

Sodium Valproate in McArdle disease

Dr Renata Scalco
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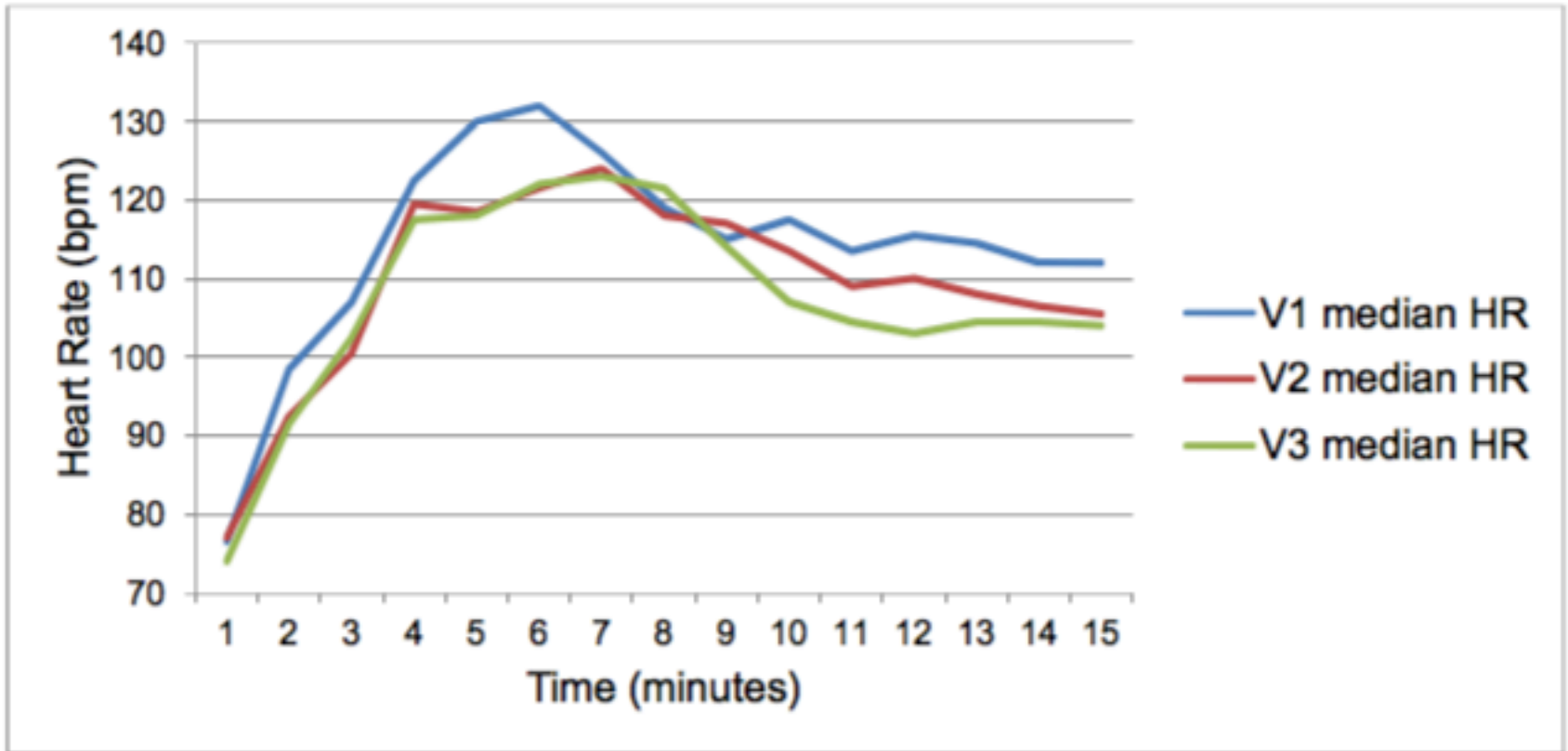
- 20 mg/kg/day sodium valproate was **ineffective** in stimulating the expression of the brain glycogen phosphorylase enzyme in skeletal muscle of people with McArdle disease
- **Next Step:** Further research into sodium valproate as a treatment option for McArdle disease is discouraged

Outcome Measures

After 6-months treatment with valproate:

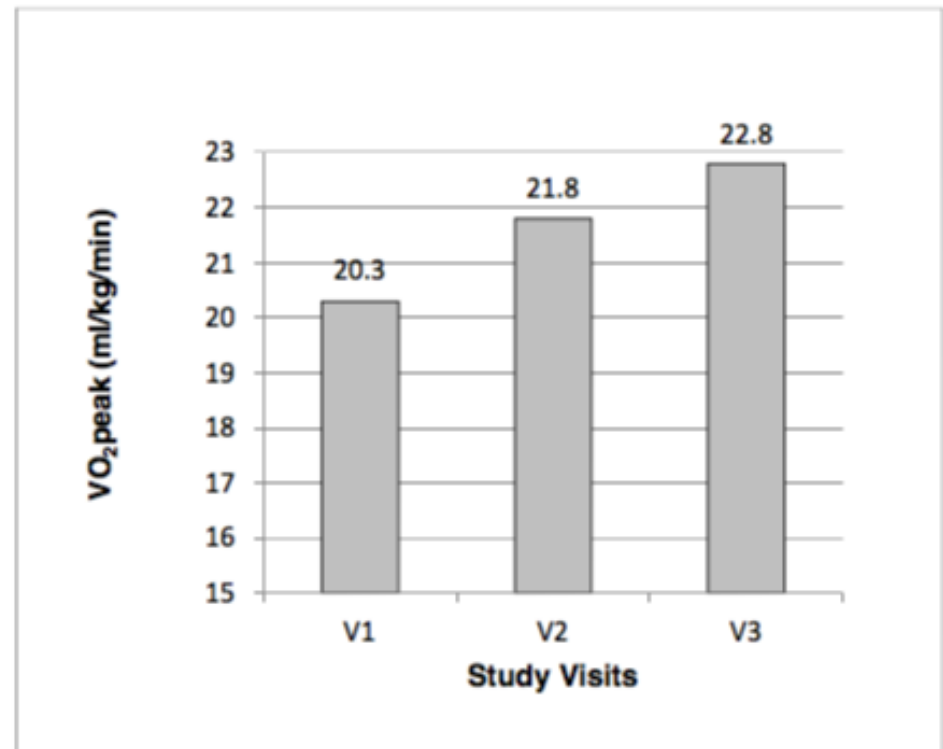
- 12-minute walk test: no significant improvement in walking distance
- Cycle test: second wind present in all visits – valproate did not reduce the second wind
- Forearm test: no lactate increase
- Biopsy: no enzyme activity

Second Wind



But...

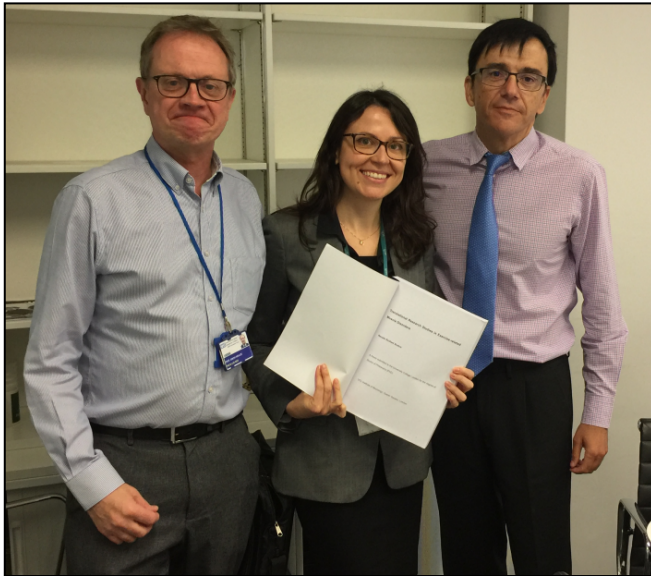
- VO₂peak – aerobic power: **IMPROVED!!!**
- Improvement of almost 13%
- Theory: ? People exercised more while on treatment (Placebo effect) – more confident to exercise more vigorously / regular
- Message: being fit is the best treatment for McArdle Disease



THANK YOU ALL!!!

PhD Completion

- Dr Renata Scalco



“Thank you all for the support and inspiration. I will miss the UK experience forever. With all the best wishes, (and I do hope to see you soon)

Renata”

