Deep and Diverse Pipeline Across CNS and Rare Disease

PROGRAM	INDICATION	PROPOSED MECHANISM OF ACTION	DISCOVERY	IND ENABLING	PHASE 1	PHASE 2	PHASE 3	LAUNCHED
CNS								
NUPLAZID [1]	Parkinson's Disease Psychosis	*5HT2A inverse agonist and antagonist*						
ACP-204 [4]	Alzheimer's Disease Psychosis	*New 5HT2A inverse agonist*						
ACP-204 [4]	Lewy Body Dementia w/ Psychosis	*New 5HT2A inverse agonist*						
ACP-711 [4]	Essential Tremor	*Selective $GABA_A$-α3 modulator*						
RARE DISEASE								
DAYBUE [2]	Rett Syndrome	*Analogue of GPE*						
ACP-101 [3 4]	Hyperphagia in Prader-Willi Syndrome	*Intranasal Carbetocin*						
ACP-2591 [4]	Rett Syndrome; Fragile X Syndrome	*cGP analogue*						
STOKE ASO 1 [4 6]	Rett Syndrome	*Antisense oligonucleotide (ASO)*						
STOKE ASO 2 [4 6]	SYNGAP1	*Antisense oligonucleotide (ASO)*						
STOKE ASO 3 [4 6]	Not disclosed	*Antisense oligonucleotide (ASO)*						
CNS/RARE DISEASE								
ACP-211 [4]	TRD/MDD/Other	*NMDA receptor antagonist*						
ACP-271 [4]	Neurology	*GPR88 agonist*						

+ Multiple undisclosed discovery programs in CNS and rare disease

[1] NUPLAZID (pimavanserin) is only approved in the U.S. by the FDA for the treatment of hallucinations and delusions associated with Parkinson's disease psychosis.
[2] Acadia has an exclusive license to develop and commercialize trofinetide worldwide from Neuren Pharmaceuticals. DAYBUE (trofinetide) is only approved in the U.S. by the FDA and in Canada by Health Canada for the treatment of Rett syndrome in adults and pediatric patients two years of age and older.
[3] Acadia acquired Levo Therapeutics and its rights/licenses to ACP-101.
[4] Investigational agents, for which the safety and efficacy of these agents have not been established. There is no guarantee these investigational agents will be filed with or approved by any regulatory agency.
[5] Acadia entered into an exclusive worldwide license agreement with Saniona for the development and commercialization of ACP-711.
[6] Acadia entered into a collaboration with Stoke Therapeutics to discover, develop and commercialize novel RNA-based medicines for the potential treatment of severe and rare genetic neurodevelopmental diseases; ASO = Antisense oligonucleotide.

Updated January 14, 2025; Acadia disclaims any duty to update.