SEE THE SIGNS

Uncover what's behind movement disorders

If your child or loved one is experiencing or has experienced ataxia (poor muscle control that causes clumsy movements) or another movement disorder that has affected their walking, balance, and muscle movements, it may be caused by a rare, but treatable, genetic disorder called cerebrotendinous xanthomatosis (CTX). With available testing, you may be able to find out the underlying issue.

Many people don't realize they have CTX

Because CTX is a rare disorder with many symptoms, it usually is not discovered in patients until 20 to 25 years after symptoms start.

Symptoms of CTX

In people with CTX, cholesterol doesn't break down properly, causing cholestanols—toxins and bile alcohols—to build up in blood and tissues throughout the body. This buildup can cause a range of symptoms, affecting the brain and nervous system and causing neurologic deterioration (developmental delay or behavioral problems).

55%
of people with CTX experience gait ataxia, which can be described as "walking unsteadiness" or "balance problems when walking"

CTX affects multiple organ systems and causes different symptoms in different people at different ages and can lead to serious health problems over time.

These are some other possible symptoms of CTX:

- Early-onset cataracts in both eyes
- Tendon xanthomas (bumps on elbows, knees, or heels)
- Cholestasis/prolonged jaundice (yellowing of the skin or eyes) in infants
- Frequent diarrhea

Early treatment of CTX may lead to better outcomes

Testing early on is so important because it can help find the underlying cause, which means doctors could start treating sooner. Early treatment may help prevent serious health problems and potentially lead to better outcomes for people with CTX.

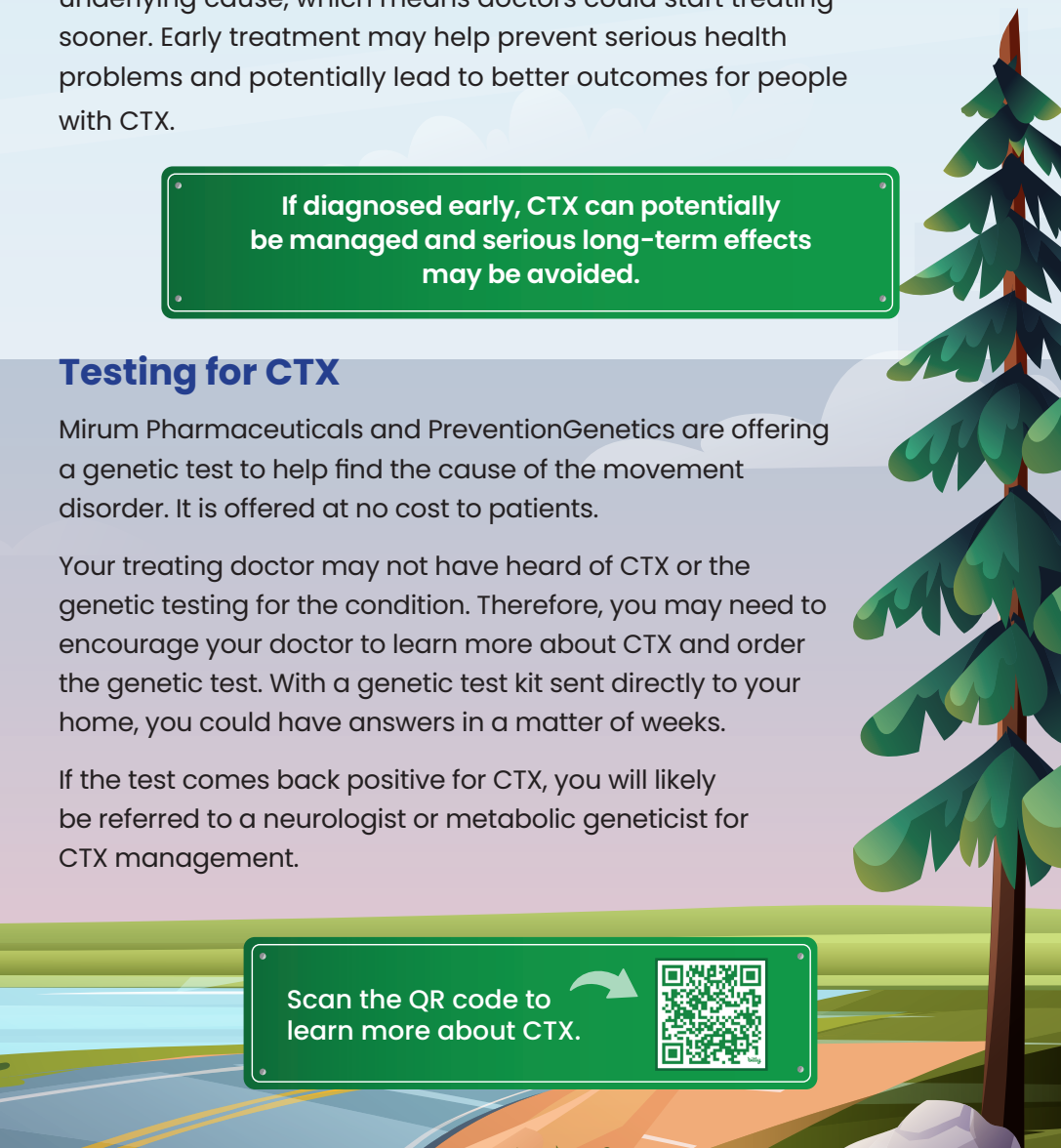
If diagnosed early, CTX can potentially be managed and serious long-term effects may be avoided.

Testing for CTX

Mirum Pharmaceuticals and PreventionGenetics are offering a genetic test to help find the cause of the movement disorder. It is offered at no cost to patients.

Your treating doctor may not have heard of CTX or the genetic testing for the condition. Therefore, you may need to encourage your doctor to learn more about CTX and order the genetic test. With a genetic test kit sent directly to your home, you could have answers in a matter of weeks.

If the test comes back positive for CTX, you will likely be referred to a neurologist or metabolic geneticist for CTX management.



Scan the QR code to learn more about CTX.

Explore a No-Cost Testing Option

Scout by Mirum: Movement Disorders Genetic Panel

If you or your child is experiencing any movement disorders (conditions that cause symptoms such as imbalance, difficulty walking, ataxia), Mirum Pharmaceuticals and PreventionGenetics are offering a genetic test for CTX at no cost*,† to patients to help find the cause of movement disorders. **Talk with your neurologist or geneticist about ordering a no-cost test.**

Criteria for No-Cost CTX Testing

Patient has documented Movement Disorder* plus one or more of the following (please check one or more):

- ☐ Current or past history of idiopathic cataracts not congenital in nature
- ☐ Tendon or tuberous xanthomas
- ☐ Unexplained chronic diarrhea

AND

Patient must meet both criterion below (please check both):

- ☐ Patient lives in the US
- ☐ Patient is under 50 years of age

*MDS (movement disorders) including but not limited to ataxia, atypical parkinsonism, dystonia, dyskinesia, chorea, tremors, myoclonus, epilepsy, seizures, isolated spastic syndrome, neurodegenerative disorders, dementia, development of cognitive and behavioral disturbances.

***Program may be canceled or changed at any time. Eligibility criteria apply.**

†Note that Mirum Pharmaceuticals cites the above-named external testing resource for information purposes only, and does not endorse or guarantee in any way the services or advice provided by them.

To learn more about the no-cost Scout by Mirum: Movement Disorders Genetic Panel, visit **AboutCTX.com**