Cost-Utility Analysis of Add-on Stiripentol vs Cannabidiol and Fenfluramine in Dravet Syndrome in the USA

Georgie Weston[1], Yunni Yi[1], Deepak Alexander[1], Marie Fourcroy[2], Benjamin Serraz[2], Carla Schad[3], Caroline Marre[2].

[1] Adelphi Values Ltd, Bollington, UK; [2] Biocodex FR, Gentilly, France; [3] Biocodex USA

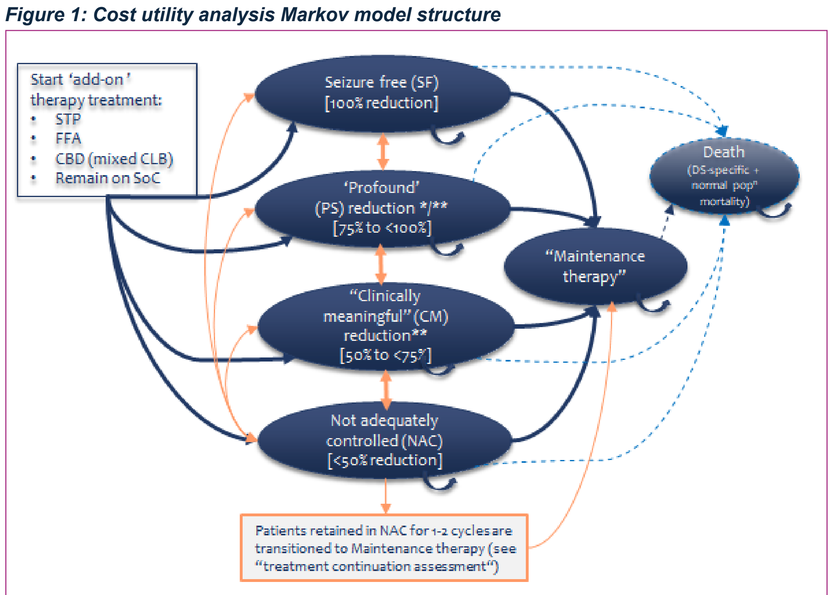
INTRODUCTION

- Dravet syndrome (DS) is a rare epileptic encephalopathy characterised by frequent (often daily) convulsive seizures, 'comorbidities' and premature mortality [1,2].
- Arising in the first years of life, the severe treatment-resistant seizures are associated with a progressive decline in behavioral, motor and cognitive function ('comorbidities') [1,3].
- An estimated 15-20% of children with DS die from status epilepticus (SE), sudden unexpected death in epilepsy (SUDEP), and accidents before reaching adulthood [2].
- The daily burden of DS significantly impacts the quality of life (QoL) of patients, as well as their carers and the broader family members [3].
- Reducing convulsive seizure frequency is a key goal of treatment to decrease the risks of morbidity and mortality, improve QoL, and lower healthcare system expenditure [1,3,7].
- Despite the use of combination standard of care (SoC) anti-seizure medications (ASMs), seizures are often intractable and sustained periods of seizure freedom [SF] is rarely possible.
- 'Add-on' therapies to background SoC ASMs are therefore often required to improve seizure control. Stiripentol (STP, DIACOMIT®) [4,] pharmaceutical grade cannabidiol (CBD, EPIDIOLEX®) [5], and fenfluramine (FFA, FINTEPLA®) [6] are specifically licensed for DS in the USA, for use from ages 6 months (≥7kg body weight), 1-year, and 2-years, respectively.
- A recent US-led international consensus-based DS treatment algorithm positions STP and FFA ahead of CBD in the treatment pathway [7]. Several network meta-analyses (NMA) of RCT data support this positioning on a clinical basis [8-9]. However, no published economic analyses have evaluated the comparative cost-effectiveness of all three licensed add-on therapies.
- This study assessed the cost-effectiveness (utility) of initiating STP versus CBD, or FFA, or continued background ASM therapy in DS patients, from a US healthcare payer perspective.

METHODS

- Informed by a systematic literature review identifying economic evaluations of STP (n=5), CBD (n=3), and FFA (n=3), a Markov model was developed in Microsoft Excel®
- The model simulated a cohort of DS patients requiring a first-line add-on therapy to their existing background ASMs (comprising valproate [VPA] and clobazam [CLB]).
- In base case analyses, patients aged 2 years old (the earliest common age patients could receive all three add-on therapies [4-6]) entered the model and were assigned to either continue receiving their background ASMs, or receive add-on STP, CBD, or FFA.
- Patients were initially distributed across health states based on risk differences versus STP in the proportion of patients achieving each respective level of seizure reduction as derived from a published NMA [9] (Table 1).
- In subsequent 3-monthly cycles, patients could then transition between the 6 health states defined by: a percentage reduction from baseline in monthly convulsive seizures (MCSF); receiving Maintenance Therapy (add on therapy removed; background ASMs only), or death (see Figure 1).
- Probabilities for the subsequent transitions between health states, discontinuation of add-on therapies for adverse events, and SoC ASMs usage and doses during "Maintenance therapy", were derived from patient-level analyses of the DIAVEY[10] real-world observational study of STP and applied equally to all model arms (Table 1).
- Patients failing to achieve a ≥50% reduction in MCSF within 3 months (1 cycle) of starting treatment were assumed to discontinue add-on therapy and transition to the "Maintenance therapy" health state. Similarly, patients residing in the "Not adequately controlled" [NAC] health state for 6 months (2 cycles) also transitioned to "Maintenance therapy" (Figure 1).

Figure 1: Cost utility analysis Markov model structure



CBD, Cannabidiol; CM, clinically meaningful seizure reduction state; DS, Dravet syndrome; FFA, fenfluramine; NAC, not adequately controlled; PS, profound seizure reduction state; SF, seizure free state; SoC, background ASMs; STP, stiripentol.

Health states:

- "Seizure Free" [SF] = 100% reduction from baseline in monthly convulsive seizure frequency (MCSF);
- "Profound Seizure" [PS] reduction = 75 to <100% in MCSF;
- "Clinically Meaningful" [CM] reduction = 50 to <75% in MCSF;
- "Not Adequately Controlled" [NAC] =<50% reduction in MCSF;
- "Maintenance Therapy" (add-on therapy removed; background ASMs only)
- "Death" (absorbing health state).

At each cycle (3 months), patients transition freely between SF, PS, CM and NAC health states. Arrows represent possible movements between health states.

- Probabilities for the subsequent transitions between health states, discontinuation of add-on therapies for adverse events, and SoC ASMs usage and doses during "Maintenance therapy", were derived from patient-level analyses of the DIAVEY[10] real-world observational study of STP and applied equally to all model arms (Table 1).
- Patient failing to achieve a ≥50% reduction in MCSF within 3 months (1 cycle) of starting treatment were assumed to discontinue add-on therapy and transition to the "Maintenance therapy" health state. Similarly, patients residing in the "Not adequately controlled" [NAC] health state for 6 months (2 cycles) also transitioned to "Maintenance therapy" (Figure 1).
- Seizure events are associated with an increased risk of premature mortality [1,2]. As RCTs cannot feasibly be powered to provide mortality data in DS, no treatment-related differences in mortality were modelled. A DS-specific mortality rate [2] was applied to US general population life tables [12] and equally applied to all modelled arms (Table 1).
- DS-specific utility values and resource use were obtained from 3 studies [11] identified in a SLR (Table 1). Average utility values from the 3 studies were mapped to the "Maintenance therapy" health state, and proportionally adjusted for "seizure free" [SF], "profound seizure" [PS] reduction, "clinically meaningful" [CM] seizure reduction, and NAC health states based on the relative % reduction in MCSF.
- Drug costs were calculated on an average cost per mg basis using commercially available bottle/pack sizes and formulations; and January 2024 wholesale acquisition costs (WACs) [13]. In the base case analyses, real-world add-on drug doses [16, 17] were used (Table 1). Dispensing fees, co-payments, co-insurance and rebates for all drugs were pragmatically assumed to be zero.
- Other direct costs and medical resource use associated with seizure events and no seizure events were derived from a published database analysis of 989 Medicaid and commercially insured DS patients in the US between 2010 and 2015 and inflated to 2023 values [14] (Table 1). The costs of periodic echocardiogram monitoring for FFA, per license requirements [6], were also included.
- Accident, SE and adverse events were implicitly assumed to be captured in seizure-event resource use, costs, and utility values within health states; alongside routine treatment appointments.
- Base case analyses adopted a 15-year time horizon and a US commercial insurer (payer) perspective in 2024. Costs and QALYs were discounted at 3% per year and varied (0-6%) in sensitivity analyses [15].
- Results were presented as ICERs (incremental costs per quality-adjusted life year [QALY] gained) and NMBs (incremental net monetary benefits) at the Institute for Clinical and Economic Review organization (ICERO) willingness-to-pay (WTP) threshold of $200,000/QALY [15] (Table 2). A positive NMB indicates a cost-effective therapy option at a given WTP threshold. **The add-on therapy with the highest NMB is considered the most cost-effective (economically preferred) therapy.**
- Base case parameter values subject to uncertainty were varied within their 95% confidence range (or else +/-20%) in one-way sensitivity analyses (OWSA). Combined uncertainty was assessed using probabilistic sensitivity analyses (PSA: 1,000 Monte Carlo simulations).

Table 1. Cost utility analysis model parameters

Parameter	Base case value					Source
Patient population						
Patient age; weight	2 years; 12.34kg					Centers for Disease Control & Prevention [12]
Efficacy - First cycle health state probabilities						
	SF	***PS***	***CM***	***NAC***	***Maint.***	Guerrini et al 2024 [9]
STP	36.36%	18.18%	15.15%	30.30%	0.00%	
SoC ASMs	0.00%	3.23%	3.23%	93.55%	0.00%	
FFA	10.36%	38.18%	20.15%	31.30%	0.00%	
CBD*	3.36%	10.64%	12.12%	73.88%	0.00%	
Efficacy - Subsequent cycle transition probabilities (from row to column health states)						
	SF	***PS***	***CM***	***NAC***	***Maint.***	DIAVEY study [10]
SF	92.05%	1.01%	0.94%	5.04%	0.96%	
PS	1.35%	92.27%	3.36%	2.07%	0.96%	
CM	0.97%	3.40%	88.36%	6.30%	0.96%	
NAC	0.66%	1.02%	2.04%	95.32%	0.96%	
Maint.	0.00%	0.00%	0.00%	0.00%	100.00%	
Mortality						
DS Mortality, probability/year	1.45%					Cooper et al 2016 [2]
Drug therapy dosing and costs						
Add-on therapy dosing	*Age <16 years*		*Age 16 to <18 years*		*Age 18+ years*	
STP (mg/kg/day)	50		34		25	Balestrini et al 2022; Chiron et al 2018 [16]
CBD (mg/kg/day)	15		15		15	Scheffer et al 2021 [17]; D'Onforio et al 2020 [17]
FFA (mg/kg/day)	0.44		0.44		0.44	Sullivan et al 2020 [17]
Add-on therapy WAC, costs per mg (US$/mg)						
STP	$0.1251					Analysource® [13]
CBD	$0.1625					Micromedex® Red Book [13]
FFA	$24.3960					
SoC ASM dosing & WAC	***Dosing (mg/kg/day)***		***Costs per mg (US$/mg)*****			
VPA	25.2		$0.0038			STICLO trials [16]; Red Book [13]
CLB	0.5		$0.9093			
Maintenance therapy dosing and WAC	***Dosing (mg/kg/day)***		***Costs per mg (US$/mg)*****		***Proportion of patients on treatment***	
VPA	42.50		$0.0038		70.97%	DIAVEY study [10]; Red Book [13],
CLB	1.25		$0.9093		35.48%	
TOP	10.00		$0.0875		38.71%	
LEV	70.00		$0.0078		9.68%	
CLON	0.20		$2.9698		19.35%	
ETHO	30.00		$0.0036		12.90%	
ZON	7.00		$0.1237		12.90%	
Health state utility values						
SF	0.760					Average of Lo et al. 2021; Auvin et al. 2021; Radu et al. 2019[11]
PS	0.567					
CM	0.350					
NAC	0.341					
Maint.	0.341					
Death	0.000					Assumption
Direct resource use and costs						
Units of resource use by health state per year	***SF***	***PS***	***CM***	***NAC***	***Maint.***	
Inpatient admissions	0.2	0.5	0.5	0.8	0.8	Reaven et al 2019 [14]
ED visit	0.2	1.0	1.0	1.8	1.8	
Hospital OP visit	6.1	7.0	7.0	8.0	8.0	
Physician visit	9.2	10.7	10.7	12.4	12.4	
Other OP visit	7.3	9.1	9.1	11.1	11.1	
Home health	5.6	5.7	5.7	5.7	5.7	
Equipment/ supply	1.1	1.0	1.0	0.8	0.8	
Rescue drugs	0.5	1.0	1.0	1.5	1.5	
Other drugs	14.8	15.0	15.0	15.1	15.1	
Costs per unit of resource use by health state (2023 US$)	***SF***	***PS***	***CM***	***NAC***	***Maint.***	
Inpatient admissions	$15,031	$24,844	$24,844	$28,451	$28,451	Reaven et al 2019, inflated to 2023 prices [14]
ED visit	$1,701	$2,074	$2,074	$2,210	$2,210	
Hospital OP visit	$876	$944	$944	$999	$999	
Physician visit	$213	$231	$231	$244	$244	
Other OP visit	$202	$229	$229	$247	$247	
Home health	$417	$309	$309	$199	$199	
Equipment/ supply	$592	$502	$502	$421	$421	
Rescue drugs	$465	$591	$591	$657	$657	
Other drugs	$185	$184	$184	$184	$184	
Other features						
Time horizon	15 years					Assumption
Discount rate	3% costs and outcomes					ICRO [15]
Initial treatment continuation assessment	6 months					Per previous NICE HTAs of CBD and FFA [18].

ASMs, antiseizure medications; CBD, cannabidiol; CLB, clobazam; CLON, clonazepam; CM, clinically meaningful seizure reduction state; ED, emergency department; ETHO, ethosuximide; FFA, fenfluramine; HTAs, health technology assessments; LEV, levetiracetam; Maint., maintenance therapy state; NAC, not adequately controlled state; OP, outpatient; PS, profound seizure reduction state ; SF, seizure free state; SoC, standard of care; STP, stiripentol; TOP, topiramate; US, United States; VPA, valproate; WAC, wholesale acquisition costs; ZON, zonisamide

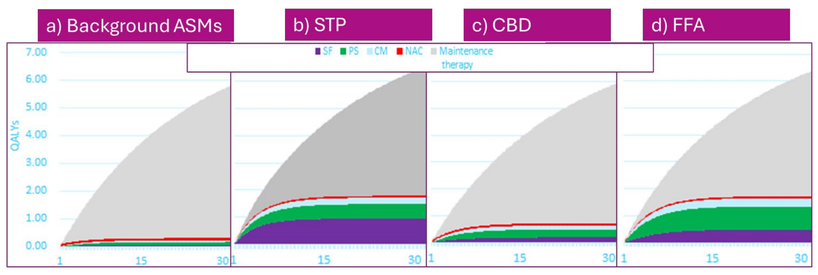
* For CBD 15mg/kg/day used in the base case model, a mean average of the matrices for the CBD 10mg/kg/day and 20mg/kg/day is used. For the scenario analysis using maximum doses, only the CBD 20mg/kg/day matrix is used (data not shown).

** Costs per mg rounded to 4 decimal places in this table (with exception of VPA). Unrounded figures used in model.

RESULTS

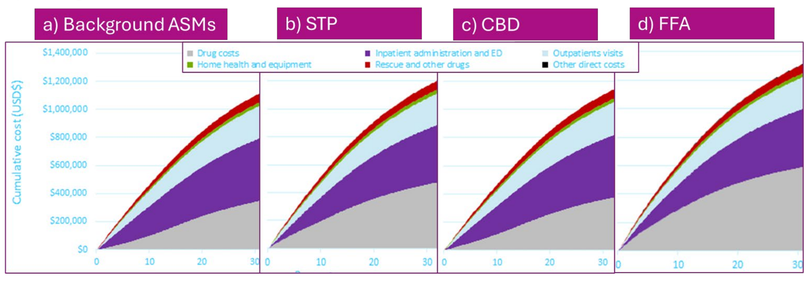
- In base case analyses, vs continued background SoC ASMs, STP generated the greatest QALY gains (+0.66), followed by FFA (+0.53) and CBD (+0.15) (Table 2, Figure 2).

Figure 2. Cumulative QALYs gained over time (30 year time horizon) for a) background ASMs, b) STP, c) CBD and d) FFA



Years from start of model

Figure 3. Cumulative costs ($USD) over time (30 year time horizon) for a) background ASMs, b) STP, c) CBD and d) FFA



Years from start of model

- NMBs were greatest with STP ($67,511), followed by CBD ($24,825), and then FFA (–$76,830) (Table 2). STP economically dominated FFA, generating more QALYs (+0.13) and costing (-$118,676) less (Table 2). STP was cost effective versus CBD, with an ICER of $116,448/QALY.
- In OWSA, the most influential parameters on the base case ICER results were the patient weight, and direct healthcare resource use costs in "Maintenance therapy". For all parameters explored, ICERs for STP and CBD versus continued background SoC ASMs, remained below the WTP threshold. All ICERs for FFA exceeded the threshold.

Table 2. Base case cost utility analysis results

		vs background ASM				vs next non-dominated best therapy*
Ranked treatments by QALYs (best to worst)	**Cost, $US**	**QALY**	**Incr. Cost ($)**	**Incr. QALY**	**ICER ($/QALY)**	**NMB ($) at $200k/QALY WTP**	**Fully incremental ICER ($/QALY)**
STP+ VPA+CLB	$729,107	4.47	$63,986	0.66	$97,319/ QALY	$67,511	$116,488/QALY (vs CBD+VPA + CLB)
FFA+ VPA+CLB	$847,783	4.34	$182,661	0.53	$345,195/ QALY	-$76,830	DOMINATED BY STP*
CBD+ VPA+CLB	$669,565	3.96	$4,443	0.15	$30,363/ QALY	$24,825	$30,363/QALY (vs VPA+CLB)
VPA+CLB (background ASM)	$665,122	3.81	-	-	-	-	-

ASM, antiseizure medication; CBD, cannabidiol; CLB, clobazam; FFA, fenfluramine; Incr., incremental; ICER, incremental cost effectiveness ratio (i.e., incremental cost per QALY gained); NMB, incremental net monetary benefits; QALY, quality-adjusted life year; STP, stiripentol; VPA, valproate sodium; WTP, willingness to pay

* STP dominates FFA as is both more effective and less costly than FFA. In rank order of QALYs gained, the fully incremental analyses therefore compares: background ASMs (VPA+CLB) vs CBD+VPA+CLB and then the "next non-dominated best therapy" CBD+VPA+CLB vs STP+VPA+CLB.

Note: This table presents rounded figure. Calculations to derive the reported NMBs and ICERs use unrounded figures.

- In PSA, probabilistic mean ICERs versus background SoC ASMs were similar to the deterministic base case ICERs. The probability that STP and CBD had ICERs <$200,000/QALY exceeded 95%, whereas FFA had a probability <5%. The probabilities that STP was cost effective versus CBD and FFA were 97% and 100%, respectively.
- In scenario analyses, including: initiation at age 6 months or in adults; longer or shorter time horizons; use of maximum licensed dosing; and adoption of a societal perspective, STP was economically preferred. STP was the only add-on therapy with a positive NMB when initiated in adults. STP retained the highest NMB at a WTP threshold of $150,000/QALY used by ICERO[15] to benchmark value-based prices (Table 3).

Table 3. Cost-utility analysis scenario analyses – Net monetary benefit (NMB)

Scenario	Base case	Scenario	NMB* ($) vs background ASMs		
A positive NMB indicates a cost-effective therapy option at a given WTP threshold			**STP**	**FFA**	**CBD**
Base case			**$67,511**	-$76,830	$24,825
Patient age at add-on initiation [4-6]	2 years	STP=6mths; CBD=12mths†; FFA=24mths‡	**$95,159**	-$60,363	$29,038
		18 years	**$1,127**	-$538,831	-$25,009
Time horizon [15]	15 years	5 years	**$72,647**	-$17,620	$19,315
		30 years	**$66,726**	-$90,710	$24,636
Payer type [14]	Commercial	Medicaid	**$16,563**	-$121,710	$11,326
Dosing approach [4-6,16, 17]	RW doses for all add-on therapies	Alternative STP RW doses (US) : - Pediatric: 27.58 mg/kg/day - Age16-<18yrs: 23.80 mg/kg/day - Adult: 15.20 mg/kg/day [23]	**$126,831**	-$76,830	$24,825
		Lowest RW doses for all add-on therapies: - STP: as above (US) [23] - CBD: 10 mg/kg/day [25] - FFA: 0.39 mg/kg/day [29]	**$126,831**	-$48,912	$32,917
		Maximum licensed doses for all add-on therapies: - STP: 50 mg/kg/day - CBD: 20 mg/kg/day - FFA: 0.7 mg/kg/day	**$66,767**	-$208,991	$17,127
Treatment continuation assessment [18]	3 mths	6 months	**$54,449**	-$96,351	$18,779
Perspective [14]	Healthcare payer	Societal	**$139,611**	-$21,060	$41,656
WTP threshold [15]	$200,000 /QALY	$150,000/QALY	**$34,637**	-$103,288	$17,508
		$100,000/QALY	$1,763	-$129,746	**$10,191**
		$50,000/QALY	-$31,111	-$156,204	**$2,874**

ASMs, antiseizure medications; CBD, cannabidiol; FFA, fenfluramine; mths, months; NMB, net monetary benefits; QALY, quality-adjusted life year; RW, real world; SoC ASMs, standard of care ASMs (i.e., valproate plus clobazam); STP, stiripentol; WTP, willingness to pay.

*Using a WTP threshold of $200,000/QALY unless otherwise indicated [15]

†Assumes background ASMs for 6 months and initiation of CBD at age 12 months, per its label [5]

‡ Assumes background ASMs for 18 months and initiation of FFA at age 24 months, per its label [6]

Note: **Bold text indicates the economically preferred treatment for a given scenario based on NMB and the given WTP threshold**

DISCUSSION

- To our knowledge, this is the first study to assess the cost-effectiveness of all three currently licensed add-on therapies (STP, CBD and FFAs) in the management of seizures in DS.
- At the ICERO WTP threshold of up to $200,000/QALY[15], STP was the economically preferred add-on therapy option, followed by CBD. FFA was economically dominated by (i.e., was more costly and less effective than) STP and found not to be cost-effective at this WTP threshold compared with continued background SoC ASMs (Table 2).
- Results were robust to sensitivity analysis and conclusions on the relative cost-effectiveness of the add-on therapies were consistent across a range of scenario analyses (Table 3).
- Both STP and CBD had base case ICERs versus background SoC ASMs below ICERO WTP thresholds of $200,000/QALY and $150,000/QALY[15]. Since the primary goals of DS treatment are to reduce the frequency of seizures as far as possible, the lower ICER (and WAC) of CBD should be considered in the broader clinical context of lower overall health gains within acceptable WTP thresholds.
- STP, as the most effective add-on therapy option, with the greatest NMB within the defined WTP thresholds, would, on average, be the clinically and economically preferred add-on therapy option.
- This cost-utility analysis supports the US-led consensus-based treatment algorithm that positions STP as a first-line add-on therapy option ahead of CBD in the treatment pathway but indicates that, at January 2024 WACs, use of FFA as a first-line add-on therapy is not supported on an economic basis.
- Whilst the results of this cost utility analysis are likely to be applicable on average in a US health care setting, they should not be the sole basis of population-level or individual patient decision-making. Seizures in DS are often very difficult to manage, necessitating a range of therapy options to be available and tailored to the specific needs of each patient at the time.

CONCLUSION

- **STP is a cost-effective add-on therapy compared with CBD or FFA and background SoC ASMs.**
- **In addition to its robust clinical evidence, these health economic data further support the utilization of STP as a first-line add-on therapy for treatment of seizures in DS.**

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