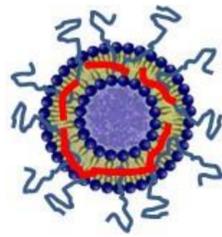


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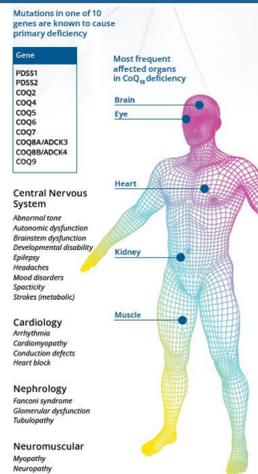
I. BPM31510

- Proprietary & **stable** formulation
- Contains oxidized (active) CoQ10 (red lines in image)
- High **bioactivity** due to presentation of CoQ10 to the cell in the **right orientation**
- Lipid nanoparticle suspension **30 to 80 nm size**
- Enrichment in **mitochondria**



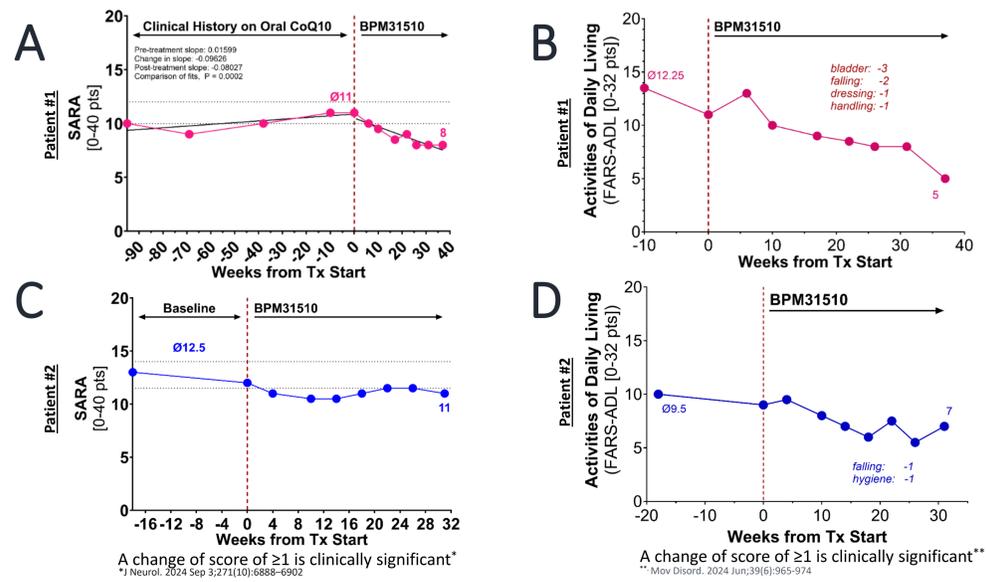
II. Primary CoQ10 Deficiency (PCQD)

- Most patients present early in childhood; the earlier the presentation, the worse the prognosis
- Identification of patients has accelerated with the advent of next-gen sequencing (mito panel)
- The current standard of care is based on over-the-counter (OTC) oral CoQ10 supplementation



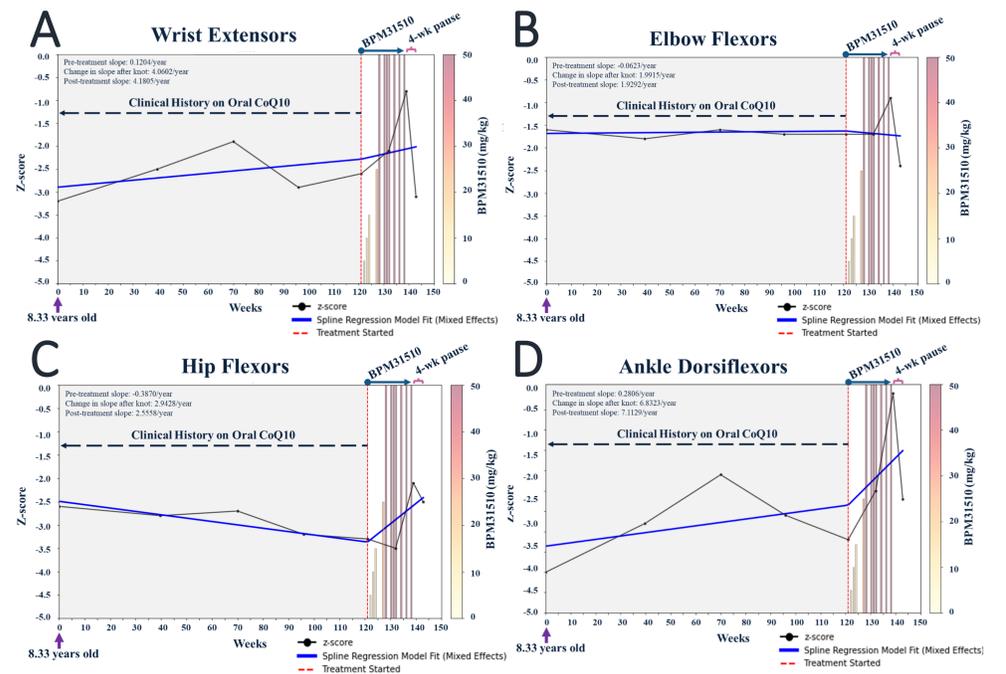
Coenzyme Q10 is a lipid-soluble molecule synthesized endogenously and plays a vital role in several essential cellular processes, including mitochondrial energy production, fatty acid beta-oxidation, pyrimidine biosynthesis, and antioxidant defense. Its biosynthesis is incompletely characterized and requires products of at least 12 known genes. The pathogenesis of PCQD is complex and related to the different functions of CoQ10. PCQD can affect any part of the body, but particularly the brain, muscle and kidney tissues. The common phenotypes are encephalomyopathy, severe infantile multisystemic disease, nephropathy, cerebellar ataxia and atrophy, and mitochondrial myopathy. The clinical trajectory of patients with PCQD is clearly progressive, serious and potentially life-threatening.

V. Ataxia Evaluations Indicating Treatment Efficacy in Patients #1 and #2



SARA scores (Scale for the assessment and rating of ataxia) for A) P#1 and C) P#2, and FARS-ADL scores (Friedreich Ataxia Rating Scale-Activities of Daily Living) for B) P#1 and D) P#2 indicate an improvement in ataxia symptoms.

VI. Patient #3 Improvements in Dynamometry Pre- vs. Post-Treatment



Graphs of age-adjusted z-score showing post treatment improvement in strength of A) wrist extensors, B) elbow flexors, C) hip flexors, and D) ankle dorsiflexors measured by hand-held dynamometry. Z-scores ≤ -2 S.D. are considered abnormal. This improvement regressed at the last data point following a 1-month unplanned treatment pause. Spline regression analysis (blue line) demonstrates the pre-treatment and post-treatment MM-COAST trajectories. Vertical red line indicates initiation of treatment. Vertical yellow columns represent administered weekly doses up to a max dose of 50 mg/kg. Gaps in between the yellow columns indicate missed doses.

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III. Schedule of Assessments (Not All Assessments Done on All Pts.)

Procedures	Specific Tests	Baseline/Screening	Day 1	Day 8 +/- 1 day	Day 15 +/- 1 day	Day 22 +/- 1 day	Weekly thereafter +/- 2 day	Monthly Starting at Day 29 +/- 2 day	End of Study
Informed Consent		X							
Demographics		X							
Medical History		X							
Administer Vitamin K and BPM31510IV			X					X	
Concomitant medication review		X	X					X	
Physical exam(including height and weight)		X							X
Vital Signs		X	X					X	
Hematology	Includes CBC, PT, INR (aPTT), Hb, MCHC, MCH	X	X					X	
Serum chemistry	Albumin, alkaline phosphatase, total bilirubin, bicarbonate, BUN, calcium, chloride, creatinine, glucose, LDH, phosphorus, potassium total protein, AST, ALT, sodium	X	X					X	
Urinalysis	Appearance, color, pH, specific gravity, ketones, protein, Urine protein creatinine ratio – UPCR, glucose, nitrite, and occult blood (microscopic examination of sediment will be performed only if the results of the urinalysis dipstick evaluation is positive)	X	X					X	
Adverse event review and evaluation		X	X					X	X
Biomarker assessments		X	X					X	X
Pregnancy test	Serum pregnancy test (women of childbearing potential)	X	X					X	X
EKG(as indicated)		X	X					X	X
MRI assessment(optional)		X	X					X	X
Clinician- reported Outcome	SARA (Gait, Stance, Sitting, Speech disturbance, Finger chase, Nose-finger test, Fast alternating hand movements, Heel-shin slide)	X	X					X	X
Patient reported outcome	FARS-E	X	X					X	X
	CGI-C relative to previous assessment	X	X					X	X
Observer-reported Outcome	FARS-ADL	X	X					X	X
	PGI-C relative to previous assessment	X	X					X	X
Performance Outcome	Goal Attainment Scale	X	X					X	X
	CaGI-C relative to previous assessment(global + 4 domains)	X	X					X	X
Digital-motor outcome	9-Hole peg test	X	X					X	X
	APDM(Gait, Romberg eyes open/closed, Tandemgait/stance if possible)	X	X					X	X
	Q-motor (Finger Tapping, Diadochokinesia Grip-Lift, Target Reaching)	X	X					X	X
MM-COAST	Video Recording (2-3 intra-individual tasks at home plus APDM gait plus Q-Motor spiral)	X	X					X	X
	Dynamometry (Elbow and hip flexion, wrist extension, ankle dorsiflexion)	X	X					X	X
	Balance (single leg eyes closed, tandem stance eyes open and closed)	X	X					X	X
	30 sec sit-to-stand	X	X					X	X
Other assessments	6-min walk test	X	X					X	X
	9-Hole peg test	X	X					X	X
	Functional dexterity	X	X					X	X
	CeCEST MRI Scan	X	X					X	X
Video Recording		X	X					X	X

IV. Patient Demographics, Treatment duration and High-level Response

Patient details	Tx Start (weekly infusion)	Mutation	Response
P#1 9-year-old Female	10/19/2024	COQ8A (c.812G>A; p.Arg271His; c.1821C>A; p.Tyr607Ter)	Improvements in SARA score, 9HPT (>5sec), activities of daily living, tandem walk, school performance and others “There have been no more urinary incidences [used to come home from school and had to change her pants].” (parents) “Her walking and running is more stable; her legs are more controlled.” (parents)
P#2 17-year-old Male	11/26/2024	COQ8A (c.1042C>T; p.Arg348* homozygous)	Improvements in SARA score, 9HPT (>5sec), activities of daily living, walking speed and endurance, school performance “I experienced no more falls since I am on treatment” “His gait is more stable, his fine motor skills have improved, and his speech is faster” (parents)
P#3 10-year-old Female	2/7/2025	COQ8A (1q42.13 28kb deletion; c.1390 C>T; p.Arg464Trp)	Improvements in MM-COAST (Flickinger et al. 2021) dynamometry measurements, tandem walk, stair climb, 10MWT, 9HPT etc. “The patient’s school and family have noted significant improvements in cognition, attention, and communication.” The patient’s teacher noted, “Patient is very engaged and excelling in her schoolwork”.

CONCLUSIONS – Preliminary Evidence of Potential Therapeutic Signal

- BPM31510 weekly IV treatment has been well tolerated with no drug related clinically significant adverse events.
- All three patients have demonstrated improvements in ataxia and motility by SARA score (ataxia) or MM-COAST objective assessments (dynamometry)
- Noticeable improvements were observed as early as 4 weeks after treatment initiation and sustained past 20 weeks.
- Patient #3 showed regression in muscle strength after a 1-month treatment pause.
- Parents, teachers, and/or parents of peers have reported substantial improvement in day-to-day activities for all 3 patients.

This preliminary evidence suggests that BPM31510 may have a substantial effect in patients with PCQD, warranting further investigation.