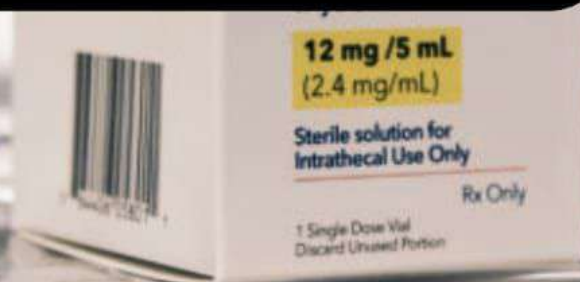


# SPINRAZA



# Definicija

- Spinraza = lijek koji se koristi za liječenje spinalne mišićne atrofije (SMA).
- SMA = genetska bolest koja uzrokuje slabost i propadanje mišića – uključujući i one za disanje.
- Sinonim: nusinersen

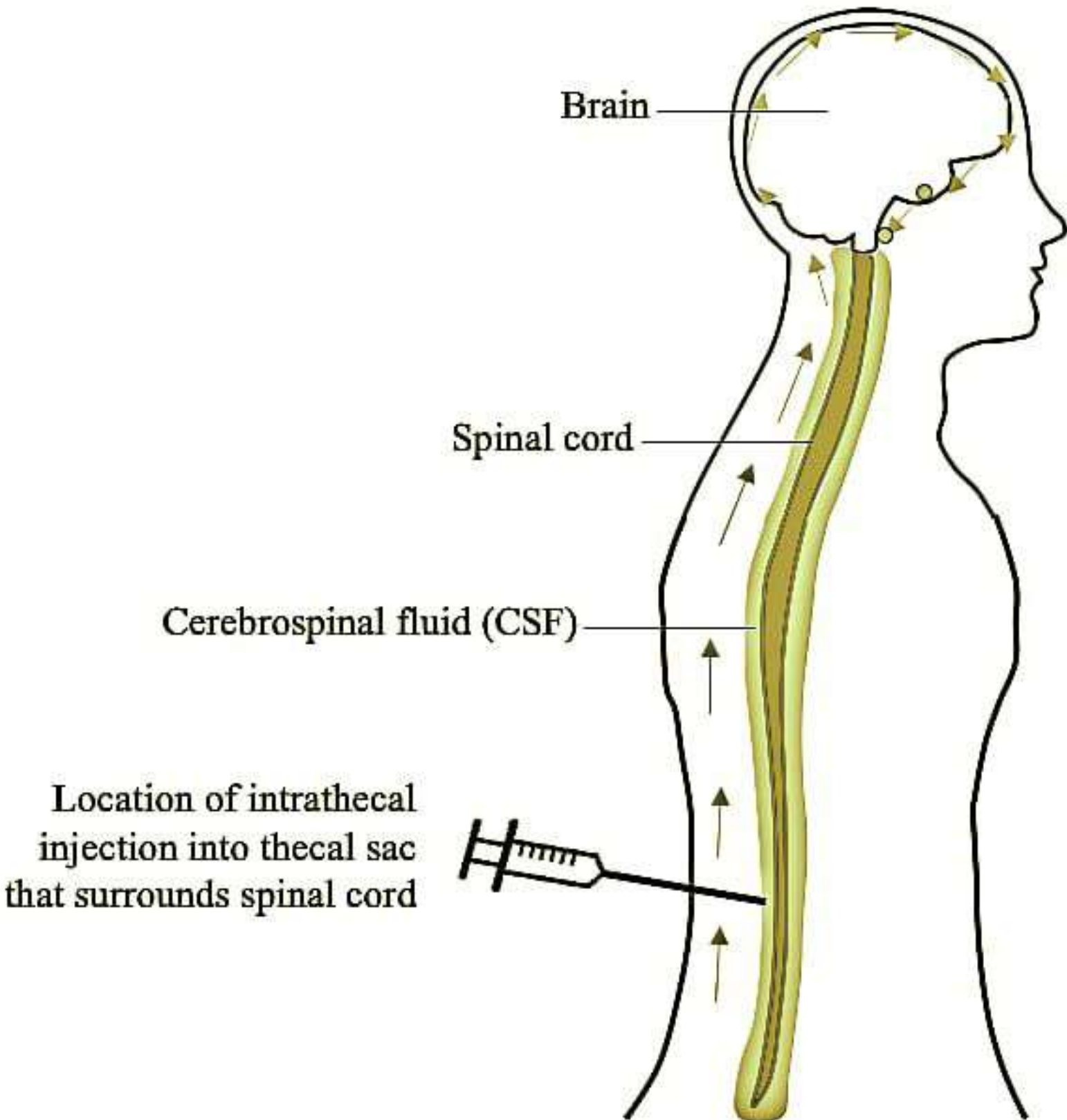


# Mehanizam djelovanja

- Problem kod SMA: najčešće se radi o mutaciji SMN1 gena, a SMN2 gen stvara premalo funkcionalnog SMN proteina u stanici (skraćeno od survival motor neuron), što narušava zdravlje i funkciju motoneurona (živčanih stanica koje kontroliraju mišiće).
  - Spinraza omogućuje SMN2 genu da proizvodi više funkcionalnog SMN proteina.

# Davanje lijeka

- Put davanja: daje se intratekalnom injekcijom odnosno iglom u donji dio leđa u kralježnični kanal.
- Doza: preporučena je doza 12 mg po primjeni.
  - Daje se u 4 udarne doze 0., 14., 28. i 63. dana. Nakon toga je potrebno primjenjivati dozu održavanja jedanput svaka 4 mjeseca.



# Nuspojave

- Moguće nuspojave: infekcije donjih dišnih putova, konstipacija (zatvor), vrućica, glavobolja, povraćanje, bol u leđima, povećani rizik od krvarenja i oštećenja bubrega.

## Literatura:

- <https://www.ema.europa.eu/en/medicines/human/EPAR/spinraza>
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