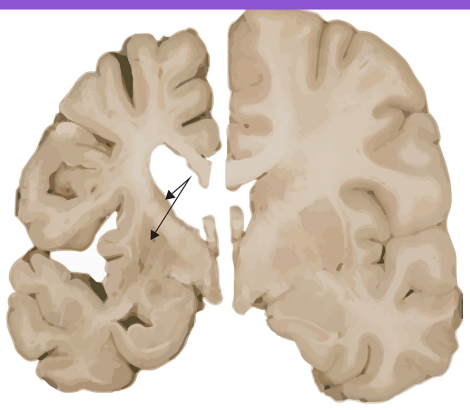
•Huntington's Disease:-

- normaly we have repeated folds of a certive gene [CAGCAGCAG] normally 26
27-36 person wont be affected 36> will effect

↳ these extra repeats will misfold and accamalte ↳cause oxidative stress -> damage the cell and it dies -> increases neuro degenration.



Huntington Disease Brain Normal Brain

Signs:-

•Initial S/S :-

1- Difficulty concentrating.
2- Memory laps-s.
3- Stumbling.
4 - Depression mood swings.

•Progressive S/S :-

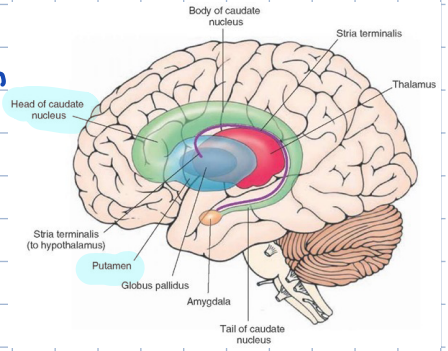
1- in voalntery jerking.
2- muscle problems rilgity.
3- slow eye movment.
4 - difficulty with speach and swallowing.

Available treatments:-

- AMT 130 :-
an experimintal gene therapy targets caudate nucleus and Putamen Parts of the brain.
↳ decreases folding of the protein.

- AMP 130 will be inserted via a needle into those areas of the brain

↳ they will target exon 1 HTT fragment which is the most toxic source of abnormal protein agreggation in HG's.



AMT-130 is done by uniQure