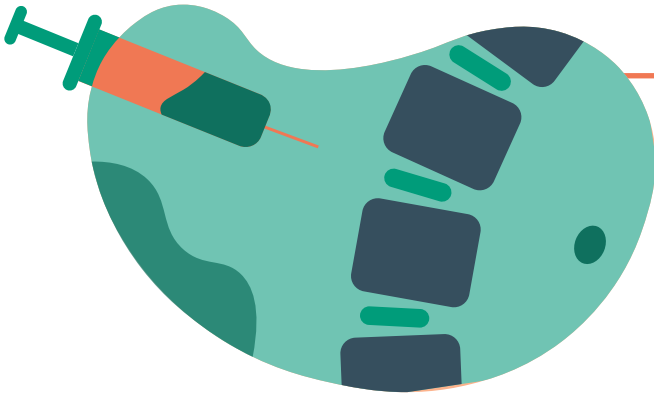


Gene Therapy for SMA

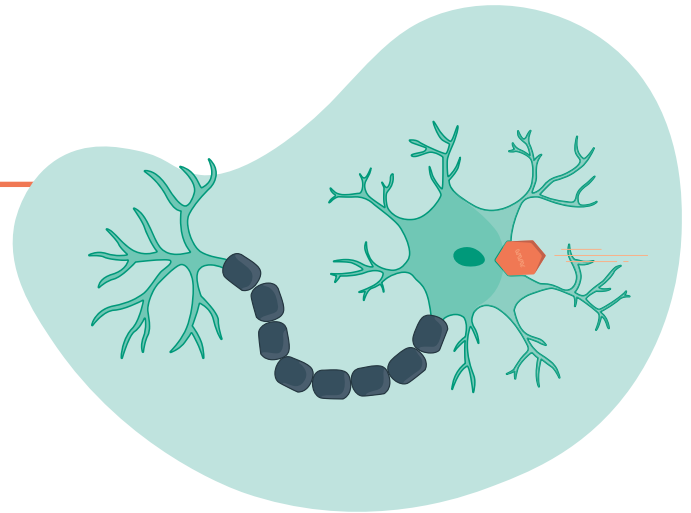


Administering the new gene

Treatment aims to be administered one time to the vein or spinal cord to reach the cells that need the SMN (survival motor neuron) protein that the faulty gene is not producing.

Vector enters motor neuron cells

A vector, which is virus without the parts that cause disease, delivers the new working SMN1 gene into the cells. This can result in the child being able to breathe better, feed themselves and sit unassisted.



Early diagnosis is key

Early diagnosis is key to treating SMA. As time passes the motor neurons are lost and can't be restored. If diagnosis is made early—and the patient is eligible—SMA progression can be minimized.

