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P1 -Early real-life with Vutrisiran in ATTRv amyloidosis: a single center experience

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BACKGROUND AND AIMS:

Hereditary transthyretin amyloidosis with polyneuropathy (ATTRv) is caused by mutations in the TTR gene, leading to misfolded monomers that aggregate generating amyloid fibrils. In Sicily, the most common genetic variant is the Phe84Leu, differently from the rest of Italy. The clinical phenotype is heterogeneous, characterized by a multisystemic disease affecting the sensorimotor and autonomic functions along with other organs. Vutrisiran is a small interfering RNA acting as a TTR silencer approved for the treatment of ATTRv. The patients switched from Patisiran, same active principle of Vutrisiran but administrated through intravenous injection every 21 days, to Vutisiran, more convenient because subcutaneous every 3 months.

METHODS:

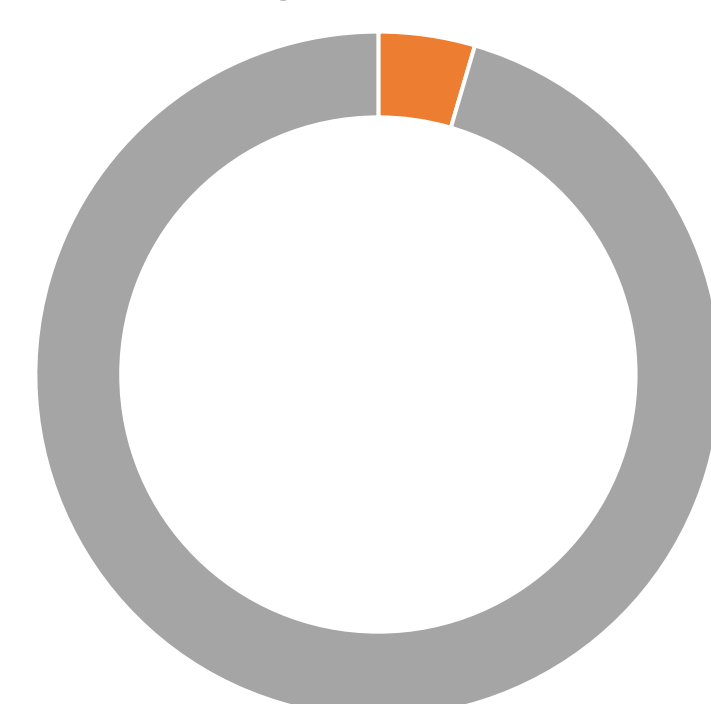
Twenty-two patients affected by ATTRv amyloidosis ($70,51 \pm 6.3$ years, twelve males) were evaluated before the start of therapy with Vutrisiran and at follow-up, performed after 7.9 months on average. The clinical and instrumental evaluation included body weight; Neuropathy Impairment Score (NIS); Karnofsky performance status (KPS); Norfolk QOL Questionnaire; COMPASS 31; Computed-Aided Triage (CADT); Six-minute walking test (6 MWT); Nerve Conduction Studies; Sudoscan. The evaluation of the degree of satisfaction was done through a close-ended questionnaire of 15 questions.

RESULTS:

The patients globally showed an unchanged clinical and instrumental picture at follow-up evaluation. However, Norfolk scale revealed significant changes before and during treatment and NCS showed a trend toward improvement in distal amplitude of the left medial nerve ($p=0.059$). In addition, we remark after the administration, no side effects were mentioned at the site of injection. And only one patient reported a systemic adverse effect. This result appeared different from Patisiran, which mainly used to cause drowsiness, hypertension or dizziness for days after the administration due to premedication. The satisfaction questionnaire highlighted, according to the patient's opinion, more comfortable administrations and an easier way to schedule with vutrisiran.

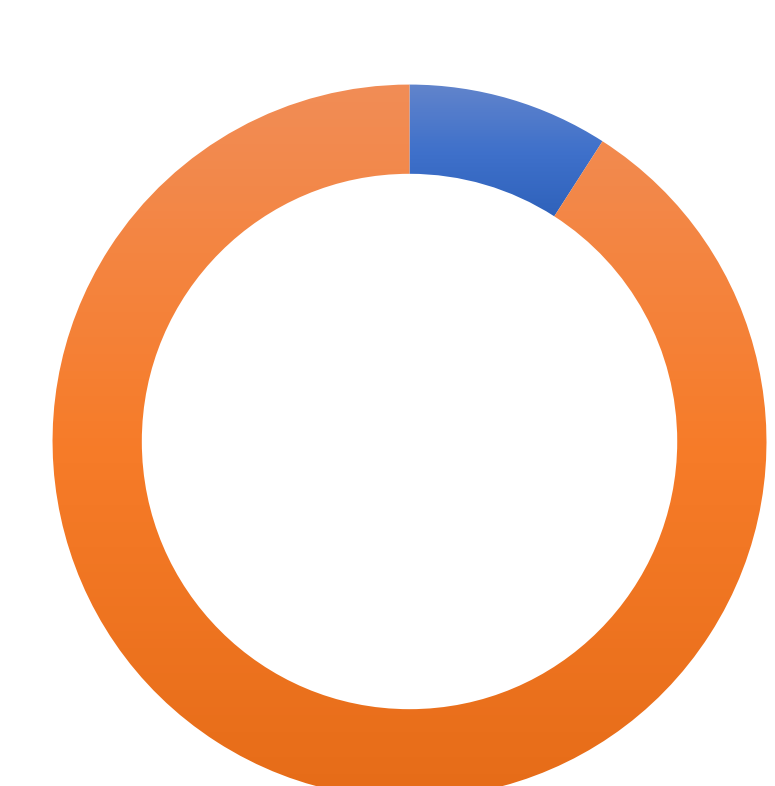
Patient code	Age (years)	Sex	Duration of Disease (Months)
001	69,26	M	13
002	59,18	M	102
003	87,54	F	28
004	70,88	F	48
005	72,94	M	108
006	70,19	F	48
007	69,23	F	48
008	73,20	F	54
009	62,86	M	51
010	66,78	M	51
011	71,07	F	60
012	73,70	F	53
013	59,74	F	30
014	74,87	M	60
015	73,59	M	10
016	74,29	F	36
017	59,31	M	80
018	75,10	M	69
019	71,56	M	44
020	74,90	M	35
021	70,73	M	6
022	70,51	F	87

Distribution of adverse events in patient



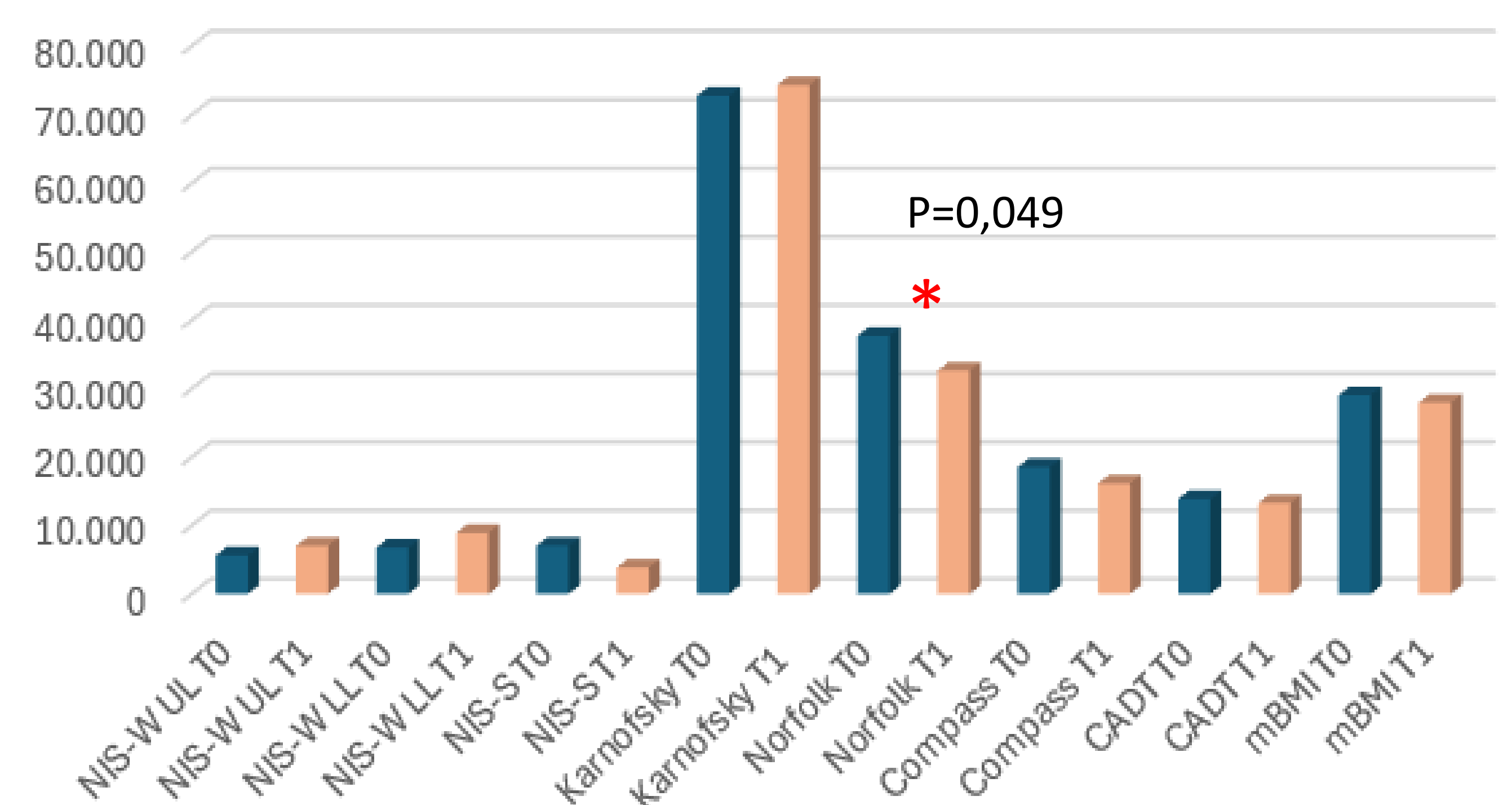
■ infusione site reaction
■ systemic reaction
■ No AEs

Preferred route of administration

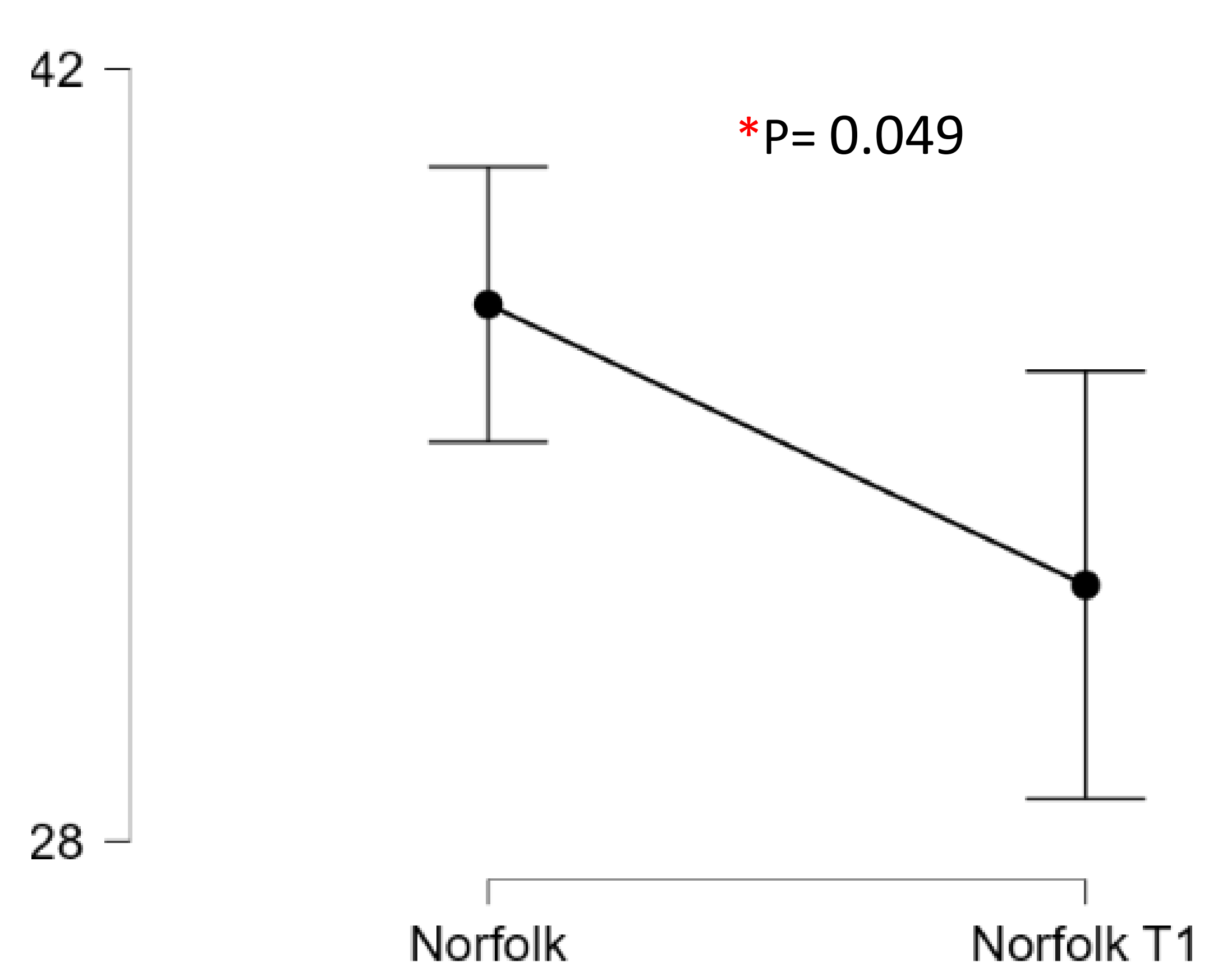


■ intravenous ■ subcutaneous

Clinical scores. T0 pre VUTTR; T1 post VUTTR



Norfolk - Norfolk T1



CONCLUSIONS

Vutrisiran has shown itself as a safety medication and as a stabilizer of the clinical and instrumental picture in Sicilian phenotypes. No patients presented a worsening of symptoms during maintenance treatment with vutisiran. In fact, patients showed a slight improvement on quality of life.

Moreover, according to the more convenient delivery compared to Patisiran, patients referred a remarkable satisfaction about the drug administration way.

References:

1) Patisiran in ATTRv amyloidosis with polyneuropathy: "PatisiranItaly" multicenter observational study Vincenzo Di Stefano¹ · Pietro Guaraldi² · Angela Romano³ · Giovanni Antonini⁴ · Alessandro Barilaro⁵ · Chiara Briani⁶ · Marco Burattini⁷ · Ilaria Cani² · Giulia Carlini⁸ · Marco Ceccanti⁹ · Vittoria Cianci¹⁰ · Pietro Cortelli² et al., Journal of Neurology (2025) 272:209 <https://doi.org/10.1007/s00415-025-12950-3>.

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