications that you take, as CTEXLI may interact with other medicines. Do not take bile acid sequestering agents or aluminum-based antacids with CTEXLI. If you take coumarin, your doctor will need to monitor your prothrombin time (PT) while taking CTEXLI and may adjust your coumarin dose.

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What resources are available?

When starting a new medication, it's hard to know what to expect and where to start.

Mirum Access Plus works closely with your doctor and insurance plan to help facilitate coverage for CTEXLI. Mirum Access Plus also offers ongoing support and education for patients and caregivers.

Sign up by calling us at

1-855-MRM-4YOU (1-855-676-4968)

Monday through Friday, 8:00 AM through 8:00 PM ET,
or by visiting **CTEXLI.com** to download
and complete the consent form.

IMPORTANT SAFETY INFORMATION (cont'd)

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CTX leaves critical gaps in bile acid production.
CTEXLI helps replenish bile acid levels.

Improvements were seen within 4 weeks

- Patients taking CTEXLI achieved lower levels of toxins than patients taking placebo
- Continued treatment with CTEXLI helps keep toxin levels lower over time

Daily oral dosing

- One tablet, by mouth, 3 times a day
- Take with or without food, and swallow tablets whole

Established safety

- The safety of CTEXLI was carefully studied in a trial
- The most common side effects of CTEXLI are diarrhea, headache, stomach pain, constipation, high blood pressure, muscular weakness, and upper respiratory infections

Talk to a health care provider and visit **CTEXLI.com** to learn more.

INDICATION

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