

	Candidate			Discovery	Pre-Clinical	Phase I	Phase II	Phase III	Approved
SCN2A + GOF	Praxis	Elsunersen	ASO					★	
	N-Lorem	nL-SCN2A-002	ASO			●			
SCN2A - LOF	Praxis	PRAX-100	ASO						
	Sangamo	SCN2A-ZFA	Gene Therapy						
	Regel	RT-102	Gene Therapy						
	Tevard	t-RNA	RNA Editing						
	Purdue Univ.	Kv-Gene-Tx	Gene Therapy						
	Purdue Univ.	CRISPR	Gene Therapy						
	Purdue Univ.	CAV-SCN2A	Gene Therapy						
	Univ. of Iowa	t-RNA	RNA Editing						
	UCLA	CRISPR-A	Gene Therapy						
SCN2A +/- MF	Multiple Academic Labs	Retigabine	Small Molecule						
SEIZURES	Praxis	Relutrigene	Small Molecule					★	
	Longboard / Lundbeck	Bexicaserin	Small Molecule						★
	SK Life Sciences	Carisbamate	Small Molecule						★
	Xenon	Azetukalner	Small Molecule						★
	Marinus / Immedica	Ganaxolone	Small Molecule				?		
	Praxis	Vormatrigene	Small Molecule						★
	SK Life Sciences	SKL-24741	Small Molecule				✕		
	UCB	Staccato Alprazolam	Small Molecule						★
	UNC / Univ of Iowa	HDAd-SCN2A-sh5CN2A	Gene Therapy						
	Trevena	TRV045	Small Molecule				?		
	Neurocrine / Xenon	NBI-355	Small Molecule				✕		
	Neurona	NRTX-1001	Cell Therapy					★	
	Rapport	RAP-219	Small Molecule					●	
AUTISM	Astrogen	AST-001	Non-Essential Amino Acid					●	
	Maplight	ML-004	Small Molecule					★	
	Intra-Cellular	Lumateperone	Small Molecule						★
	John Hopkins	Cannabidiol	Small Molecule					★	

Abbreviations: GOF, Gain of Function; MF, Mixed Function; LOF, Loss of Function.

★ Recruiting
 ● Active, not recruiting
 ✓ Not yet recruiting
 ✕ Enrollment Complete
 ? Unknown