	Candidate			Discovery	Pre-Clinical	Phase I	Phase II	Phase III	Approved
DEE-FOCUES EPILEPSY/SEIZURES	Praxis Precision Medicines	Relutrigine	Small Molecule					⊗	
	Lundbeck	Bexicaserin	Small Molecule					⊗	
OTHER EPILEPSY/SEIZURES	Neurocrine Biosciences	NBI-355	Small Molecule			?			
	Xenon	Azetukalner	Small Molecule					★	
	Biohaven	BHV-7000	Small Molecule					⊗	
	SK Life Sciences	Carisbamate	Small Molecule					★	
	UCB	Staccato Alprazolam	Small Molecule					★	
	Praxis Precision Medicines	Vomotrigine	Small Molecule					⬢	
	Mid-Atantic Sleep Center	Multiple candidates	Small Molecule			★	★	★	
	Rapport	RAP-219	Small Molecule					⊗	
	Neurona	NRTX-1001	Cell Therapy					★	
	Boston Children's Hospital	Ketogenic Diet	Dietary Intervention			⊗			
AUTISM SPECTRUM DISORDER BEHAVIORS	Astrogen	AST-001	Non-Essential Amino Acid					⊗	
	Maplight	ML-004	Small Molecule				⊗		
	Intra-Cellular	Lumateperone	Small Molecule					★	
	John Hopkins	Cannabidiol	Small Molecule			★			
	New-York Presbyterian	Propranolol	Small Molecule				★		

Candidate			GoF +	LoF -	MF +/-	Discovery	Pre-Clinical	Phase I	Phase II	Phase III	Approved
ANTISENSE OLIGONUCLEOTIDE (ASO)	Praxis Precision Medicines	Elsunersen								★	
	ARIA	Multiple candidates		✓	✓						
	Mahzi Therapeutics	Multiple candidates	✓								
	Praxis Precision Medicines	PRAX-100		✓							
	Undisclosed	Multiple candidates	Undisclosed								
GENE THERAPY	Purdue University	CRISPR		✓							
	Regel Therapeutics	RT-102		✓							
	Undisclosed	Multiple candidates	Undisclosed								
N OF 1	N-Lorem	Multiple candidates	✓	✓	✓					⬢	

Abbreviations: ARIA, Aligning Research to Impact Autism; DEE, Developmental and Epileptic Encephalopathy; GoF, Gain of Function; MF, Mixed Function; LoF, Loss of Function.

★ Recruiting ⬢ Active, not recruiting ✓ Not yet recruiting ⊗ Enrollment Complete ? Unknown