

Have you or a loved one been diagnosed with **OTC deficiency**?

ORNITHINE TRANSCARBAMYLASE (or-nuh-theen trans-car-bamma-lace) **DEFICIENCY**, or **OTC DEFICIENCY**, is a rare genetic disorder caused by a missing or deficient OTC enzyme. When someone has OTC deficiency, ammonia does not get removed from the body, which can cause serious illness or life-threatening symptoms.

NEW TREATMENTS ARE BEING STUDIED TO ADDRESS THE ROOT CAUSE OF OTC DEFICIENCY AND LESSEN DISEASE BURDEN FOR PEOPLE LIVING WITH THIS CONDITION.

What is **ARCT-810**?

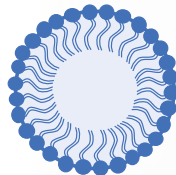
ARCT-810 is a new investigational treatment that is designed to address the cause of OTC deficiency.

ARCT-810 CONSISTS OF 2 KEY PARTS



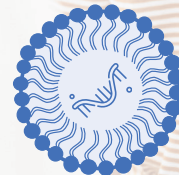
Messenger RNA, or **mRNA**, carries the template for making functional OTC enzyme.

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Lipid nanoparticle sphere contains tiny fat particles that protect the mRNA and allow it to be delivered into liver cells.

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We believe these 2 parts work together in ARCT-810 to enable the body to make functioning OTC enzyme to remove excess ammonia.

How is **mRNA therapy** different from gene therapy?

FIRST, the mRNA is short-lived and does not change one's genes or cause permanent and irreversible effects.

SECOND, gene therapy uses viruses to deliver the treatment, while mRNA therapies use lipid (fat) nanoparticles.

How does **ARCT-810** potentially work in OTC deficiency?

IN OTC DEFICIENCY, THE OTC GENE CONTAINS INCORRECT INSTRUCTIONS THAT LEAD TO LOW-FUNCTIONING OR ABSENT OTC ENZYME, RESULTING IN ILLNESS IF NOT TREATED.

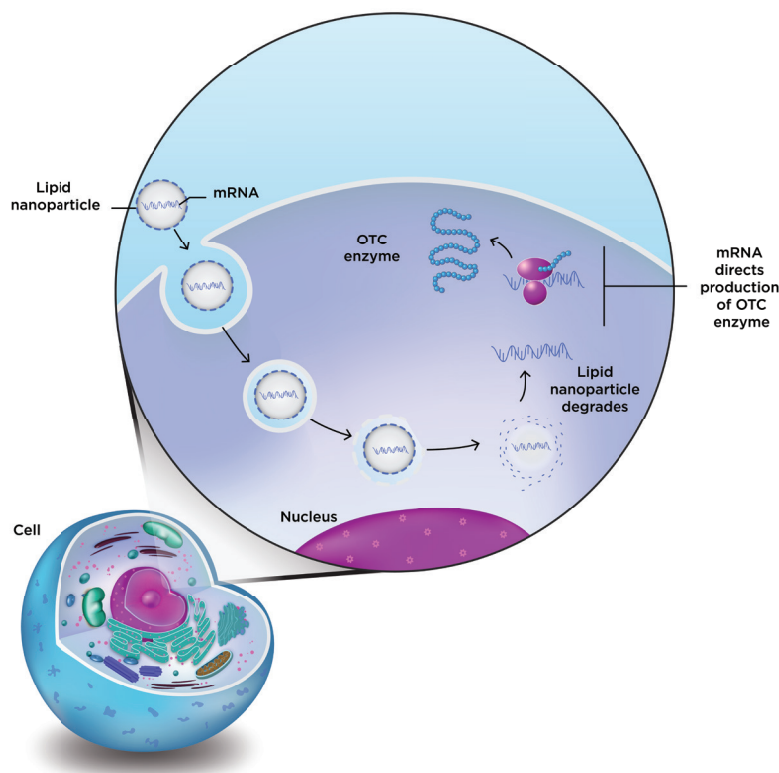
The mRNA in ARCT-810 is designed to be a template for making OTC enzyme. Arcturus uses its lipid nanoparticle technology, called LUNAR[®], to deliver the mRNA template for OTC to the liver, the place where OTC enzyme is made.

Animal studies show that:

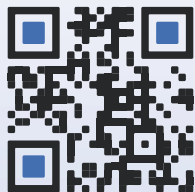
ARCT-810 is taken into liver cells and releases the mRNA with the correct instructions to make functional OTC enzyme.

The mRNA is broken down over time and does not enter the nucleus, so it does not have the potential to alter or replace existing genes.

Therefore, it is believed that ARCT-810 will not cause permanent or irreversible changes to DNA.



AT ARCTURUS, WE ARE DEVELOPING mRNA MEDICINES DESIGNED TO TREAT THE UNDERLYING CAUSE OF GENETIC DISEASES LIKE OTC DEFICIENCY WITH THE HOPE OF PROVIDING MEANINGFUL TREATMENT OPTIONS.



SCAN THE QR CODE TO
LEARN MORE ABOUT OTC
DEFICIENCY, OR VISIT:
www.OTCmRNAStudy.com

LUNAR-OTC



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