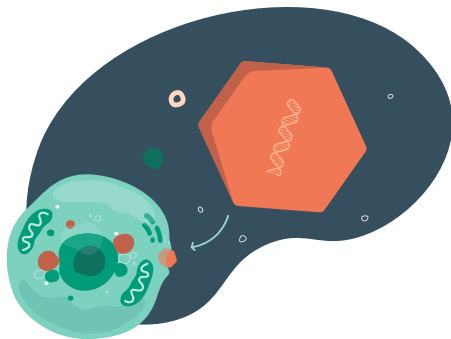


Gene Therapy for XLMTM

XLMTM is a serious and rare genetic disorder. Fortunately, gene therapy has created cause for optimism—here's how it works.

Identify the Gene

The faulty MTM1 gene is identified through genetic testing, then eligibility for treatment can be determined.

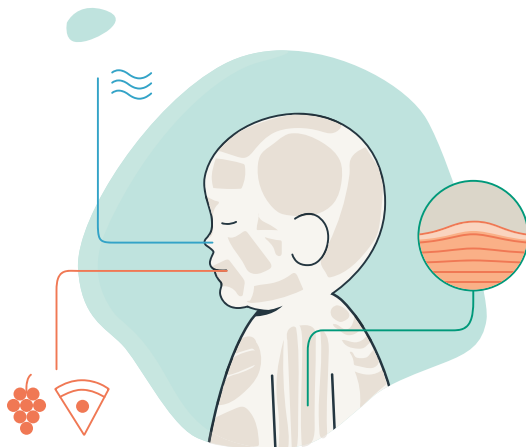
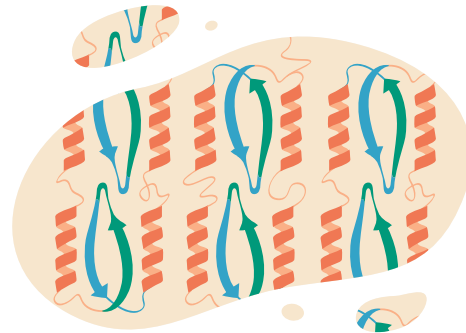


Vector delivers new gene

A vector - which is a virus that is not known to cause disease - is used to deliver the new working MTM1 genes into the cells.

Missing protein is produced

When the vector is administered into the bloodstream it is able to reach the affected muscles to produce enough myotubularin protein.



Controlling the Disease

The new MTM1 gene is now doing the work that the faulty gene could not. Effects of the disease are not reversed, but it should improve the ability to breathe, swallow and move on their own.