Pharmacological treatment in ataxia

Current status and trials

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Diagnosis and Treatment of Cerebellar Ataxias
MDS-ES
Online, July 13-14 2026

Outline of the presentation

1. Symptomatic treatments
2. Supplementary treatments
3. Etiological treatments
4. Emerging therapies
5. Perspectives

Symptomatic treatments

Cerebellar ataxia	- Physical therapy, adaptive devices (e.g., canes, walkers, motorized chairs). - Occupational therapy
Dysarthria	- Speech therapy, alternative means of communication
Diplopia	- Orthoptist - Pharmacologic treatment (4-AP, clonazepam, baclofene, Acetyl-DL-Leucine, memantine)
Dysphagia	- Feeding therapy programs, education, gastric tube placement
Weight loss	- Nutritional & vitamin supplementation
Cognitive/Neurobehavioral/ Psychiatric Manifestations	- Psychotherapy - Cognitive & behavioral therapy - Pharmacologic treatment for depression (SSRIs, SNRIs), anxiety, psychosis
Spasticity	- Pharmacologic treatment (Baclofen, Tizanidine, Clonazepam) - Botulinum toxin
Parkinsonism	- L-Dopa, dopamine-agonists
Tremor	- Propranolol, Primidone, Clonazepam, Topiramate, Zonisamide
Neuropathic pain	- Gabapentin, Pregabalin, Amitriptyline
Bladder dysfunction	- Pharmacologic treatment (Oxybutynin, Solifenacin, etc.), intermittent catheterization
Orthostatic hypotension	- Midodrine, Fludrocortisone, Droxidopa
Seizures	- Antiepileptic drugs
Sleep disorders	- Melatonin, BDZ, sleep hygiene

Minimal metabolic screening in cerebellar ataxias

- **Low vitamine E**: ataxia with vitamin E deficiency (FA like) and Abetalipoproteinaemia
- **Increased α-fetoprotein**: ataxia atelengectasia and ataxia with oculomotor apraxia AOA2
- **Increased cholestanol**: Cerebrotendinous xanthomatosis
- **Low albumin** (high serum cholesterol): Ataxia with oculomotor apraxia AOA1
- **Increased oxysterols** (cholestane-3β,5α,6β-triol) **and Lyso-Sphingomyelin-509**: Nieman Pick type C
- **Low Coenzyme Q10** (plasma and muscle): Coenzyme Q10 Deficiency

Ataxia with Vitamine E deficiency (AVED)

- α-tocopherol transfer protein (*TTPA*): gene defect in the integration of vitamin E in the VLDL and further transfer of vitamin E from the liver to the CNS

- Pathogenic variants: 744delA in North Africa (*Ben Hamida et al Neurology 1993);* 513insTT, 400C>T in patients of North European origin *(Elkamil et al J Mov Disord 2015)*

- Treatment: oral administration of high-dose vitamin E (alpha-tocopherol) from 1000 to 3000 IU/day. The goal is to normalize plasma vitamin E concentrations.

- Vitamin E should be taken with meals to enhance absorption.

- Monitoring: Baseline and periodic serum alpha-tocopherol levels are essential to guide dosing. Long-term therapy is maintained lifelong to prevent recurrence or progression.

Abetalipoproteinemia

- *MTTP* gene: absence of apolipoprotein B containing lipoproteins (chylomicrons, VLDL, LDL). This results in severe fat malabsorption and deficiencies of fat-soluble vitamins (A, D, E, K),

- Dietary management: low-fat diet, especially reduced long-chain triglycerides, use of medium-chain triglycerides (MCT oil) for caloric needs

- High-dose fat-soluble vitamin supplementation: vitamin A-D-E-K

- Monitoring: Regular neurological assessments, weight and nutritional status

Other vitamin supplementations:

- *SLC19A3:* Biotin-thiamine responsive basal ganglia disease; high oral dose of biotin, associated with thiamine.
- *SLC52A2:* encoding a riboflavin transporter involved in Brown-Vialetto-Van Laere syndrome; responsive to riboflavin supplementation *(Guissart et al., 2016).*
- *COQ8A* mutations, the synthesis of coenzyme Q10 itself is deficient. Supplementation by coenzyme Q10 seems to improve motor dysfunction in some COQ8A-mutated patients *(Mignot et al., 2013).*

Cerebrotendinous Xanthomatosis (CTX)

- *CYP27A1* gene: impaired bile acid synthesis and accumulation of cholestanol in tissues

- Chenodeoxycholic acid (CDCA): for adults 250 mg three times a day and 15 mg/kg per day in three divided doses for children. It restores feedback inhibition in bile acid synthesis, reducing cholestanol levels.

- Treatment in presymptomatic individuals appears to prevent clinical manifestations.

- Monitoring: Plasma cholestanol levels, clinical follow-up, MRI brain and tendon imaging

- Adjunctive therapies: Statins to further reduce cholestanol (+/- CoQ10). Cataract extraction. Neurologic complications of epilepsy, spasticity, and parkinsonism are treated symptomatically. Calcium and vitamin D are supplemented for patients with osteoporosis. Xanthomas may be surgically removed for cosmetic reasons.

Niemann-Pick Type C

- *NPC1 or NPC2:* accumulation in lysosomes of cholesterol, glycosphingolipids and sphingosine

- Miglustat (OGT 918, N-butyl-deoxynojirimycin) is an iminosugar that acts as an inhibitor of glucosylceramide synthase

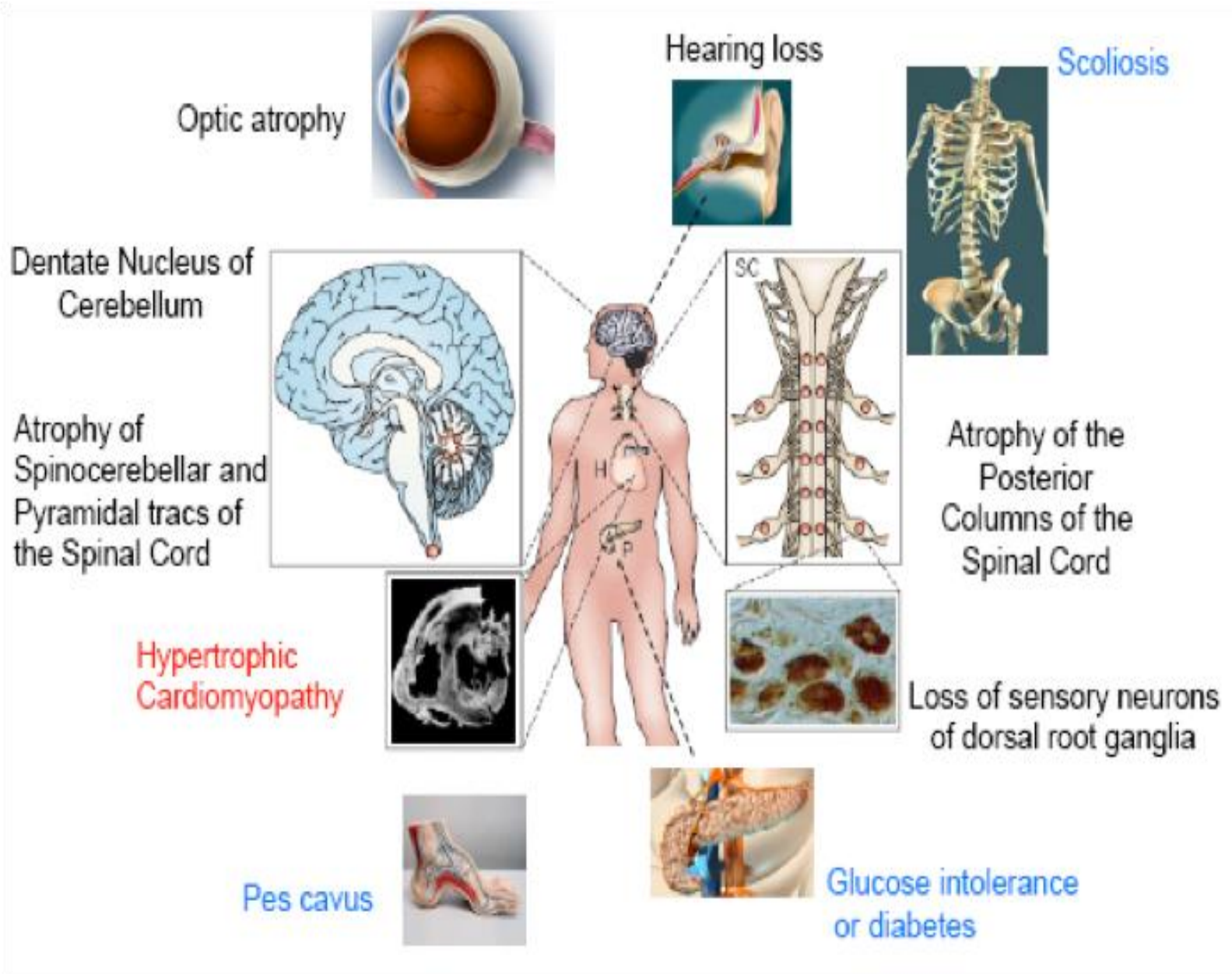
- Adults: 200 mg three times daily (total: 600 mg/day); children: weight-adjusted dosing (commonly 100 mg twice or three times daily); oral administration, with or without food

- No effect on visceral involvement (e.g. hepatosplenomegaly or pulmonary symptoms)

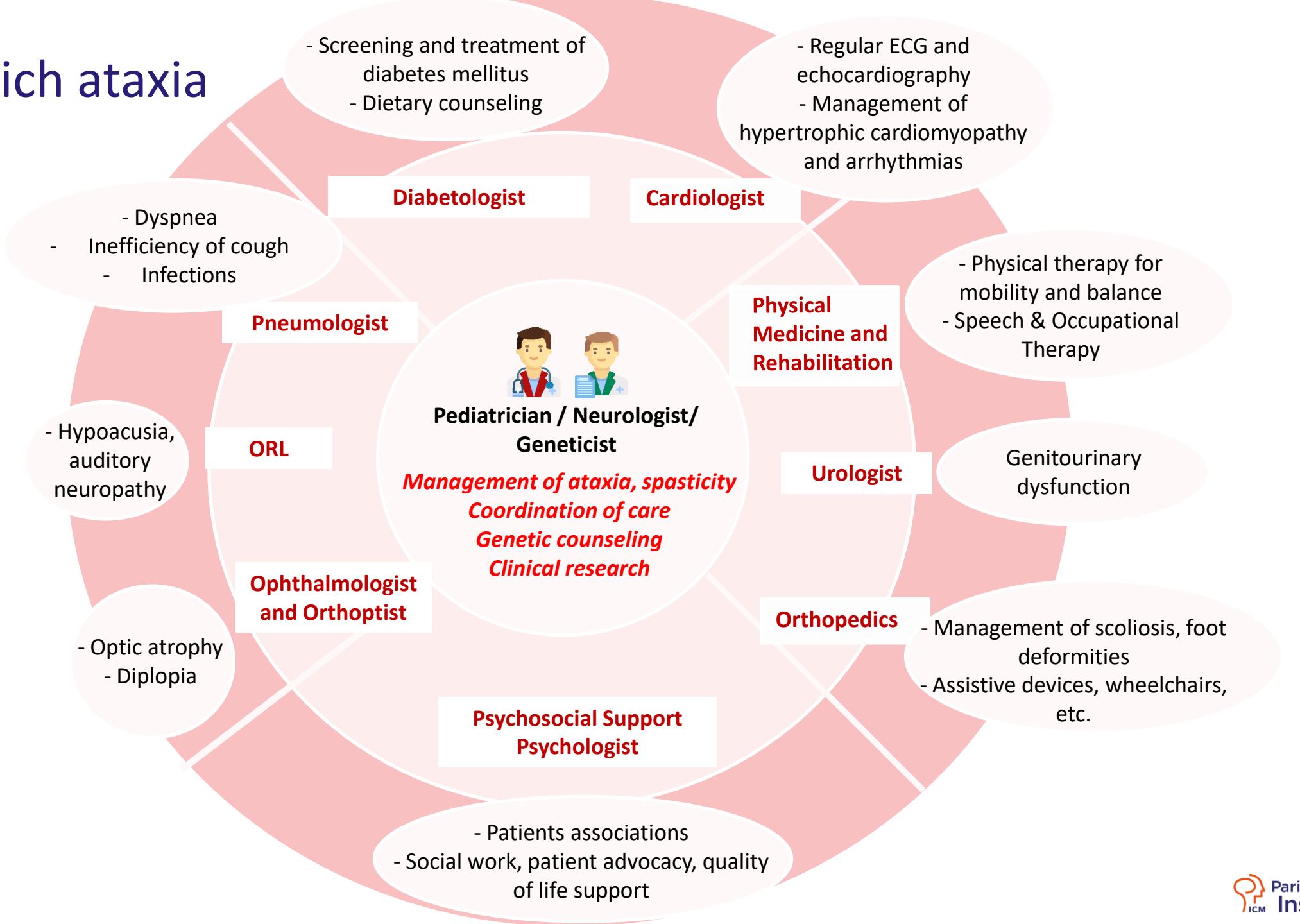
- Monitoring: Regular neurological assessments, liver function tests and complete blood counts (especially during first 6–12 months), weight and nutritional status

Friedreich ataxia

98% homozygote for GAA repeat expansions on both alleles in *FXN*
2% compound heterozygotes



Friedreich ataxia



QUESTION 1

What is one of the main challenges in demonstrating efficacy in ataxia clinical trials?

- A. Slow disease progression
- B. Placebo effect
- C. Clinical heterogeneity
- D. All of the above

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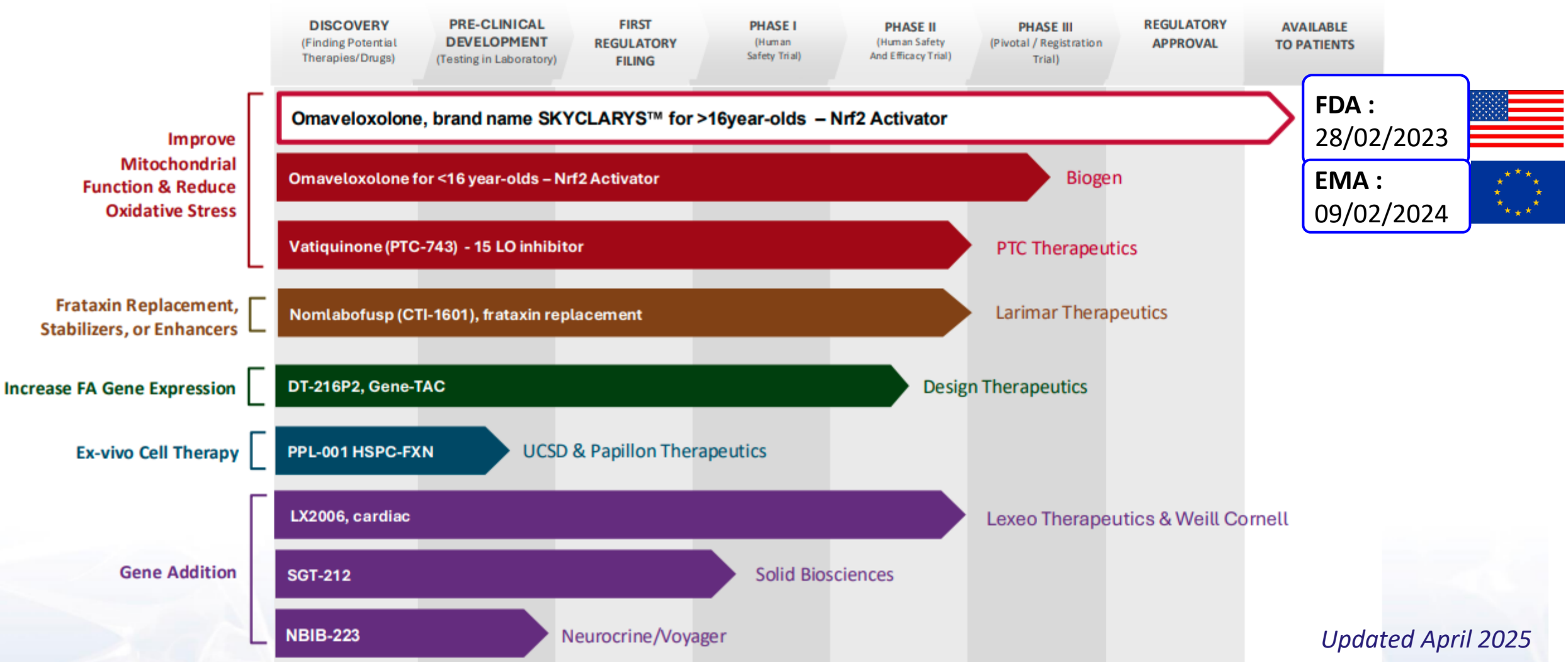
Friedreich's Ataxia Drug Development Pipeline

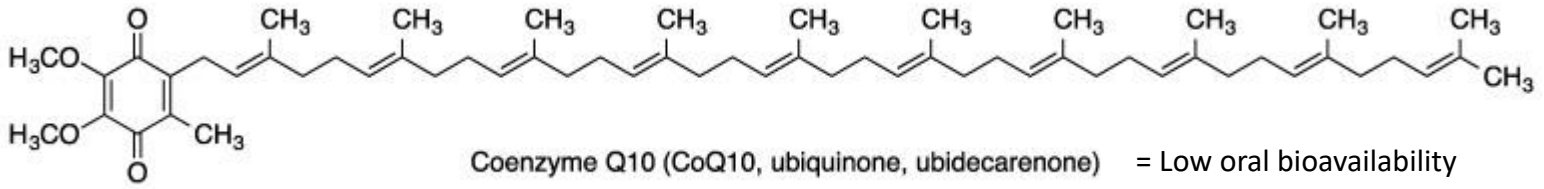


Updated April 2025

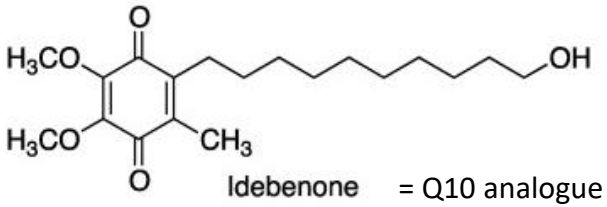
Paris Brain Institute ICM

Endogenous quinones and analogues

Ex: CoQ10, Vatiquinone

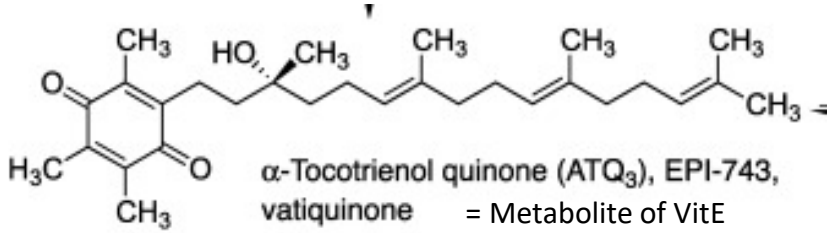


Coenzyme Q10 (CoQ10, ubiquinone, ubidecarenone) = Low oral bioavailability



Idebenone = Q10 analogue

Toxicity/protection: depends on dose, exposure time, electrophylicity



α-Tocotrienol quinone (ATQ_3), EPI-743, vatiquinone = Metabolite of VitE

Good oral bioavailability
Anti-oxydant potency : x100
Crosses BBB
Nrf-2 activator (anti-oxydant)
Inhibitor of 15-lipoxygenase (anti-ferroptotic)

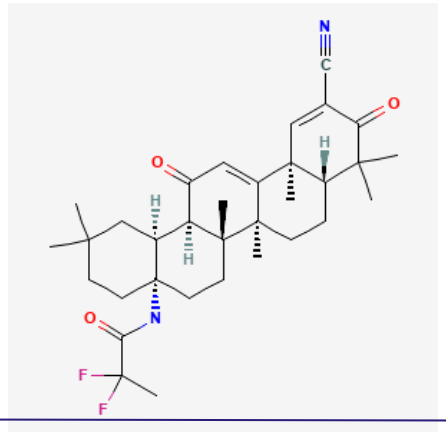
MoveFA trial

Rescue FA cellular models

Moxie Trial

Quinone synthetic derivatives

Ex: Omaveloxolone

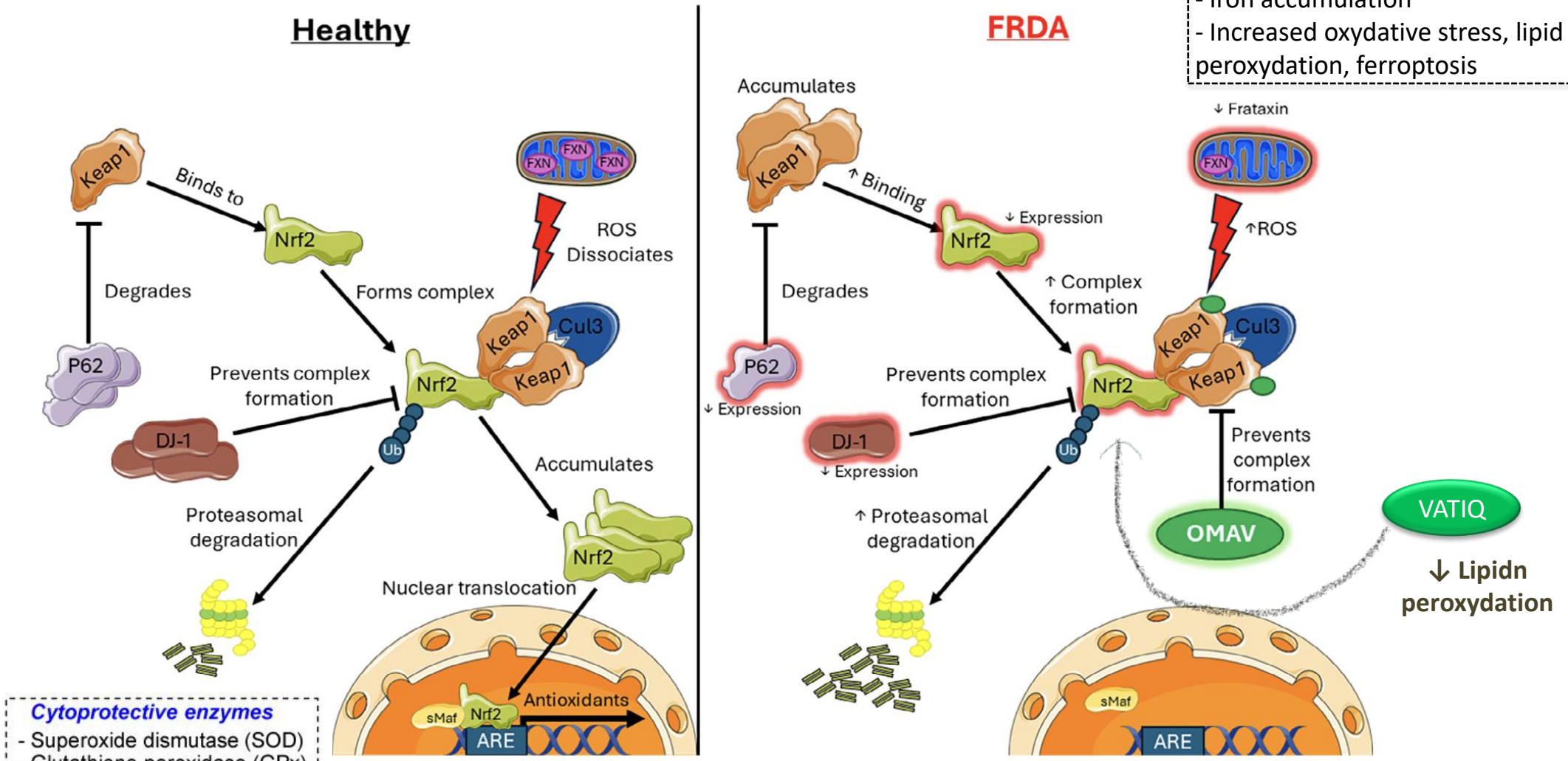


Good oral bioavailability
Potent anti-oxydant
Crosses BBB
Nrf-2 activator

Paris Brain Institute ICM

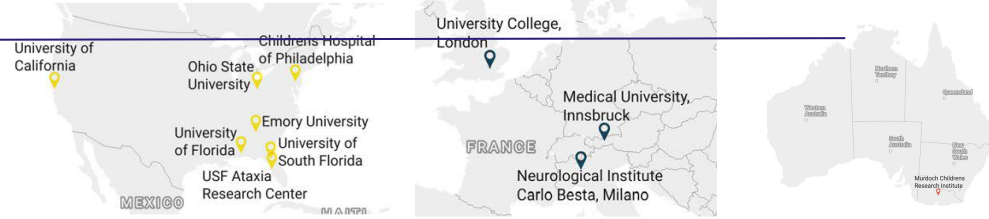
Nrf2: a master transcription factor

- Disruption of iron homeostasis
- Iron accumulation
- Increased oxydative stress, lipid peroxydation, ferroptosis



Naghipour et al, Mov Disorders, 2025

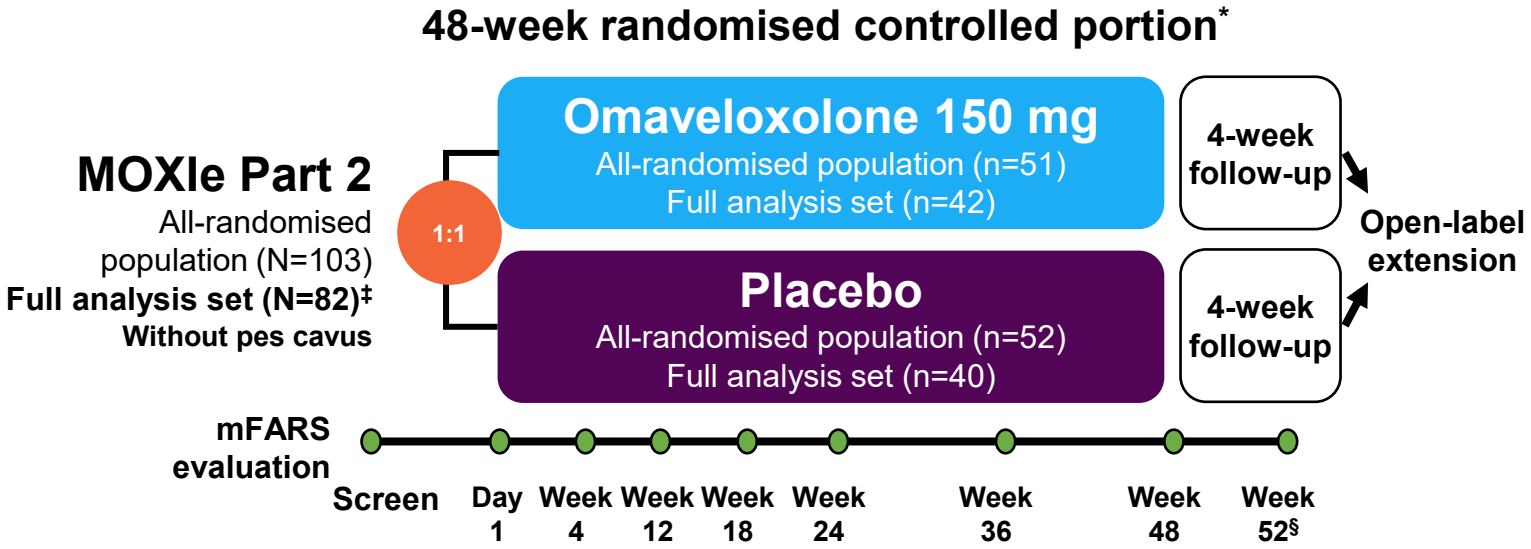
Paris Brain Institute



MOXIe Part 2 study design

Phase 2, international, double-blind, placebo-controlled, randomised, parallel-group trial

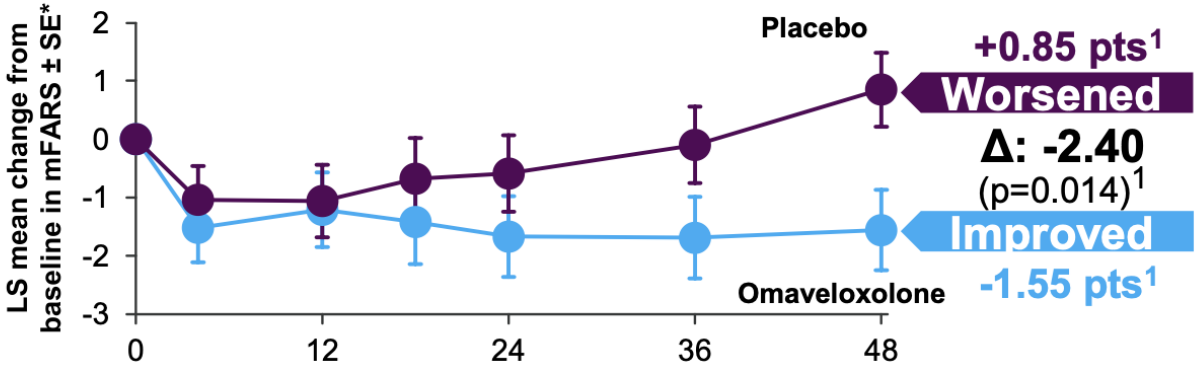
Key inclusion criteria	Key exclusion criteria
✓ Age **16-40** years	× Uncontrolled diabetes
✓ Baseline mFARS score of 20–80	× **Clinically significant cardiac disease** (LVEF<40%, previous cardiac insufficiency or arrhythmia)
✓ Ability to complete maximal exercise testing	× **Anticoagulation** (inductor of CYP3A4 -> numerous interactions)



Primary endpoint:
Change from baseline in mFARS at week 48

Lynch DR, et al. Ann Neurol. 2021

MOXIe Part 2 results “FAS population”



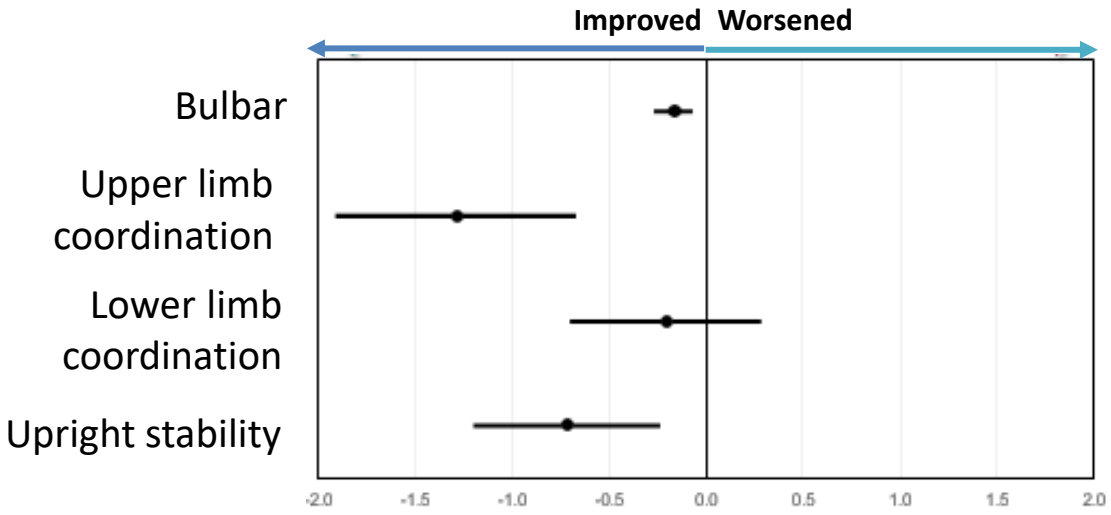
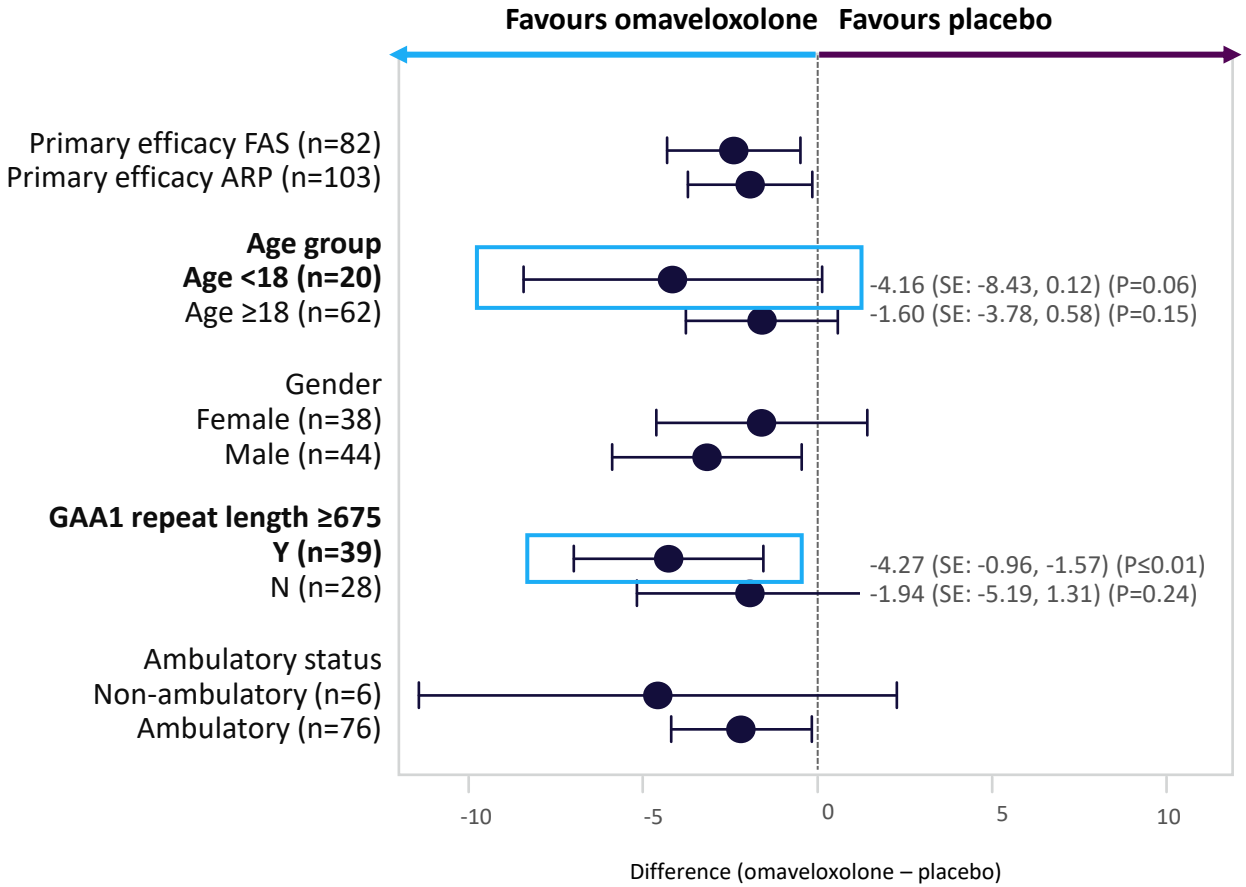
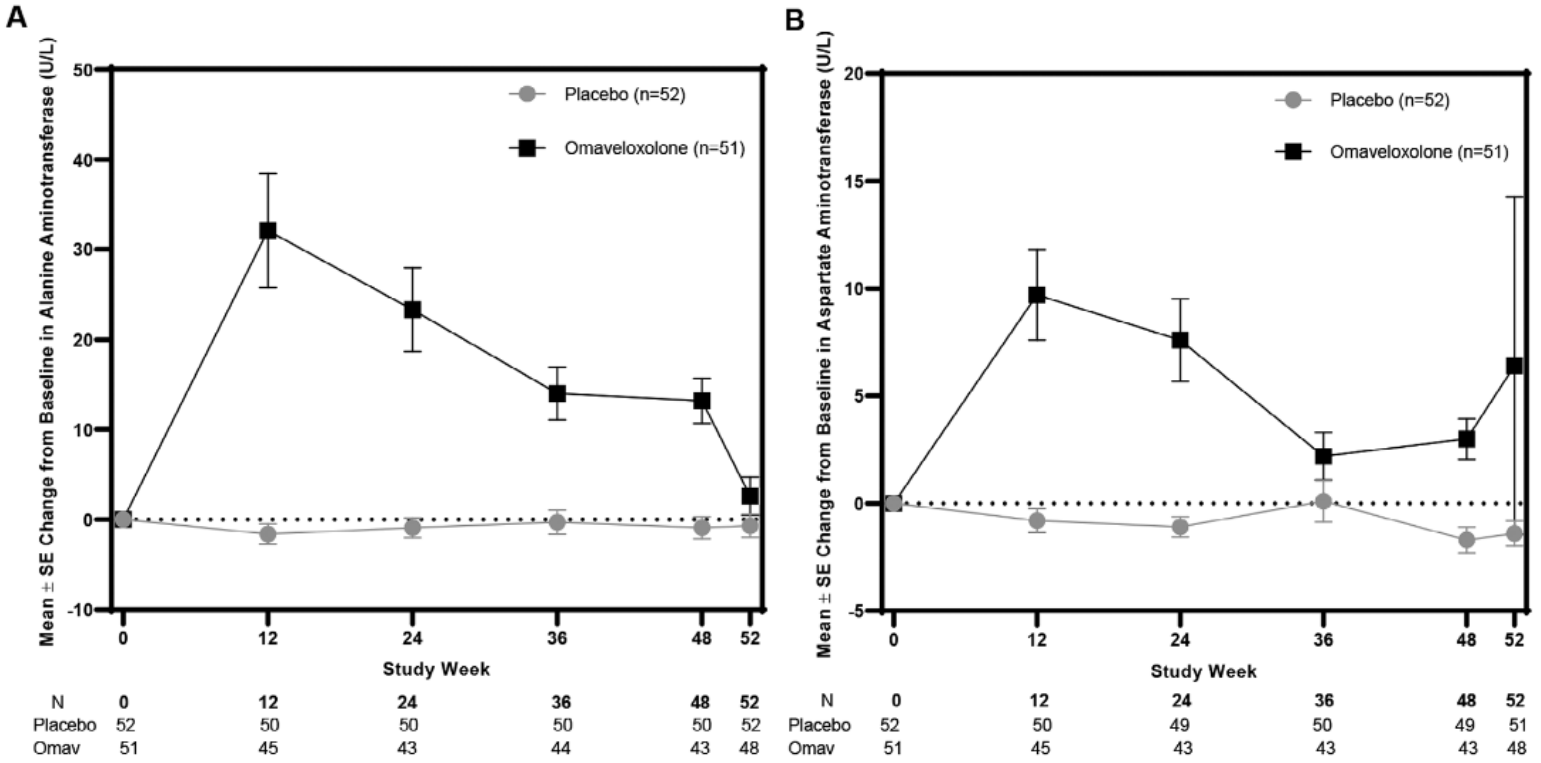


Figure adapted from Boesch S, *et al.* 2024



Lynch et al, Ann Neurol 2021

MOXIe Part 2: adverse events



AE	Placebo n=52	Omav. n=51
Headache	25%	37%
Nausea	14%	33%
Abdominal pain	6%	22%
Diarrhea	10%	20%
Fatigue	14%	21%

	Placebo n=52	Omaveloxone n=51
↑ ALAT	2%	37,3%
↑ ASAT	2%	21,6%

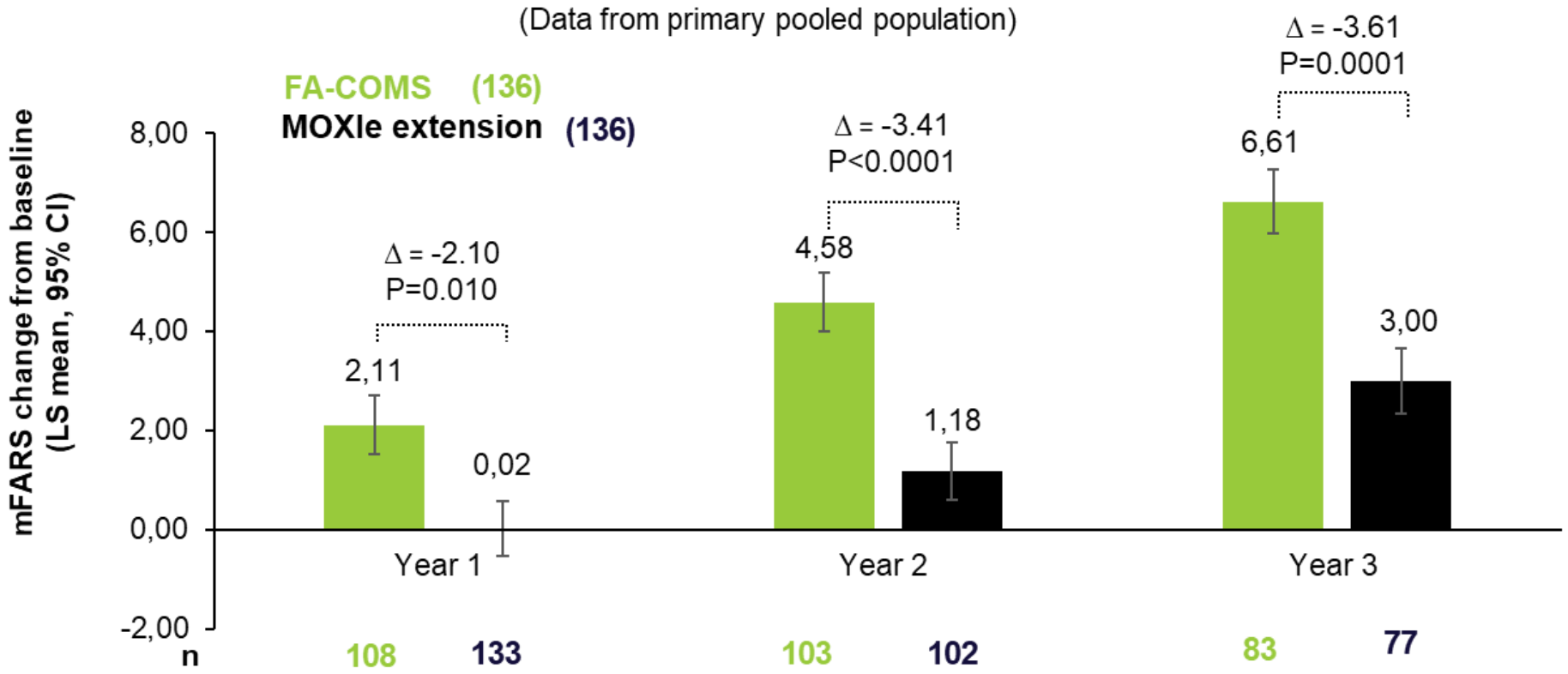
ASAT or ASAT >3N : 31,4%
ASAT or ALAT >5N : 15,7%
ASAT or ALAT >10N : 3,9%

French experience:

- Increases in LDL and decreases in HDL cholesterol
- Weight loss
- Increase in BNP

Lynch et al, Ann Neurol 2021

MOXIe extension



Lynch DR, et al. Ann Clin Transl Neurol. 2024

January 2024 : early access program, patients ≥ 16 yrs, 150 mg/j

09/02/2024

31/12/2025 : N = 459 exposed

411 patients exposed (lock : 29/07/2025)

→ **13.1% (N=54) permanently discontinued** omaveloxolone

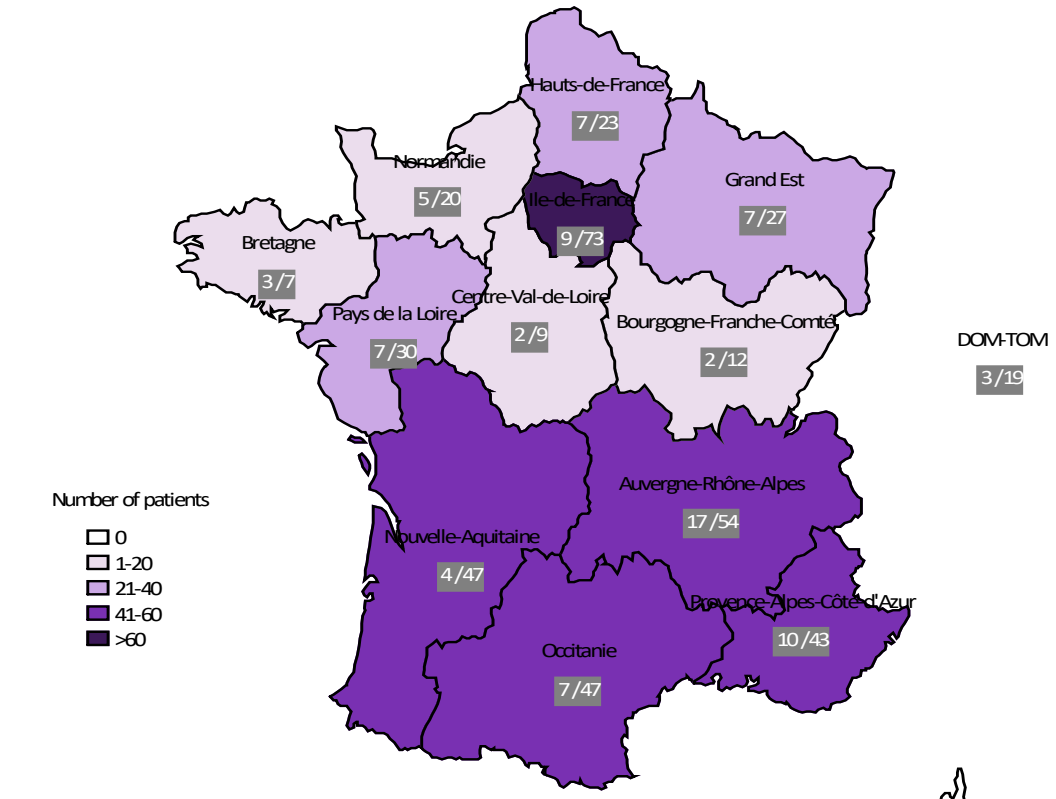
357 patients still on treatment (12 months n=212)

Mean age = 36 (15)
Ambulatory = 62%
SARA = 21,7 (8,7)
Older and more severe than Moxie study

BRAIN-TEAM Filière Nationale de Santé Maladies rares du système nerveux central

Filnemus Filière Neuromusculaire

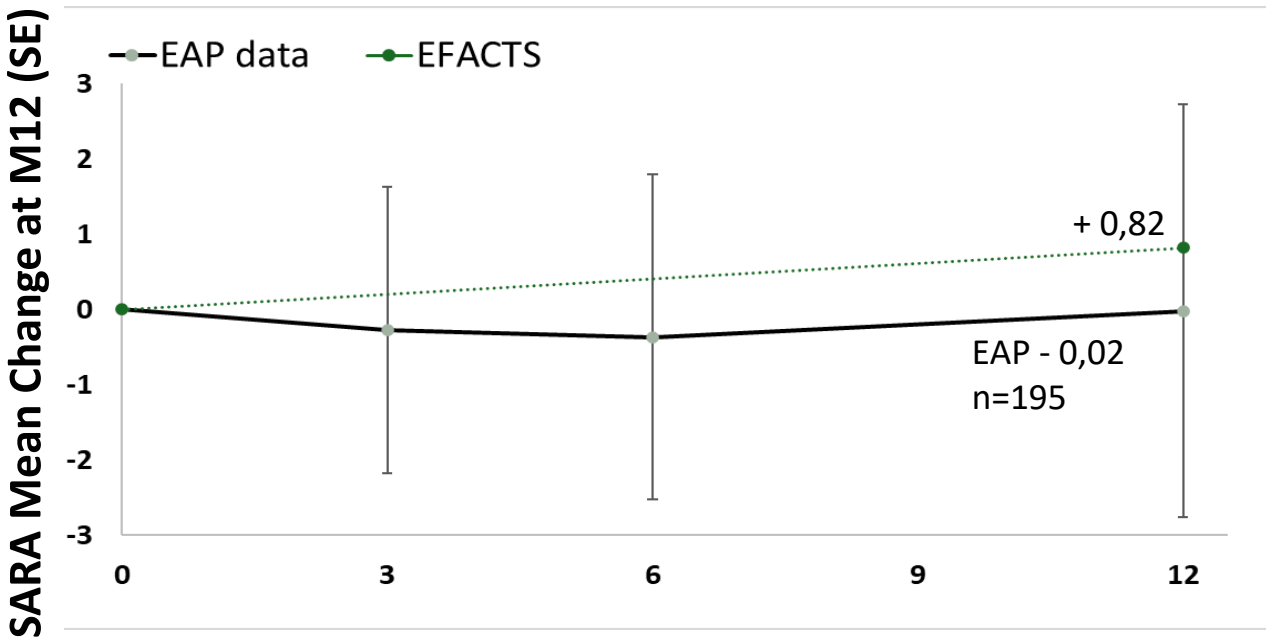
BaMaRa (1 par hôpital) → SDM- Désidentification → entrepôt BNDMR INS

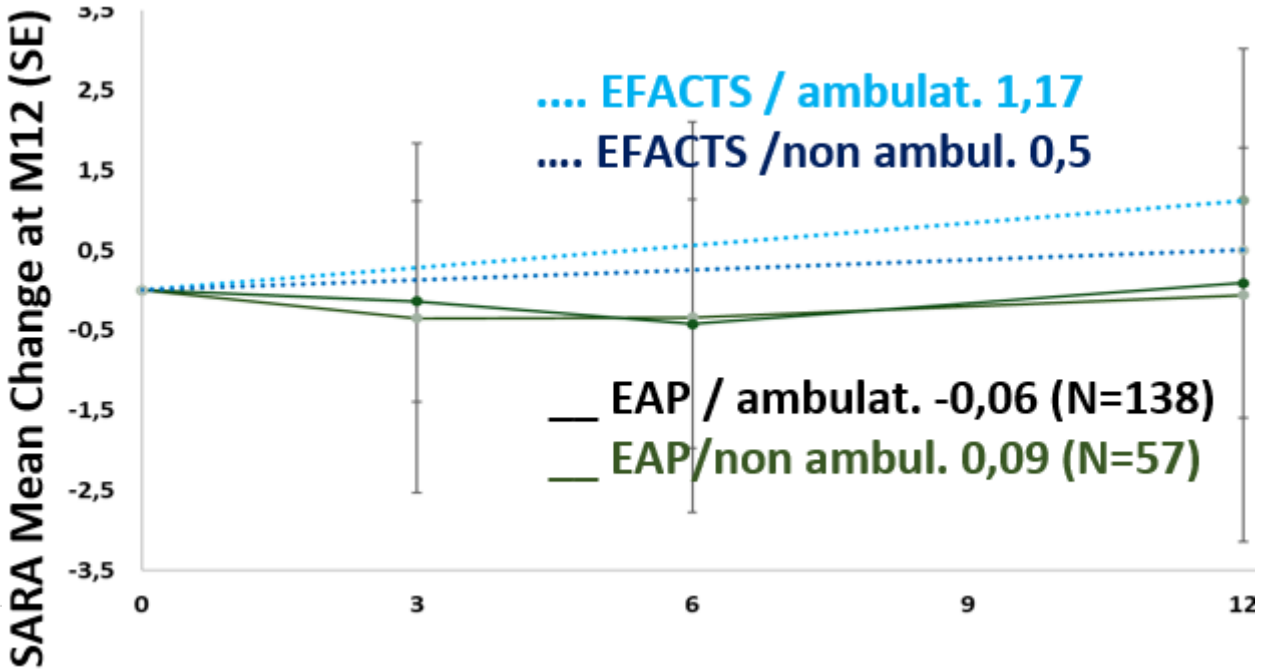


33 CHU/GHU

Prescribers (N=83) / Exposed (N=411)

Efficiency : SARA change at M12 (n=212)





→ These preliminary data suggest neurological stabilization at 1 year

→ Similar conclusions for <u>M18 (ongoing analysis)</u>

MoveFa study design

Phase 2, international, double-blind, placebo-controlled, randomised, parallel-group trial

Key inclusion criteria	Key exclusion criteria
✓ **Age ≥ 7 years**	× Point mutation or deletion
✓ Baseline mFARS score of 20–70	× LVEF <50%
✓ **Ability to walk 3 m < 1 min**	× Creatinine >1,5 N
✓ Homozygous GAA repeat	

Vatiquinone : 400mg 3 times/day

Primary endpoint:
- Change from baseline in mFARS at Week 72 in participants aged 7-21 years

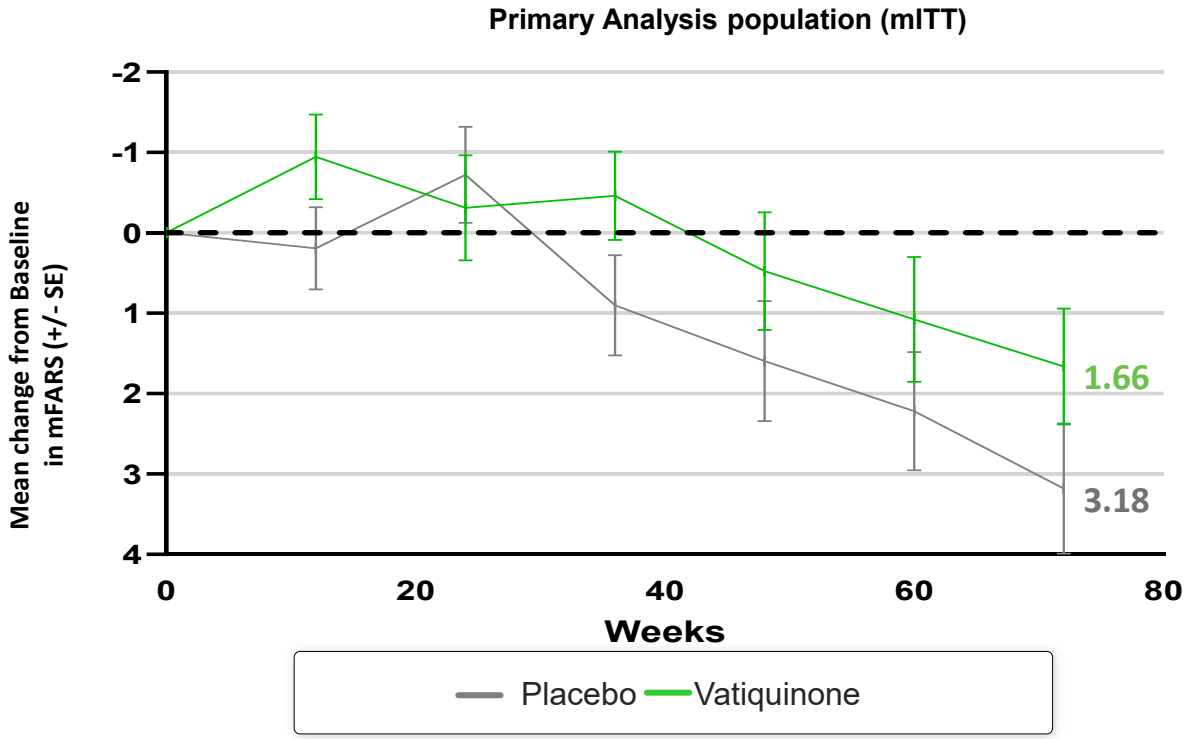
MoveFA
Overall (N=143)
Primary analysis population 7-21 years (N=123)

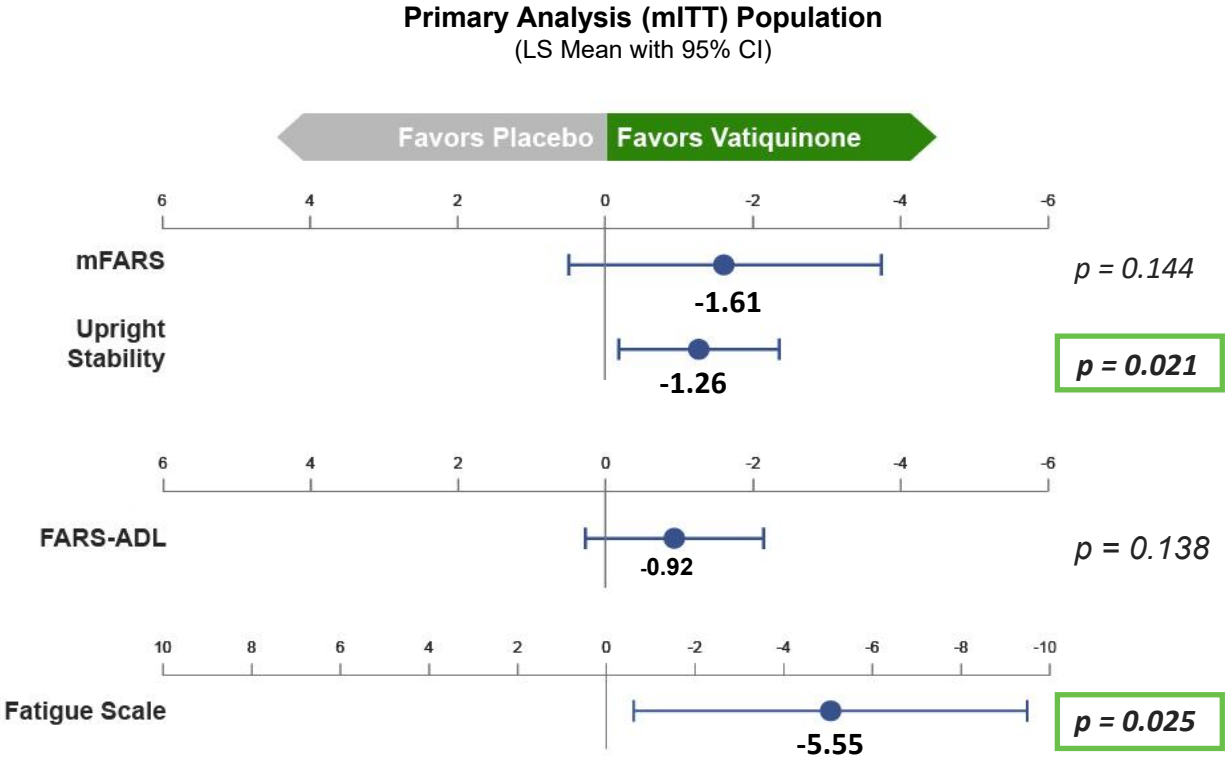
Placebo controlled 1:1
18 months (72 weeks)

Extension
24 weeks

Open-label extension 3 years
Start > sept 2023

MoveFa study design





Abstract MDA Conference 2025

Gene therapy for cardiomyopathy associated with FA

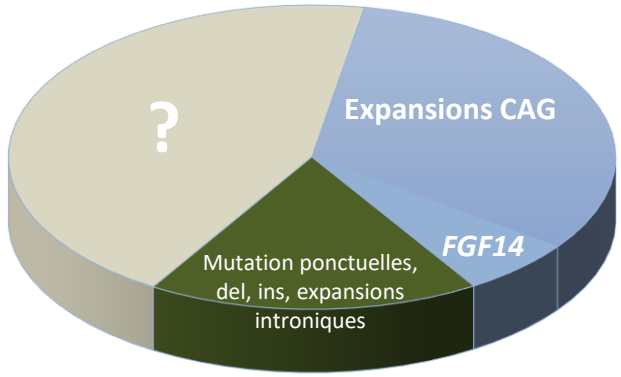
Up to 65 participants
19 locations: US, Canada, Brazil, Czechia, Germany, France, Italy, Spain
IV administration
Low, mild, or high dose of LX2006 (AAV-*FXN*)

Key inclusion criteria	Outcomes
✓ Ages ≥6 years	× Presence of other form(s) of CM contributing to heart failure (HF)
✓ Diagnosis of FA (GAA expansion on both alleles or compound heterozygous)	× Intermittent or continuous intravenous (IV) inotrope infusion, presence of a ventricular assist device, or history of prior heart transplantation
✓ Onset ≤ 25 years of age	× Contraindication to cMRI
✓ Confirmed left ventricular hypertrophy	× Prior organ transplantation
✓ Left ventricular ejection fraction ≥40%	× Initiation of cardiac resynchronization therapy (CRT) within 6 months prior to screening
	× History of prior gene transfer or cell therapy.
	× Poorly controlled diabetes (hemoglobin A1c ≥8%)

➡ Primary outcome: Treatment-emergent adverse events and serious events

➡ Secondary outcomes: Cardiac parameters (ex. Change from baseline in left ventricular mass index), mFARS, PGI-C, PGI-S, adverse events

Clinical trials in Spinocerebellar Ataxias (SCAs)



Paris cohort, ICM

	Spinocerebellar ataxia	Treatment (number treated with drug or placebo)	Outcome measures and results	Clinical trial registration number
Completed				
Phase 3, randomised[85]	SCA1, SCA2, SCA3, SCA6, SCA7, SCA10	Troriluzole 200 mg/day for 48 weeks (n=218)	m-SARA change at week 48; no improvement	NCT03701399
Phase 3, randomised[72]	SCA2	Riluzole 100 mg/day (n=45)	SARA change at month 12; no clinical or radiological improvement	NCT03347344
Phase 1–2, randomised[86]	SCA2	Erythropoietin 1 mg/6 months nasally (n=34)	SCAFI change at month 6; no serious adverse events; no improvement in ataxia score but a reduction in saccade latency	RPCEC00000187-Sp
Phase 3, randomised, crossover[87]	SCA3	Acetyl-DL-leucine 5g/day for 6 weeks (n=15/108*)	SARA change at week 6; no improvement	EudraCT 2015-000460-34
Phase 3, randomised[88]	SCA6, SCA31	Rovatirelin 1·6 or 2·4 mg/day for 24 weeks; SCA6 (n=165 cohort 1, n=83 cohort 2); SCA31 (n=72 cohort 1, n=57 cohort 2)	SARA change at week 24; no improvement	NCT01970098; NCT02889302
Phase 2B, randomised[89]	SCA38	Docosahexaenoic acid 600 mg/day (n=10)	SARA change at week 16 and 40; SARA stabilisation, but generalisability limited by small sample size	NCT03109626

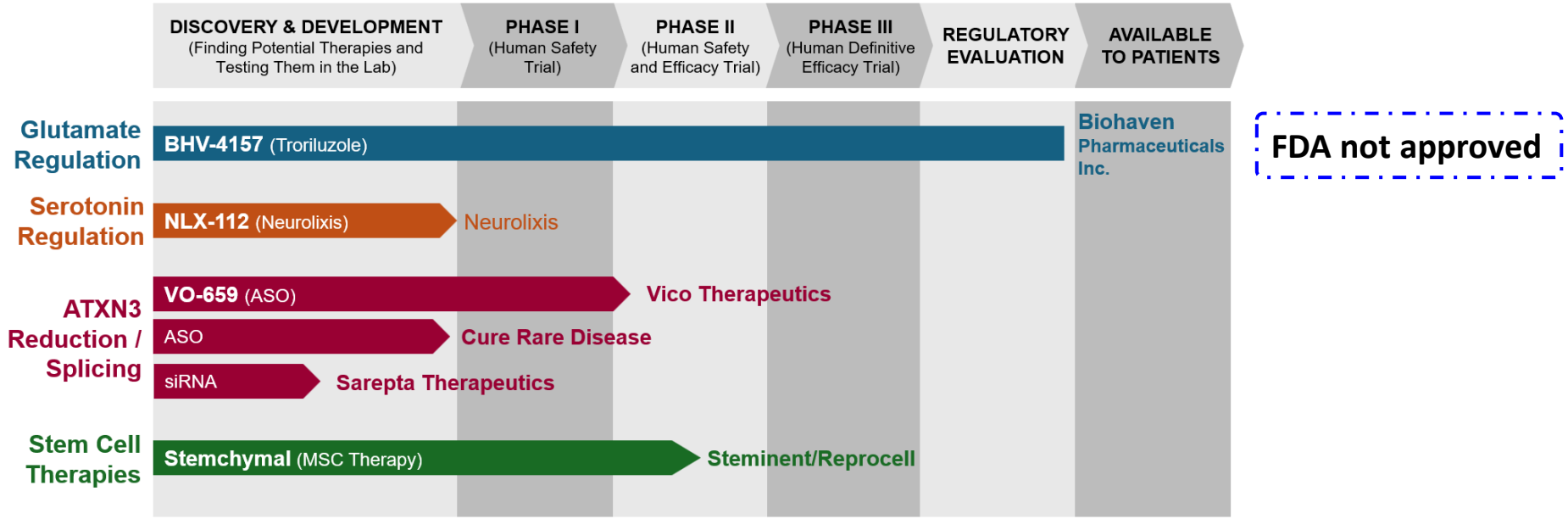
List of trials with sample sizes of at least 10 patients that have either completed or been active within the past 7 years

Coarelli , Coutelier and Durr. Lancet Neurology 2023

Paris Brain Institute

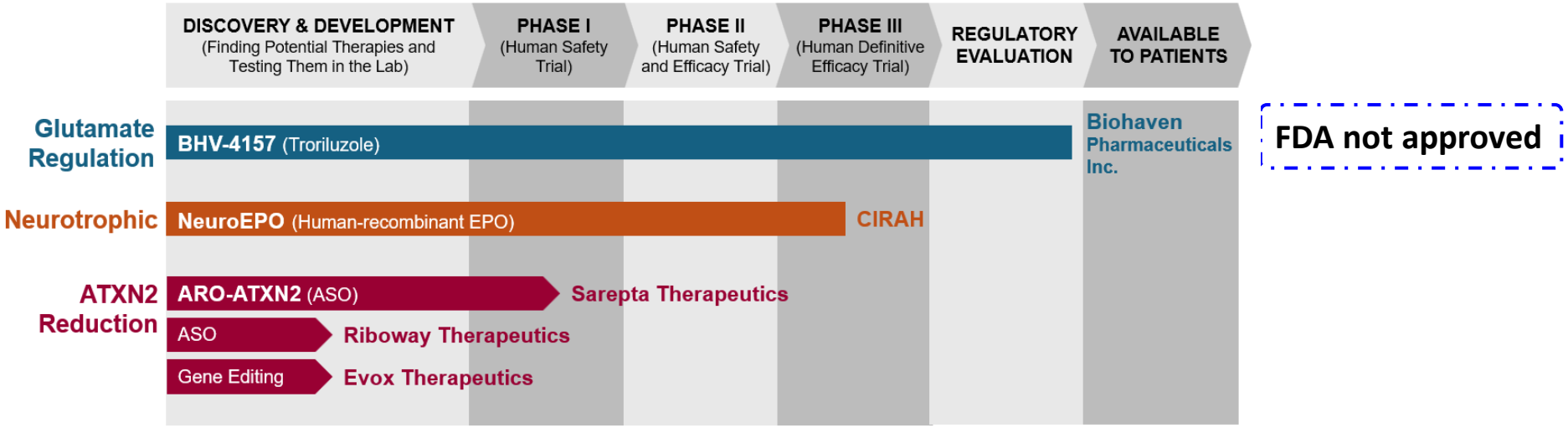
Therapies in development for SCA2 and SCA3

SCA3



FDA not approved

SCA2



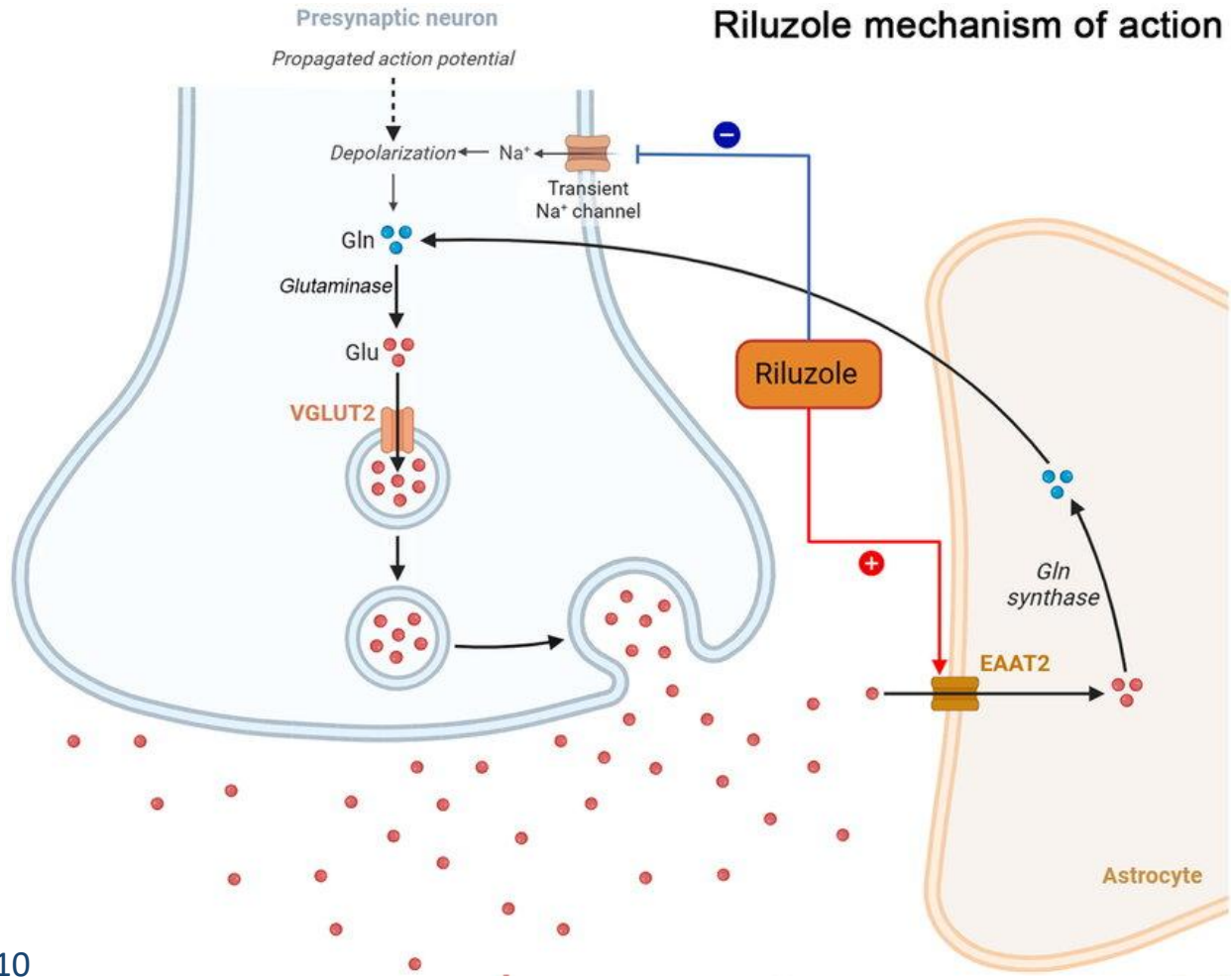
FDA not approved

Troriluzole trial in SCAs

Troriluzole is the oral prodrug formulation of Riluzole
Anti-glutamargic drug
Mechanism of action:

- **Inhibition of presynaptic glutamate release:** Reduces excitotoxicity by decreasing excessive glutamate signaling at synapses.
- **Inactivation of voltage-gated sodium channels:** Stabilizes neuronal membranes and reduces abnormal repetitive firing.
- **Restoration of the EAAT2 expression in astrocytes:** Promote the glutamate reabsorption reducing excitotoxicity.

- Phase 3 clinical trial in SCAs: The primary endpoint, change from baseline to Week 48 on f-SARA, did not reach statistical significance.
  - Different genotypes : SCA1, SCA2, SCA3, SCA6, SCA7, SCA8 et SCA10
  - Different stage of disease and different progression
  - High rate of drop out



Yuan et al., Annals of Medecine 2024

Pro and contra of riluzole in SCAs

CONTRA

In vivo assessment of riluzole as a potential therapeutic drug for spinocerebellar ataxia type 3

Post-symptomatic treatment with riluzole for 10 months in SCA3 mice: reduction of soluble ATXN3 and increase in ATXN3 aggregates, no improvement in motor deficits. *Schmidt et al, Journal of Neurochemistry 2016*

Safety and efficacy of riluzole in spinocerebellar ataxia type 2 in France (ATRIL): a multicentre, randomised, double-blind, placebo-controlled trial

Giulia Coarelli, Anna Heinzmann, Claire Ewenczyk, Clara Fischer, Marie Chupin, Marie-Lorraine Monin, Hortense Hurmic, Fabienne Calvas, Patrick Calvas, Cyril Goizet, Stéphane Thobois, Mathieu Anheim, Karine Nguyen, David Devos, Christophe Verny, Vito A G Ricigliano, Jean-François Mangin, Alexis Brice, Sophie Tezenas du Montcel, Alexandra Durr

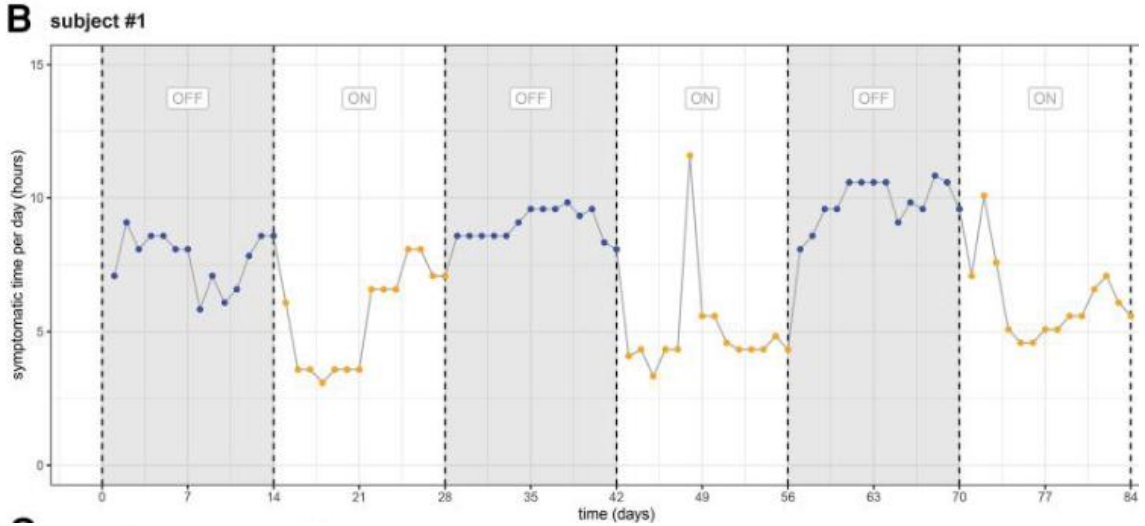
Lancet Neurol 2022

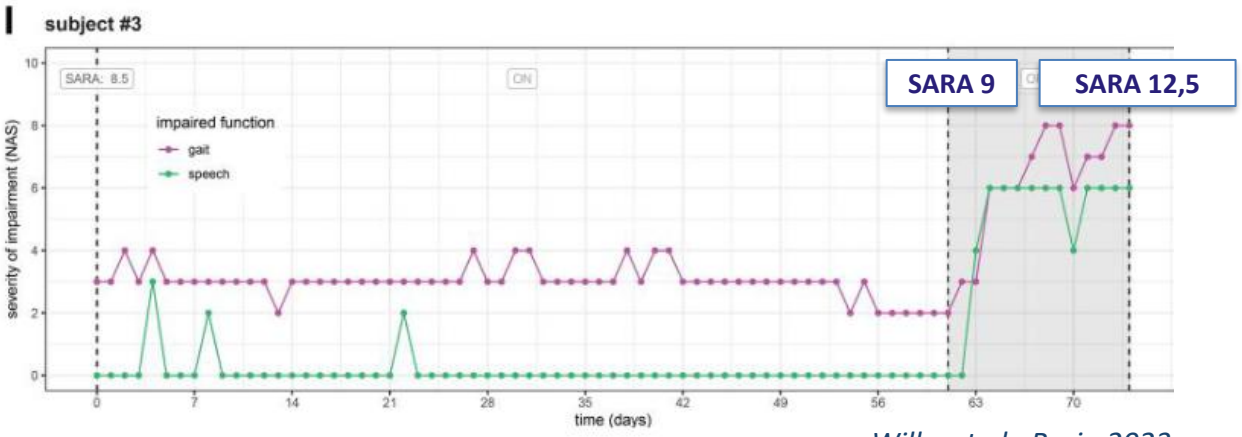
	Riluzole (n=22)	Placebo (n=22)	Mean difference (95% CI)	p value
Primary outcome: SARA score improvement of at least 1 point at month 12	7 (32%)	9 (39%)*	−10·3% (−37·4 to 19·2)	0·75
Secondary outcomes				
Change in the SARA score between inclusion and month 12	0·5 (−1·5 to 1·5)	0·3 (−1·0 to 2·5)	0·23 (−1·06 to 1·51)	0·70
Change in the CCFS between inclusion and month 12	0·055 (0·014 to 0·086)	0·004 (−0·040 to 0·020)	0·064 (0·015 to 0·110)	0·0050
Change in the INAS between inclusion and month 12	0 (−1 to 1)	−1 (−2 to 0)	−0·8 (−1·7 to 0·0)	0·070
Change in SF-36 PCS between inclusion and month 12	−1·9 (−6·2 to 11·1)	0·7 (−5·4 to 8·6)	−2·1 (−6·4 to 2·3)	0·89
Change in SF-36 MCS between inclusion and month 12	−0·8 (−4·7 to 3·5)	−4·7 (−6·8 to 3·2)	−1·5 (−8·4 to 5·5)	0·31
Percentage of MRI volume variation between inclusion and month 12				

No difference in MRI volumetry for cerebellum and brainstem

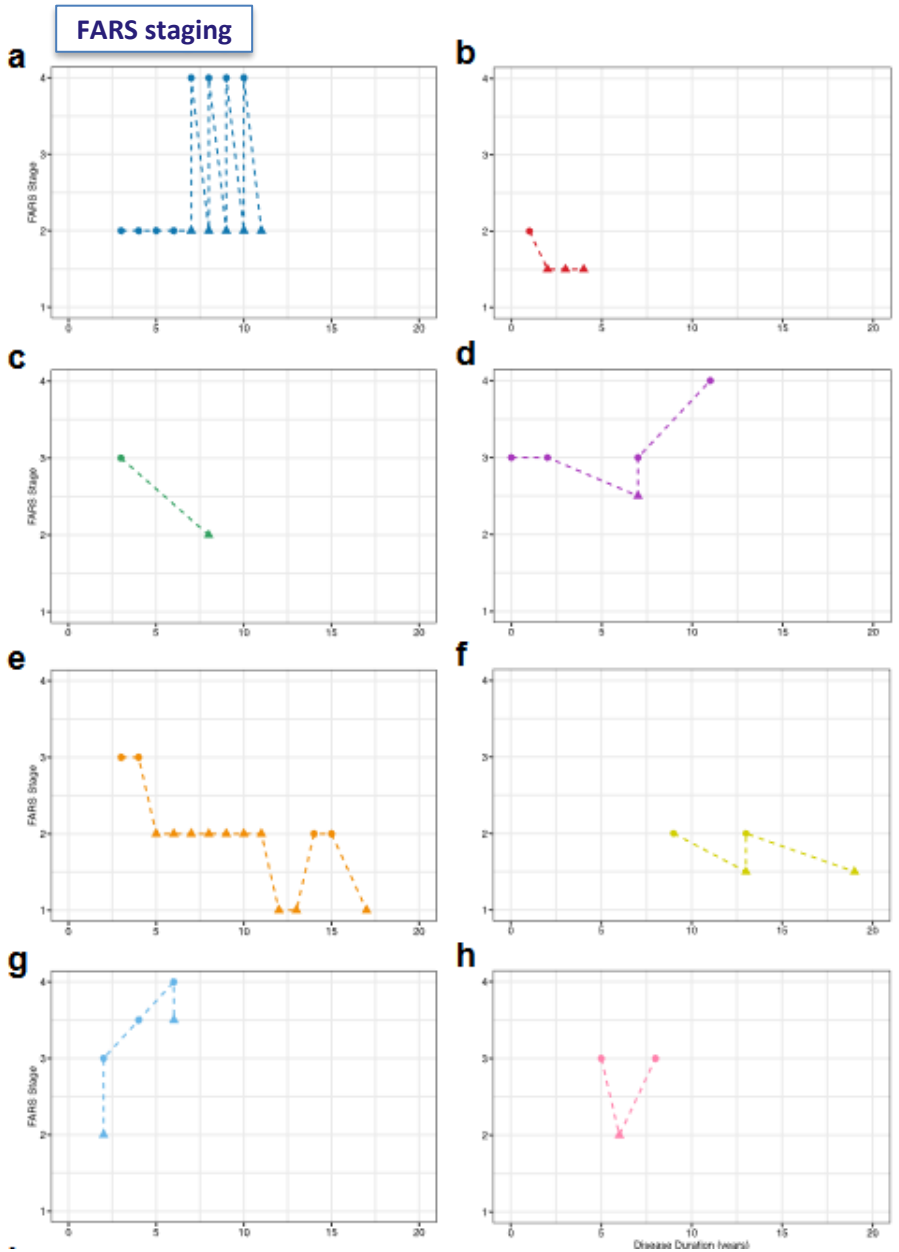
SCA27B and 4-aminopyridine

4-AP: K^+ channel blocker, approved for MS





Wilke et al., Brain 2023



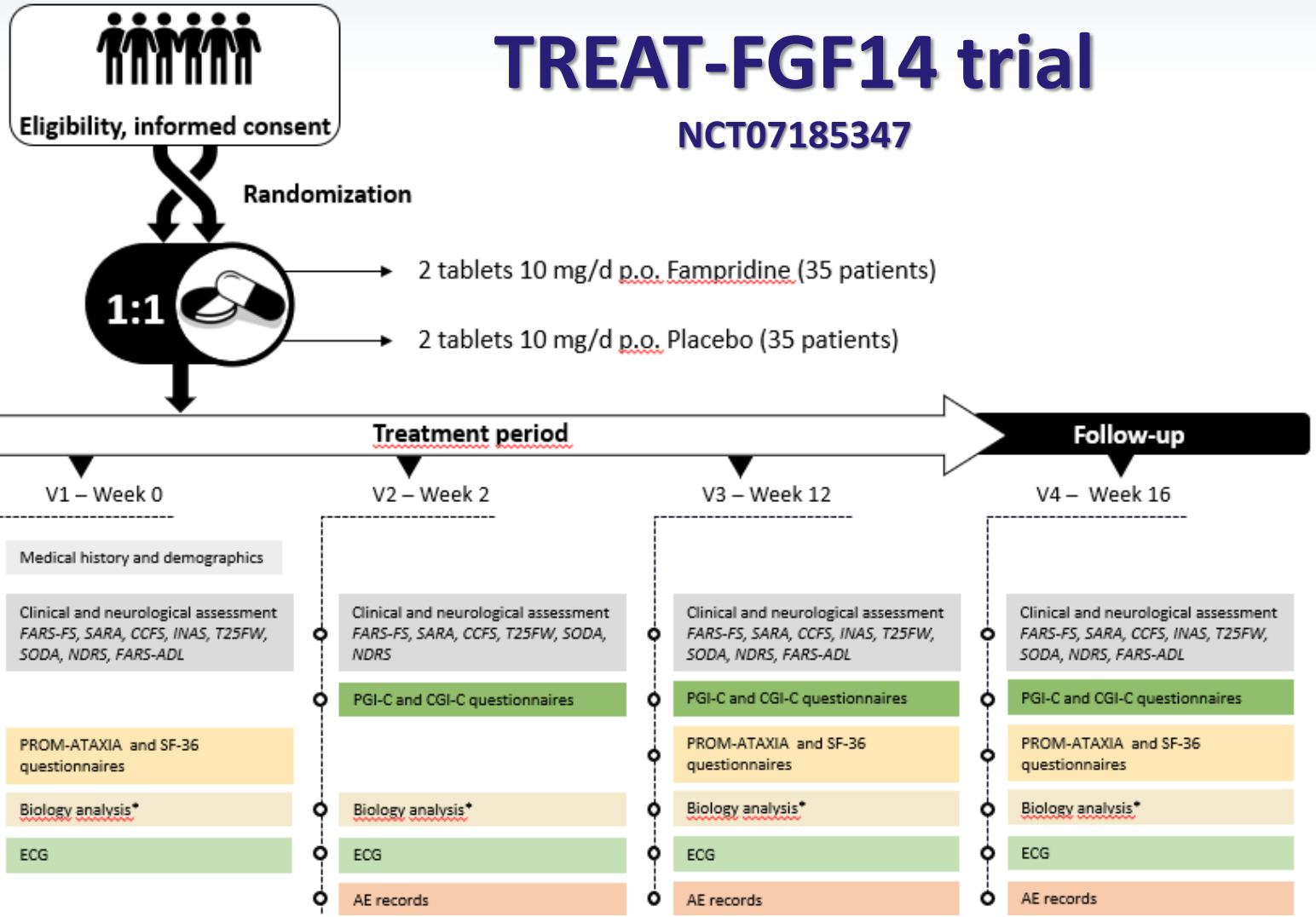
Pellerin et al., eBioMedicine 2024

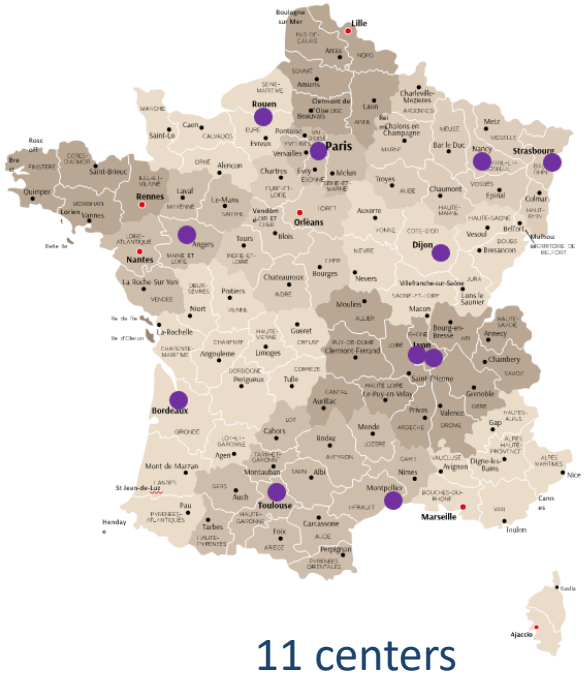
Paris Brain Institute ICM

SCA27B and 4-aminopyridine treatment

TREAT-FGF14 trial

NCT07185347





11 centers

Ongoing, 70/78 enrolled patients

Inclusion criteria:

- ≥ 250 GAA repeats in *FGF14*
- At least 18 years of age
- SARA total score > 3
- No previous use of fampridine

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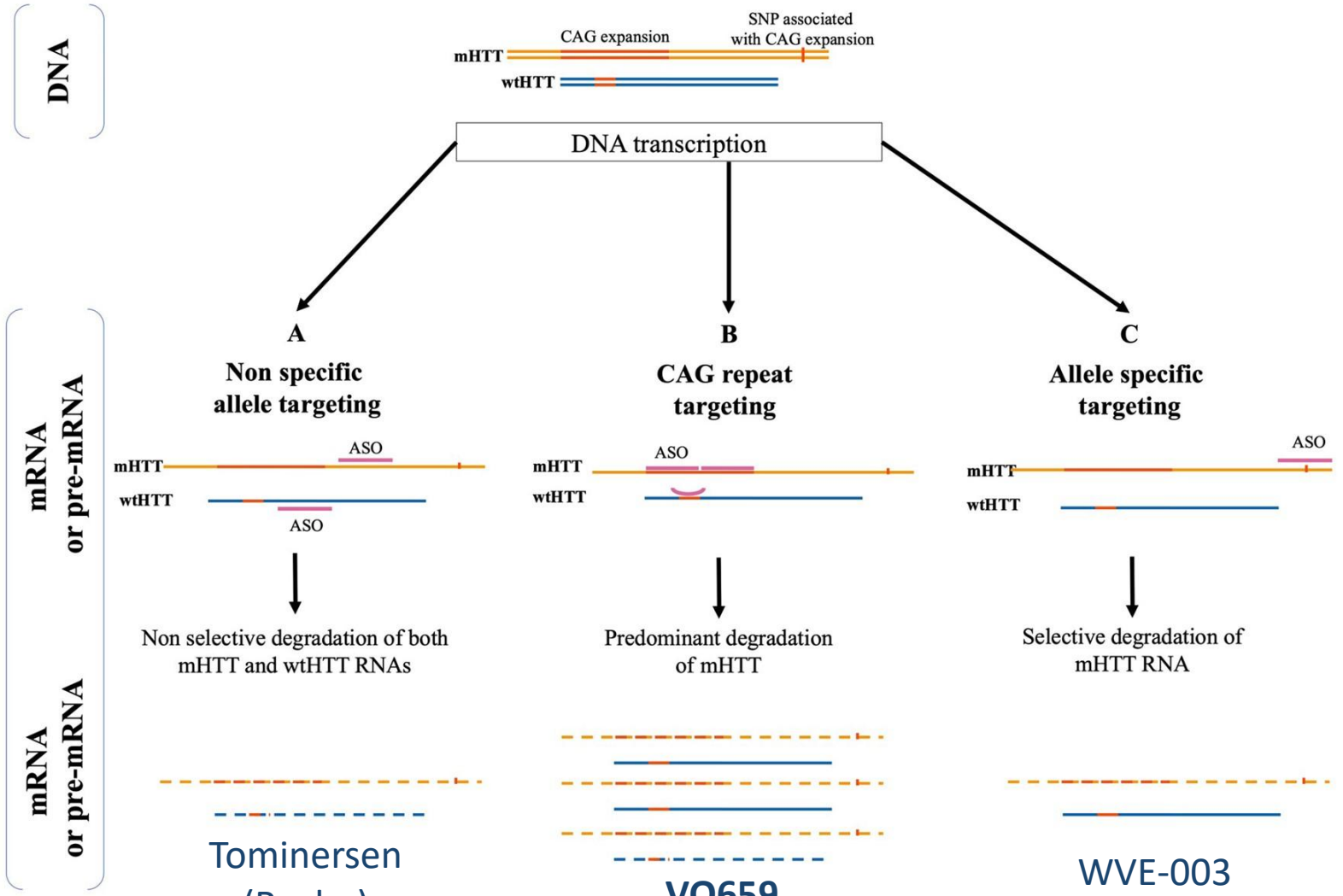
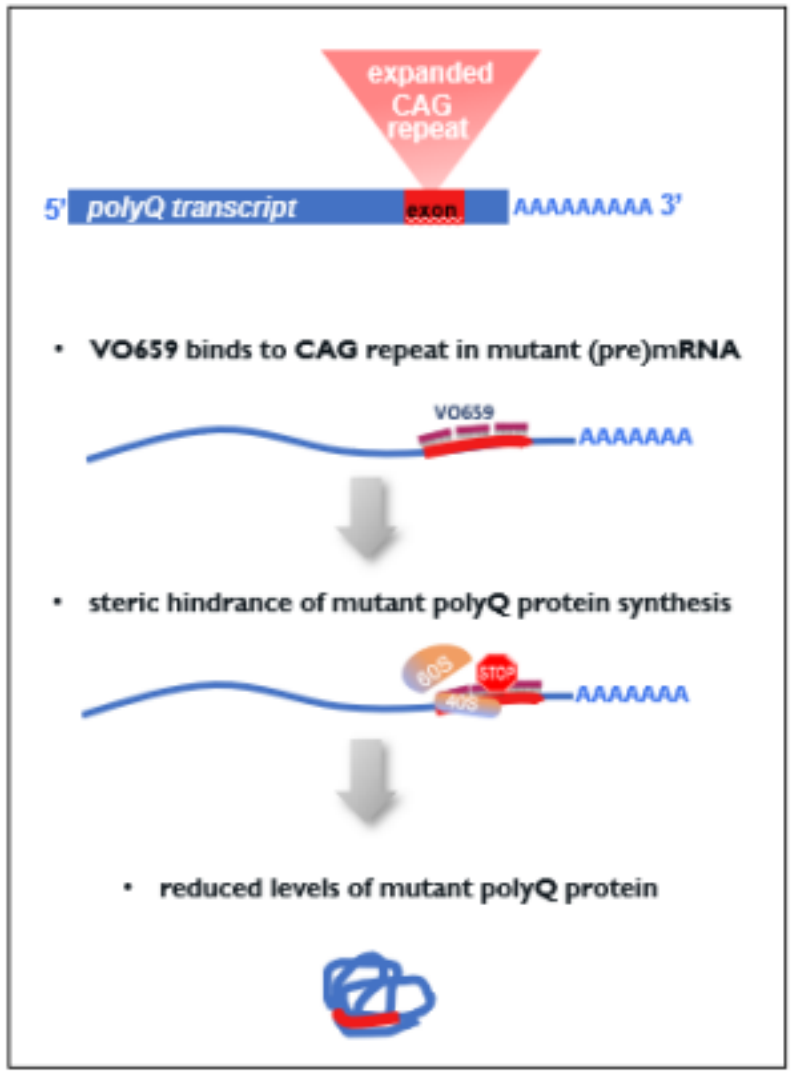
Treatment with Antisense Oligonucleotides (ASO)



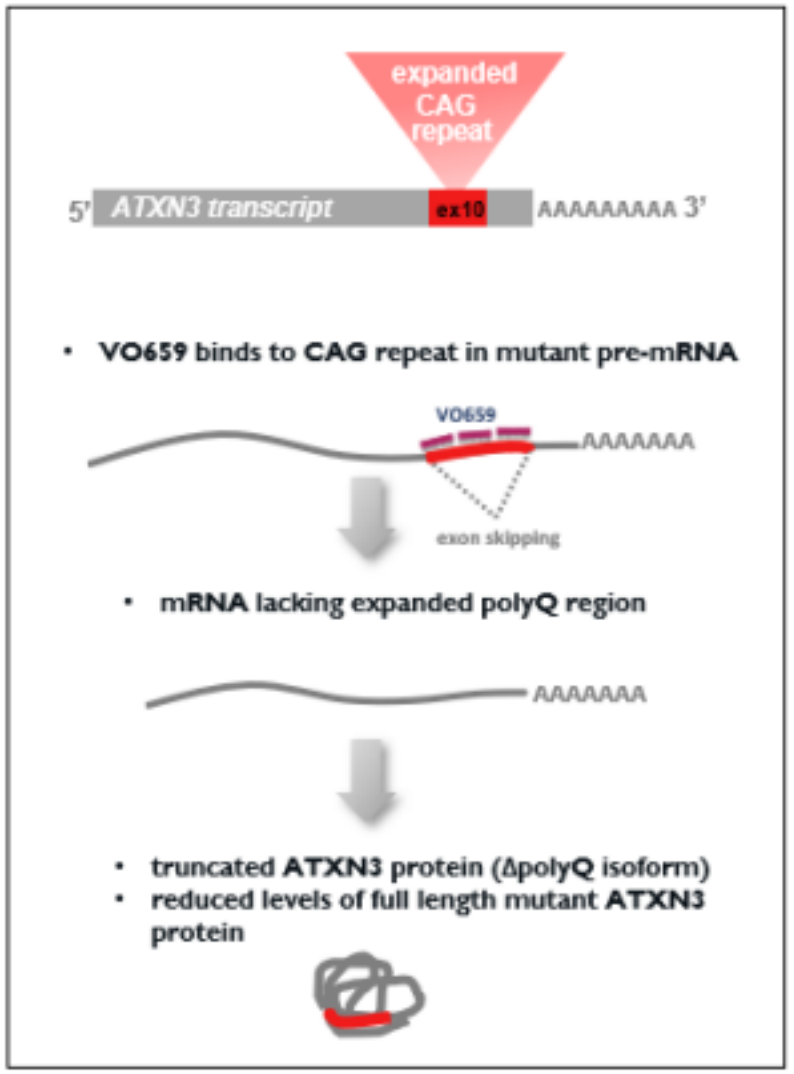
Brunet de Courssou et al., Brain 2022

VO-659: ASO in SCA1 and SCA3

MoA in majority of polyQ disorders: steric hindrance of protein synthesis



MoA in SCA3: exon skipping



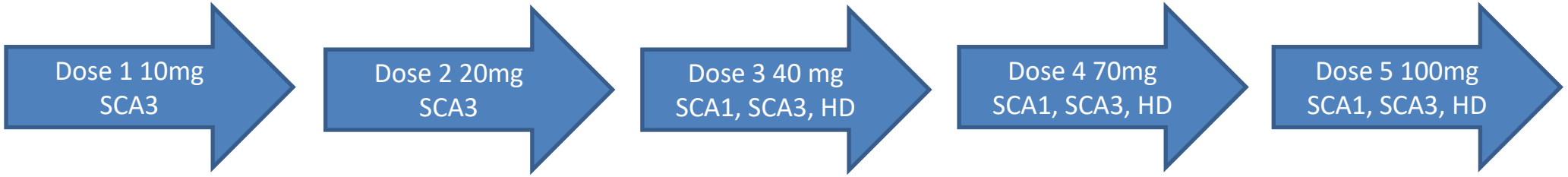
VO-659 VICO study design

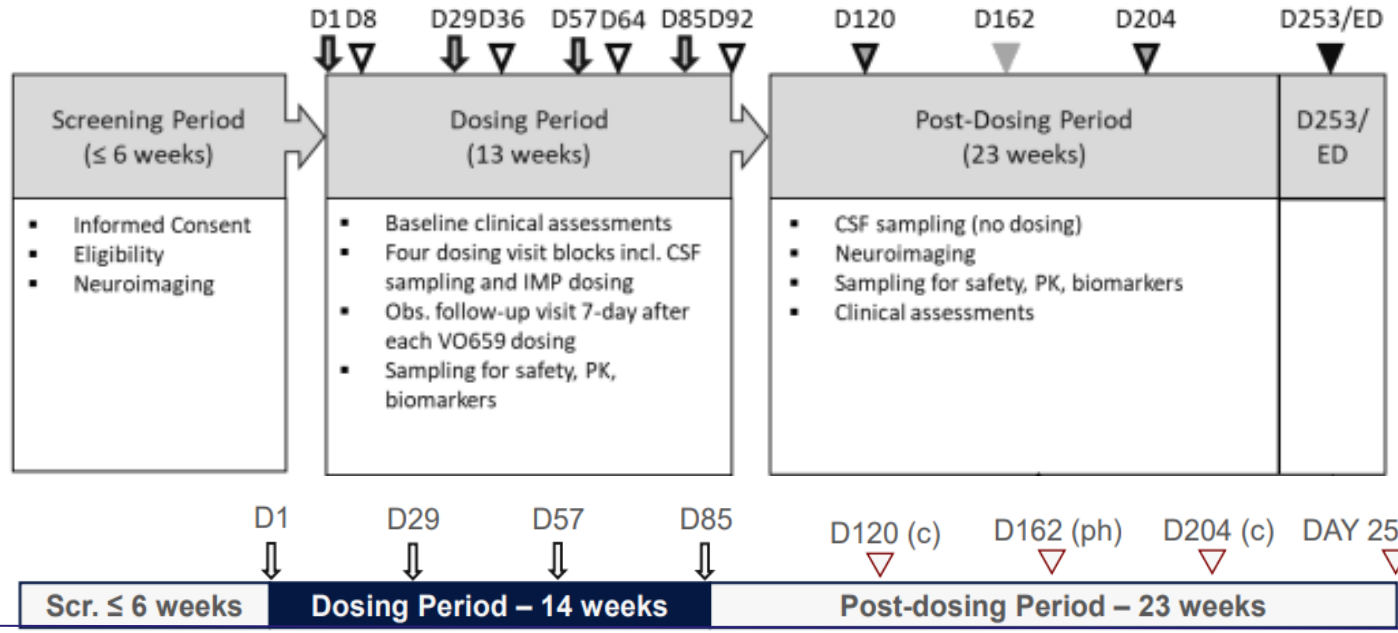
Phase 1/2a, open-label trial

- Sentinel participant
- Dose escaladation
- IDMC review
- 7 countries, 15 sites

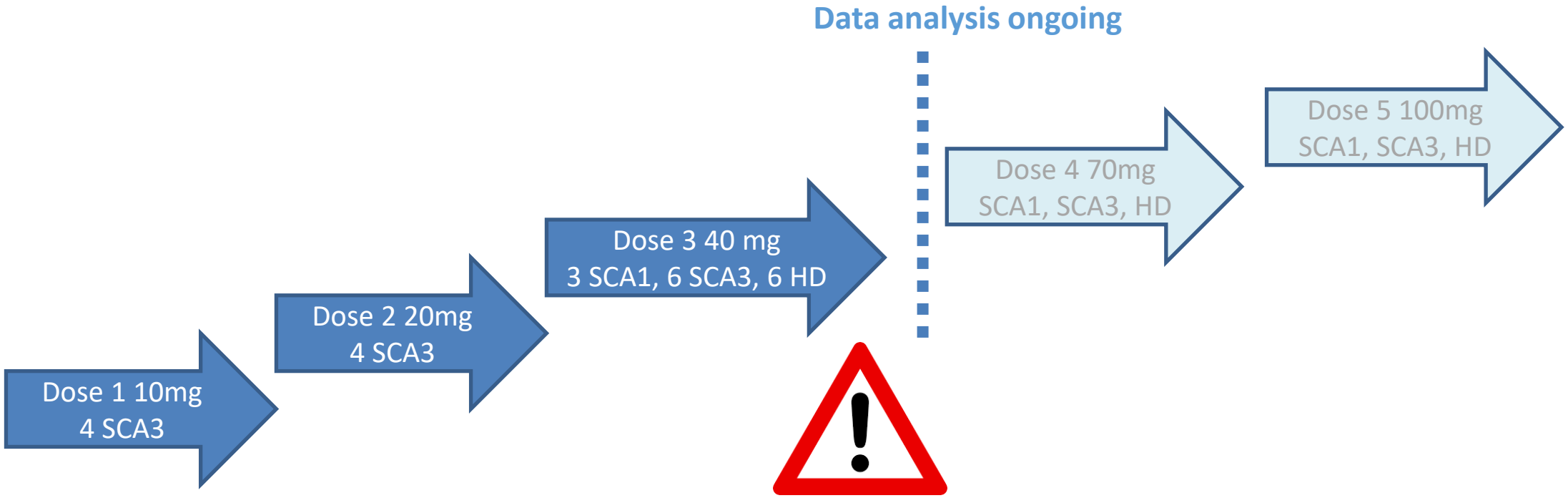
Inclusion criteria

- 25-60 years old
- SCA1/3: SARA 3 – 18
- HD: TFC 11-13 and DLC=4





VO-659: safety concerns



Dose 3

- Several cases of lumbosacral radiculitis = SUSARs
- Increased ventricular volume
- Increased protein levels in the CSF

In HD:

- ➢ Average reduction of 28% in mHTT in the CSF at day 85
- ➢ No prolonged increase in NfL in the CSF

VO-659: revised protocol

	Doses	
	# of doses	Total dose VO659
Cohort 1: 10mg VO659 (D1, D29, D57, D85)	4	40mg
Cohort 2: 20mg VO659 (D1, D29, D57, D85)	4	80mg
Cohort 3: 40mg VO659 (D1, D29, D57, D85)	4	160mg
Cohort 4: Randomization 1:1 to • 4a: 3x 20mg VO659 (D1, D85, D169) • 4b: 2x 40mg VO659 (D1, D169)	 3 2	 60mg 80mg
Cohort 5: 60mg VO659 single dose (D1)	1	60mg

Other gene silencing trials in SCAs

arrowhead pharmaceuticals

SCA2
Ongoing

- **Phase 1 Placebo-Controlled**, Dose Escalating Study to Evaluate the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of ARO-ATXN2
- **Recruiting sites**: Canada, Australia, New Zeeland, France, Germany, Italy, Spain, Taiwan
- ≥33 CAG repeats in *ATXN2*
- **SARA score ≤ 14**
- **Single dose** of ARO-ATXN2 by intrathecal administration (10, 25, 50, 100 mg) or placebo
- Double-stranded small interfering RNA (siRNA) duplex conjugated to a lipid

Biogen

SCA3

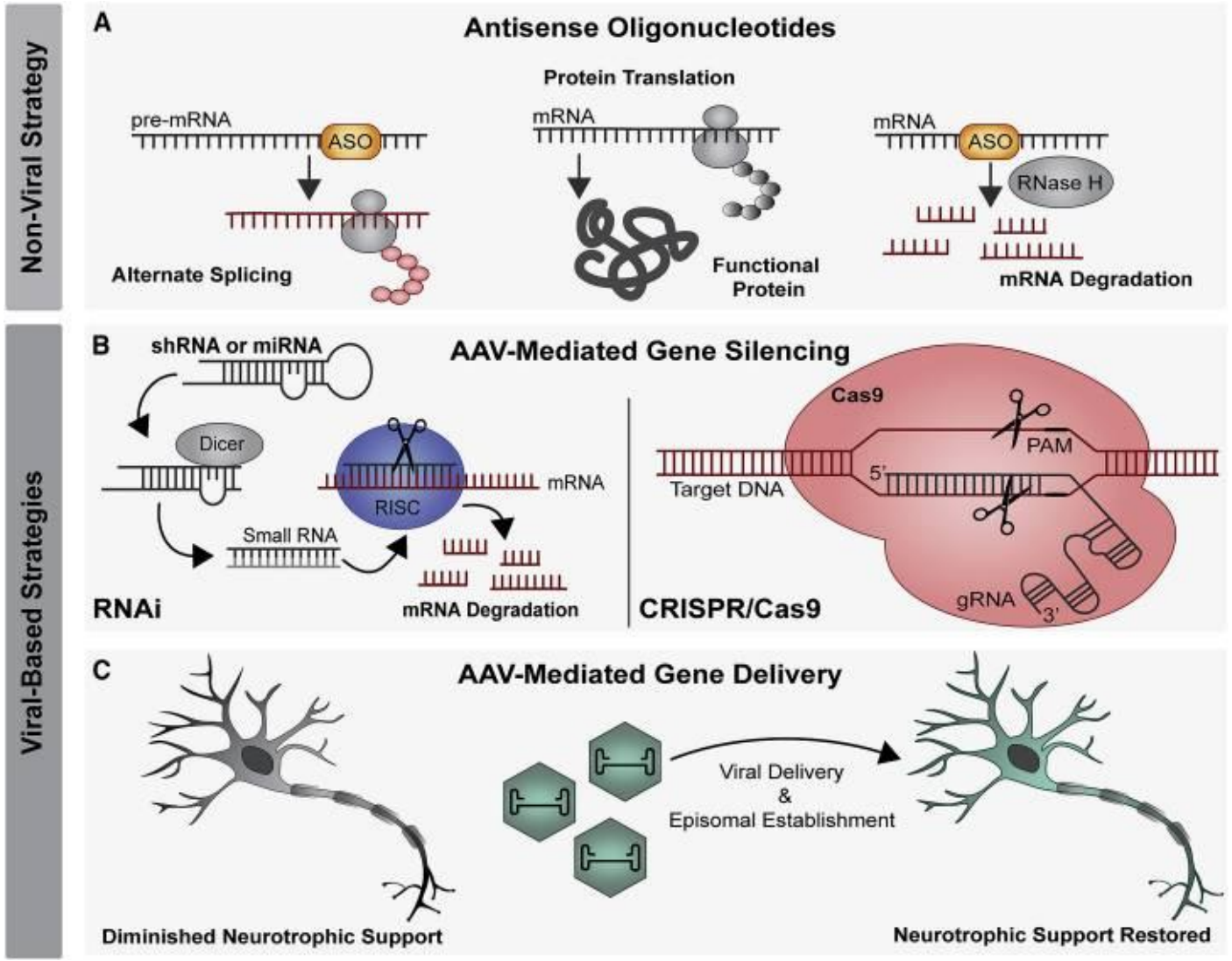
- Phase 1, Placebo-controlled, Dose Escalating Study to Investigate the Safety, Tolerability, and Pharmacokinetics of BIIB132
- 6 countries, 20 sites
- ≥60 CAG repeats in ATXN3 gene.
- SARA score 3 to 15 (still ambulatory)
- Intrathecal administration of BIIB132or placebo every 4 weeks for 12 weeks
- Trial stopped "*The decision was made after careful assessment of the nonclinical safety data, clinical pharmacodynamic data, and future development of BIIB132.*"

Different approaches to gene therapy

Gene Silencing:
- ASO
- siRNA, shRNA
- miRNA

(no gene therapies)

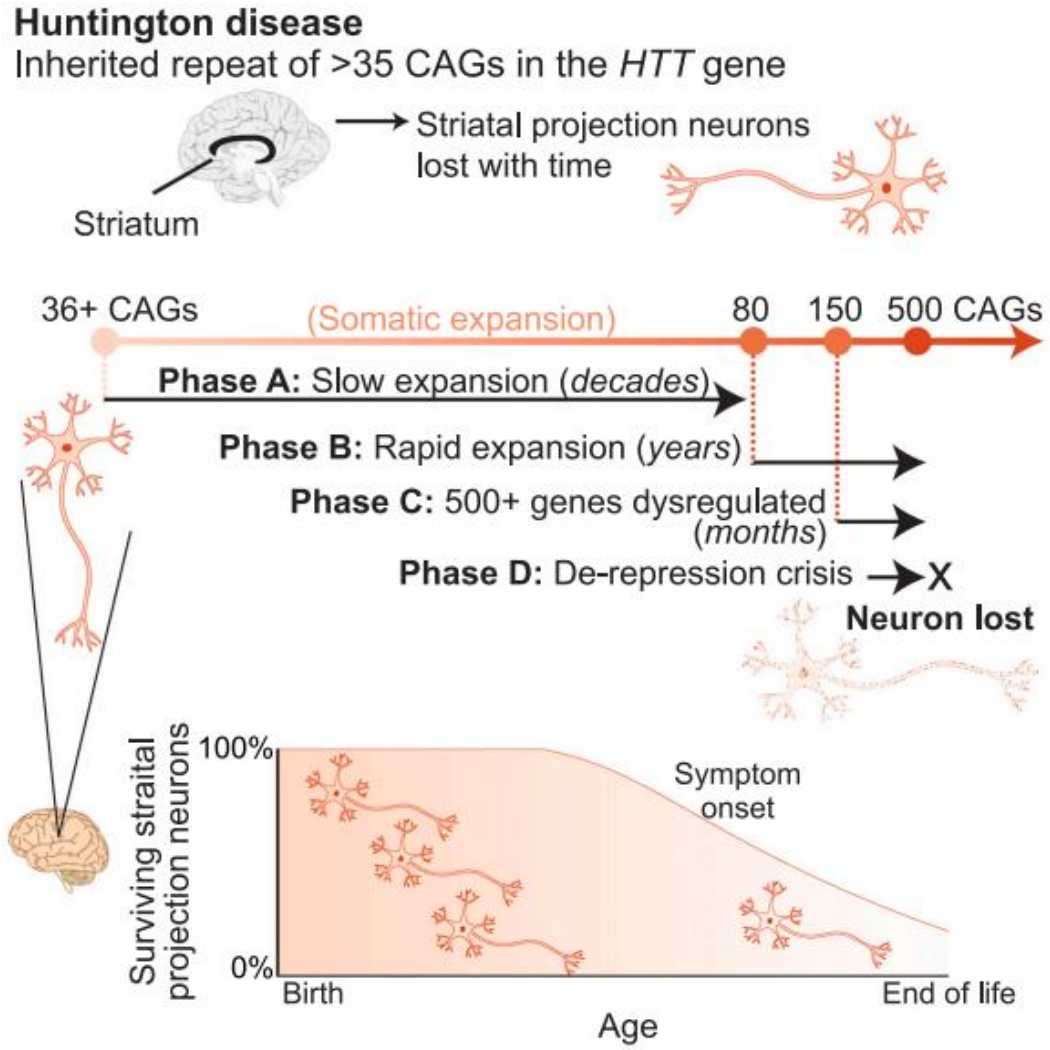
Gene Augmentation:
- Viral vectors: AAV, lentivirus
- Non-viral vectors: plasmids, liposomes, or nanoparticles
- CRISPRa, promoter editing
- Epigenetic therapy



Gene editing:
- CRISPR-Cas9
- TALENs
- Zinc Finger Nucleases
- Prime and base editing

Amado D et al, Mol Ther 2021

Target the somatic instability

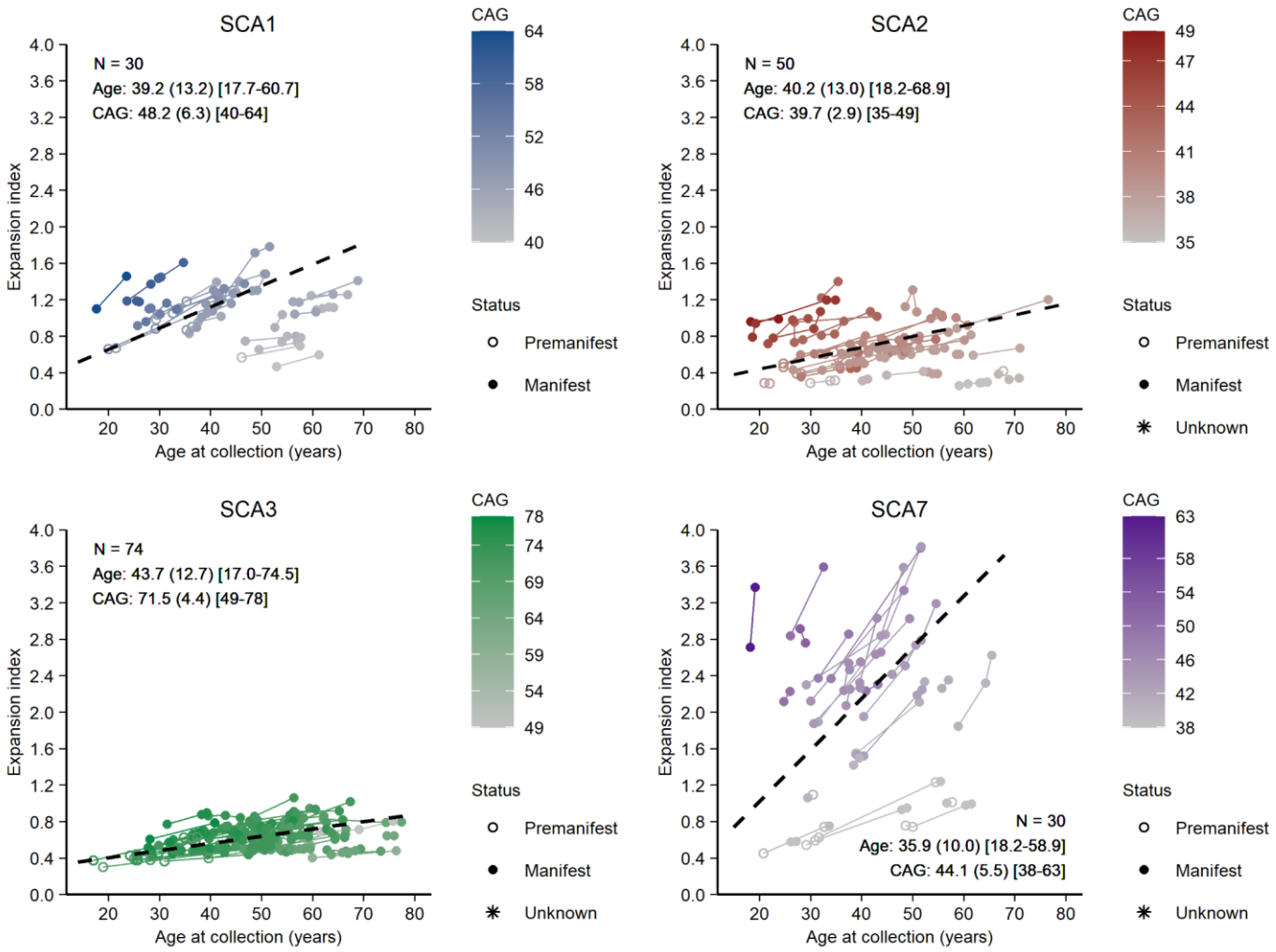


- Measurement of the CAG size at the cellular level and transcriptional deregulations

- The CAG repeat can expand into the hundreds in postmitotic projection neurons of the striatum in HD
- Somatic repeat expansion beyond 150 CAGs causes striatal neurons degeneration

Handsaker et al., Cell. 2025

Target the somatic instability



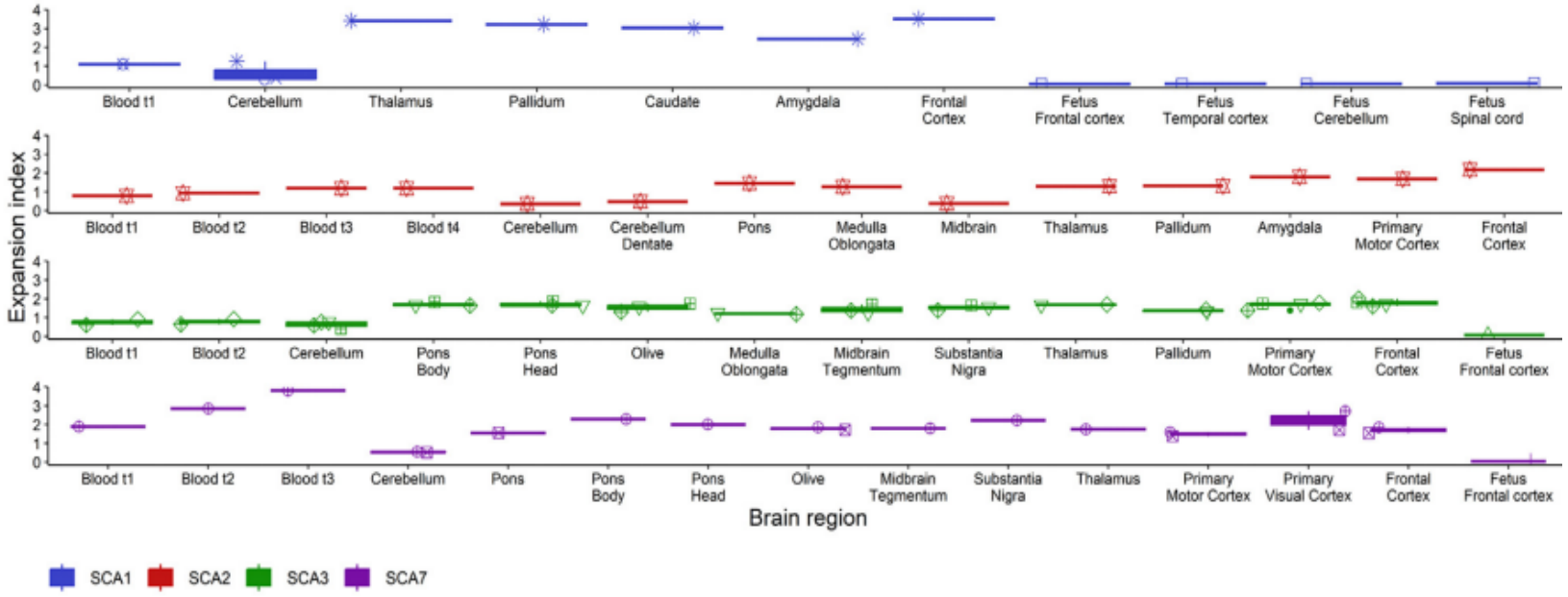
- Expansion increases with age
- Most striking increase for SCA7 patients
- Slowest increase for SCA3 patients

- The somatic instability is modulated by proteins involved in DNA repair pathways, particularly the **mismatch repair (MMR) system**.
- *MLH1*, *MSH3*, and *PMS2* play key roles: knockout models for these genes display reduced somatic instability.

Kacher et al., ASHG 2024

Target the somatic instability

Post-mortem PolyQ SCAs brains



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Kacher et al., ASHG 2024

QUESTION 2

At which stage do you think disease-modifying therapies are most likely to be effective?

- A. Advanced disease
- B. Moderate symptomatic stage
- C. Presymptomatic or very early symptomatic stage
- D. The timing does not matter

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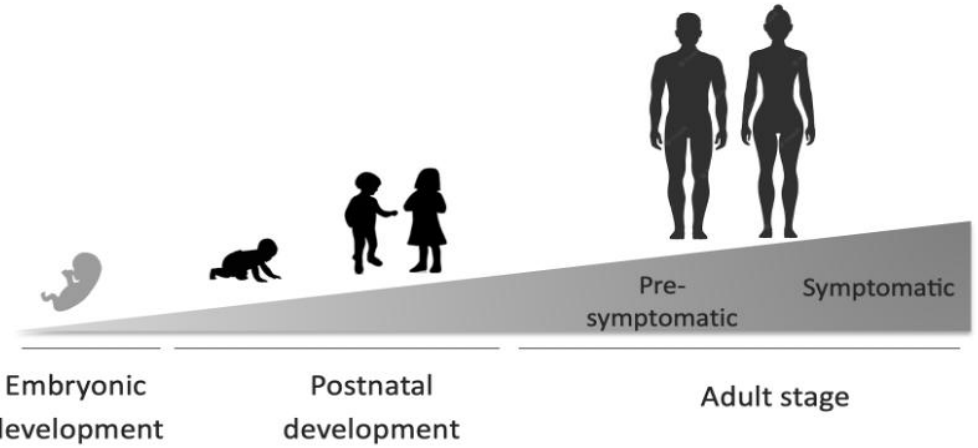
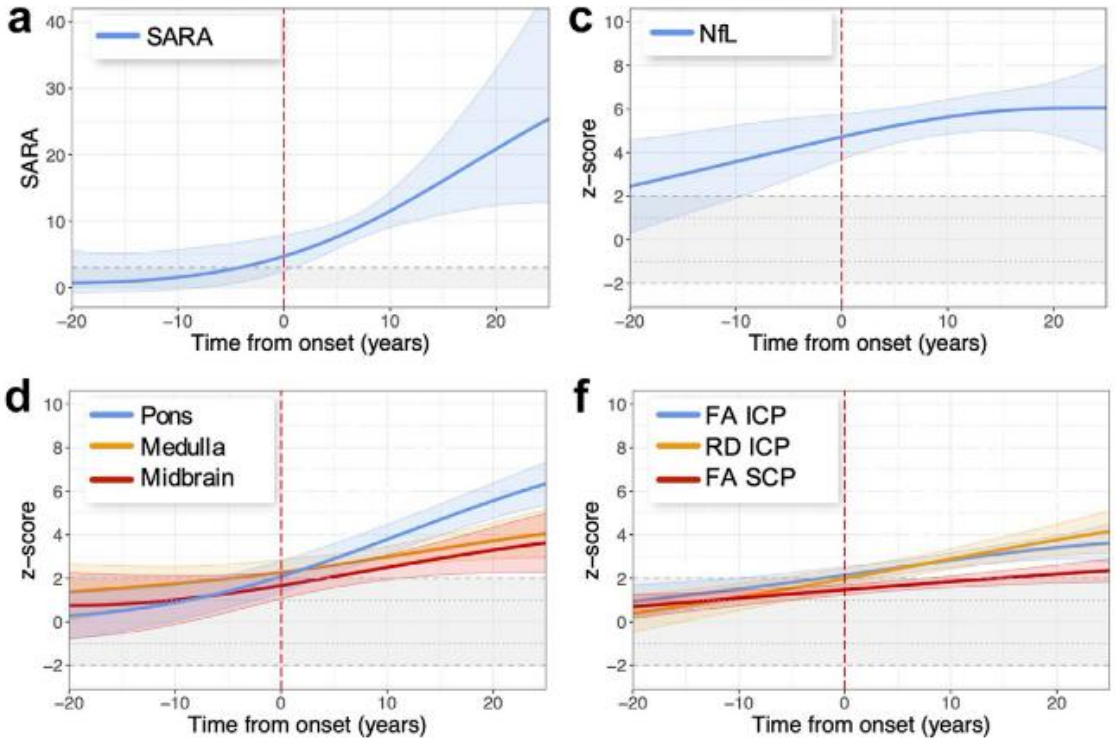
Perspectives

Preventive therapies?

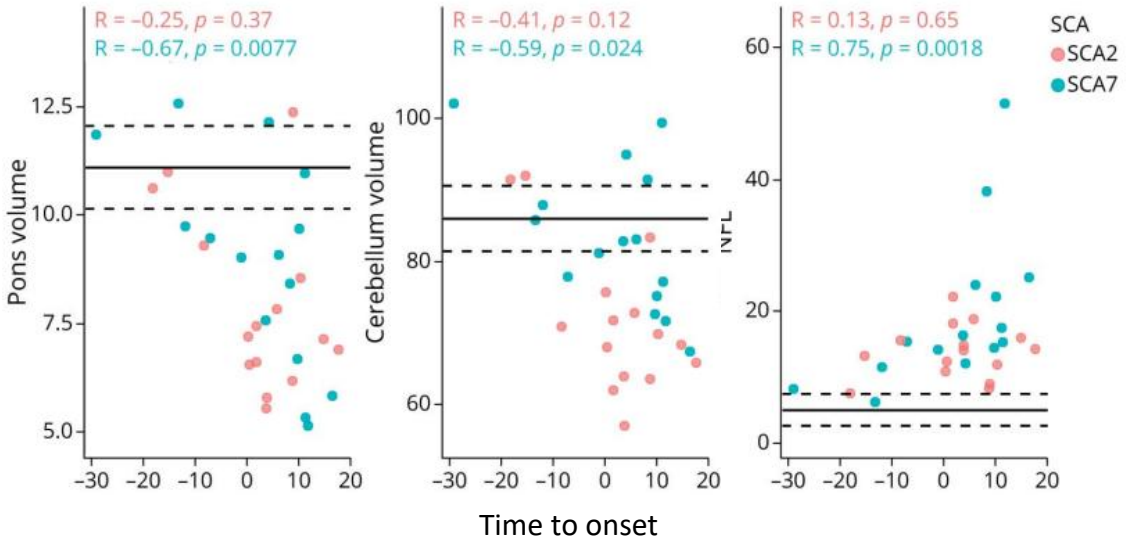


SCA3
ESMI cohort



Berger et al., Lancet Reg Health Eur 2025

SCA2-SCA7
ICM cohort



Coarelli et al., Neurology 2024

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QUESTION 3

Which biomarker do you think is currently the most promising for future clinical trials in spinocerebellar ataxias?

- A. Blood biomarkers (e.g., NfL)
- B. MRI biomarkers
- C. Digital biomarkers
- D. Combination of biomarkers

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Conclusions

- Symptomatic treatments and multidisciplinary care remain the cornerstone of management
- Crucial to identify treatable ataxias early (e.g. AVED, CTX, NPC)
- Several emerging disease-modifying therapies are in development, especially for Friedreich's ataxia and SCAs
- ASOs and gene therapy show promise but face challenges in efficacy, delivery, and safety
- The future lies in precision medicine and preventive therapeutic strategies

Paris Brain Institute
ICM

International Parkinson and Movement Disorder Society

Thank you!

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