Gene therapy for the treatment of temporal lobe epilepsy





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ATMP



Neurology

SUMMARY

The project aims at developing a gene therapy for the treatment of drug-resistant focal epilepsy. An adeno-associated viral (AAV) vector will be delivered to the epileptic focus, re-expressing a neuropeptide that will be released in an activity-dependent manner, i.e. in periods of high neuronal activity which precedes the onset of a seizure. Suppression of neuronal excitability thereby suppresses the epileptic event. Strong proof of concept data in mice and rats have supported the feasibility of this strategy. The team is setting up a startup and has acquired follow-up funding to further pursue the strategy and develop the gene therapy for the use in patients.

PROJECT ACHIEVEMENTS DURING & AFTER SPARK

- Patents filed in 2016
- Preclinical Proof-of-concept in vivo and human brain tissue ex vivo in 2016
- Secured GoBio funding of 3.9 Mio. € in 2018 for 3 years
- Science4Life Venture Cup 2021
- GMP production in preparation
- Startup EpiBlok Therapeutics founded in 2022

LONG-TERM GOALS

· Clinical trial phase I