Development of eIF4E ASO treating childhood intractable epilepsy as a first-in-class drug and establishment of R&D environment for RNA therapeutics

Sovargen Co.,Ltd SoVarGen

NEUROSCIENCE	Non-Clinical
Product Type	Anti-sense Oligonucleotides (RNA Therapeutics)
Indication	Control and management of seizures in Focal Cortical Dysplasia type II (FCD T2) patients who are refractory to neuro-surgery
Target	eIF4E
MoA(Mechanism of Action)	 Brain somatic mutations in mTOR pathway (mTORopathy) genes leads to hyperactivation of eIF4E activity, resulting in abnormal protein translation which is causative for epilepsy. SVG102 complementarily binds to eIF4E mRNA, leading to its degradation by RNase H cleavage.
Competitiveness	 First-in-class No available drug for FCD T2 epilepsy patients Neuro-surgery is the only option but 40% of surgery patients still have seizures (refractory to neuro-surgery) Potential for other disease Potential to expand the indication to mTORopathy disease such as Tuberous Sclerosis Complex (TSC) epilepsy patients
Development Stage	Pre-Clinical Stage (US IND filing is planned in 2023)
Route of Administration	Intrathecal administration

