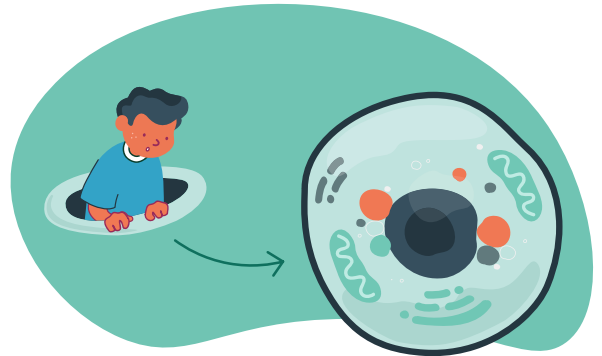


# Ex-Vivo Gene Therapy for Leukodystrophy

*With early diagnosis, this approach targets the cause of the disease – a faulty gene – that leads to the deterioration of myelin sheath that affects our nervous system’s ability to think, move, talk and see.*

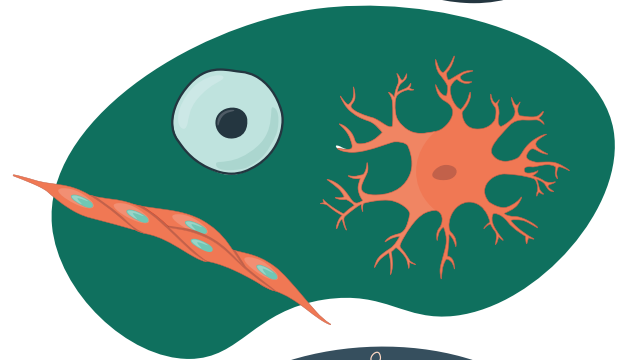
## Remove the Cells

Ex-vivo means outside the body, so the hematopoietic stem cells (HSCs) are removed from the patient via blood draw.



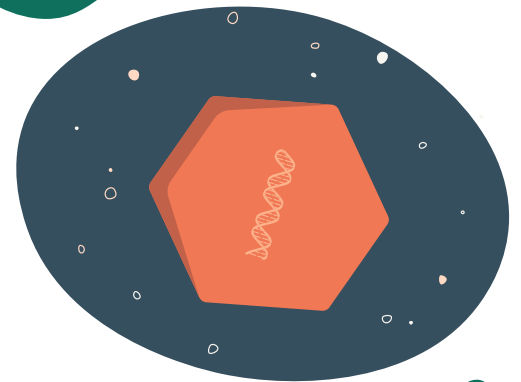
## Use of Stem Cells

Stem cells serve a variety of roles in our body and HSCs are the ones that form our blood. By modifying the HSCs their role is to stop disease progression.



## Modify Stem Cells

Once the HSCs are outside the body, they are modified using a vector, which is a virus without the disease causing parts. The vector delivers new working genes to the cells.



## Return the Cells

The modified cells are returned to the patient to generate more working HSCs in the body that are able to do the work that the cells with faulty genes cannot. The therapy aims to be a one time administration.

