

Table S1. Treatment groups for compound A efficacy trials

	Strain	Group	Treatment
Study 1 Treatment from day 18 postnatal age until 8 weeks of age	<i>mdx</i>	VEH	Oral cherry syrup
			Intraperitoneal water
	<i>mdx</i>	PNSL5.0	Oral prednisolone 5.0 mg/kg/day (in cherry syrup)
			Intraperitoneal water
	<i>mdx</i>	CpdA3.75	Oral cherry syrup
			Intraperitoneal CpdA 3.75 mg/kg/day
	<i>mdx</i>	CpdA7.5	Oral cherry syrup
			Intraperitoneal CpdA 7.5 mg/kg/day
	WT	VEH	Oral cherry syrup
			Intraperitoneal water
	WT	PNSL5.0	Oral prednisolone 5.0 mg/kg/day (in cherry syrup)
			Intraperitoneal water
WT	CpdA7.5	Oral cherry syrup	
		Intraperitoneal CpdA 7.5 mg/kg/day	

Study 2 [Ⓞ]	<i>mdx</i>	VEH	Oral cherry syrup Intraperitoneal water
Treatment from week 4 postnatal age until 8 weeks of age	<i>mdx</i>	PNSL5.0	Oral Prednisolone 5.0 mg/kg/day (in cherry syrup) Intraperitoneal water
	<i>mdx</i>	CpdA7.5	Oral cherry syrup Intraperitoneal CpdA 7.5 mg/kg/day

Study 1, *n* = 15/group, once-daily dosing; Study 2, *n* = 10/group, once-daily dosing.

[Ⓞ]Study 2 was performed in the context of a validation study comparing oral CpdA at 2.5 mg/kg/day, 5.0 mg/kg/day and 7.5 mg/kg/day with intraperitoneal CpdA at 7.5 mg/kg/day. Outcome measures were limited to H&E analysis and cytokine protein levels in the gastrocnemius muscles. This study was performed in order to confirm bioactivity of intraperitoneal CpdA prior to the conduct of a more comprehensive study (Study 1).