

# Discussing therapeutic objectives and decision making



## Clinical suitability

Scoliosis, AAV 9Ab, liver, renal, cardiac function, full blood count



## Individual Goals

Lived experience and variability, response may not be the same as clinical trials



## How is the treatment delivered

Frequency, route of administration



## Expected outcome

Determinants include age/disease duration, clinical status/motor function, prior therapy



## Risks

There may be higher risk of side effects with combination therapy



## How response will be evaluated after treatment



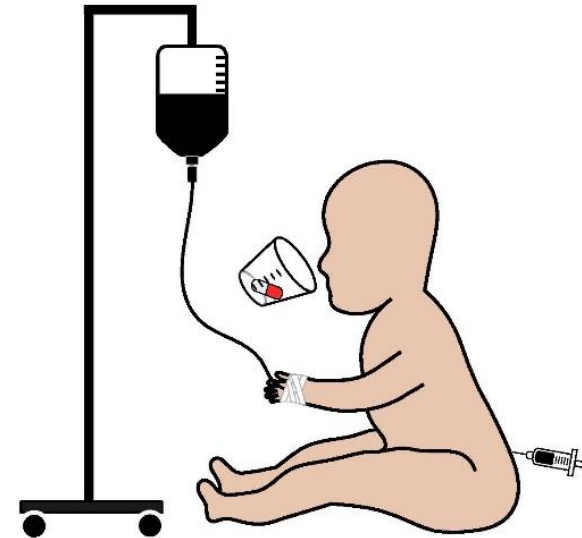
## Requirements for ongoing care

Multidisciplinary care, therapy and monitoring

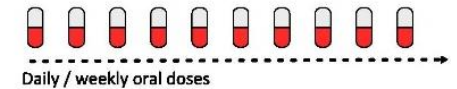


## Cost and access

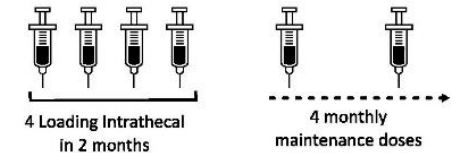
Differs between health systems



## Small molecule splicing modifiers



## Antisense oligonucleotide splicing modifier



## Gene replacement / gene therapy

