



CPP-115 Progress in Refractory Infantile Spasms

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AEDD Trials XIV



CPP-115, A Novel GABA-AT Inhibitor

- A new molecular entity
 - Analog of Vigabatrin
 - Under development for treatment of refractory IS
 - Other potential uses
- Designed to be safer and more potent
 - Enhanced potency, specificity, and safety
 - Animal testing shows better visual safety than Vigabatrin
 - Epilepsy animal models show enhanced performance
 - Conducted in NIH's Anticonvulsant Screening Program
 - Testing in IS animal model confirms superior tolerability and efficacy
 - Epilepsia, 55(1):94-102, 2014
- Successful use in refractory IS patient
- Currently in Phase 1 development
- Drug available for emergency use

CPP-115 Anticonvulsant Screening

Animal Model	CPP-115	Vigabatrin
6 Hz Minimal Clonic Seizure	ED ₅₀ =5.2 mg/kg	ED ₅₀ =23.4 mg/kg
6 Hz Minimal Clonic Seizure (higher current)	ED ₅₀ =28.4 mg/kg	ED ₅₀ =153.5 mg/kg
Corneal Kindling	ED ₅₀ =20.1 mg/kg	ED ₅₀ =80.4 mg/kg
Maximal Electroshock	ED ₅₀ =58.9 mg/kg	ED ₅₀ >1000 mg/kg
Subcutaneous Picrotoxin	ED ₅₀ =60.7 mg/kg	Not Available

- CPP-115 was effective in the MES model
 - “Gold Standard” model for anticonvulsant screening
 - Vigabatrin does not work in this model
- Where vigabatrin is effective, CPP-115 is also effective and substantially more potent
- Data provided by NIH’s Anticonvulsant Screening Program
 - A variety of data from other models is available

Development Status

- Completed Phase I enabling toxicology
 - Not metabolized, renally eliminated, with linear dose related PK
- Single dose safety
 - No significant adverse events up to 500 mg single dose
- Multiple dose safety
 - Approximately doubles brain GABA
 - Somnolence observed after about a week of treatment
 - Additional phase 1 studies needed to optimize dose and dose titration
 - Like vigabatrin, CPP-115 will require an escalating titration to an effective dose in order to minimize somnolence effects

Clinical Use of CPP-115 in IS

- Emergency IND at NYU
 - Dr. Orin Devinsky
 - Treating IS case refractory to other treatments
 - Last treatment was vigabatrin (unsuccessful)
 - Treated with CPP-115
 - First as a combination therapy (vigabatrin and CPP-115)
 - Now on CPP-115 1.2 mg/kg/day and clobazam 0.4 mg/kg/day
 - Treatment for >2 years
 - No seizure activity on most recent EEG.
 - *Epilepsy and Behavior Case Reports 6 (2016) 67-69.*



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