Tenosynovial Giant Cell Tumor Observational Platform Project (TOPP): 2-Year Observational Sub-Analysis Of Age And Patient-Reported Outcomes From An EU/US Prospective Registry

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Objective

Tenosynovial giant cell tumor (TGCT) mainly affects a younger working population, and this analysis assesses how quality of life (QoL) can be affected by age

Conclusions

- This is the first analysis to describe the impact of age on QoL as a 2-year follow-up in diffuse-type TGCT (dt-TGCT) patients
- The youngest group (<40 years) showed the most improvement in patient-reported outcomes (PRO) over the 2-year observational period, potentially due to a higher percentage of younger patients being actively treated or progressive degenerative arthritis being present in older patients
- The oldest group (≥ 60 years) who were predominantly in the Wait-and-See treatment strategy, had a decrease in EuroQoI-5 Dimension Visual Analog Scale (EQ-5D VAS) scores over time, and more discomfort
- As TGCT is most present in a younger working population, these findings could be considered in future studies
- This study underscores the need to consider age when assessing PROs in TGCT patients

Plain language summary



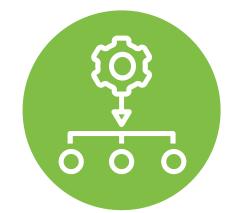
Why did we perform this research?

Previous work from this Tenosynovial Giant Cell Tumor Observational Platform Project (TOPP)* demonstrated that diffuse-type tenosynovial giant cell tumor (dt-TGCT)⁺ can have a negative impact on patient quality of life (QoL).¹⁻³ Because this disease primarily affects a younger working population,⁴ we assessed the potential impact of age on QoL in patients with dt-TGCT



How did we perform this research?

Using the patient population from the TOPP Registry (N = 176),^{1,2} we performed analyses and collected results from patient questionnaires focusing on QoL, tumor status, and treatment options from 3 different age groups (<40 years, 40–59 years, ≥60 years)



What were the findings of this research and what are the implications?

The results indicated that the younger patients demonstrated the most improvement in QoL—specifically, less pain intensity and severity as well as lower worst stiffness compared to the oldest group. This could be attributed to the younger working populations opting for more active treatment options. The frequency and severity of associated degenerative arthritis may have impacted these results. These findings can be applicable in future studies



Where can I access more information?

https://clinicaltrials.gov/ct2/show/NCT02948088

*This is an international study designed to evaluate the management of tenosynovial giant cell tumor.

[†]Diffuse-type tenosynovial giant cell tumor (dt-TGCT) is a debilitating disease that affects various joints in the body (most commonly the knee).

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- 1. Bernthal NM et al. Orphanet J Rare Dis. 2021;6(1):191.
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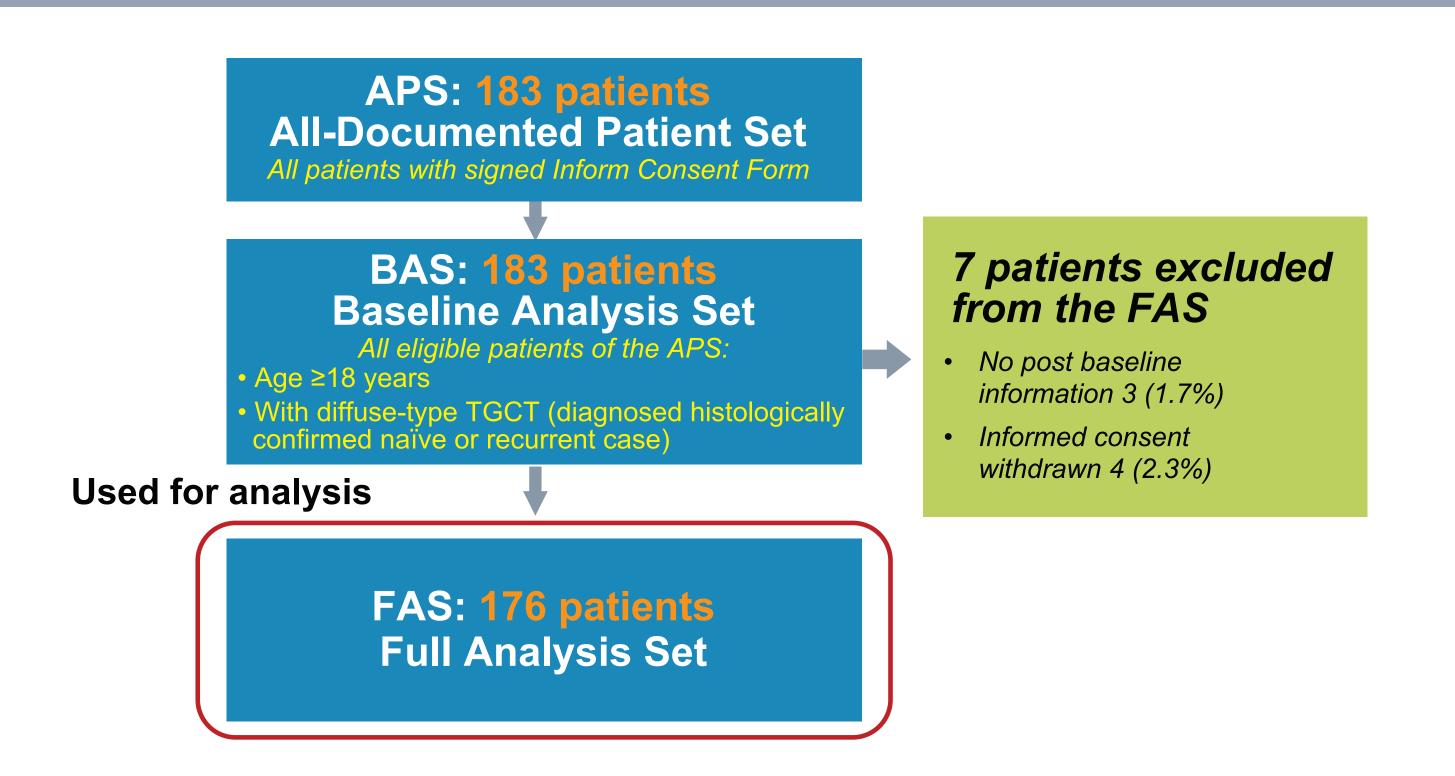


- Diffuse-type tenosynovial giant cell tumor (dt-TGCT) is a rare, locally aggressive neoplasm with a wide clinical spectrum. It generally affects a younger population and can considerably affect patients' quality of life (QoL)¹⁻⁶
- The prospective international Tenosynovial Giant Cell Tumor Observational Platform Project (TOPP) Registry described the impact of dt-TGCT on patient-reported outcomes (PRO) from a Baseline snapshot, and more recently, as a 2-year follow-up based on treatment strategies⁷⁻⁸

Results

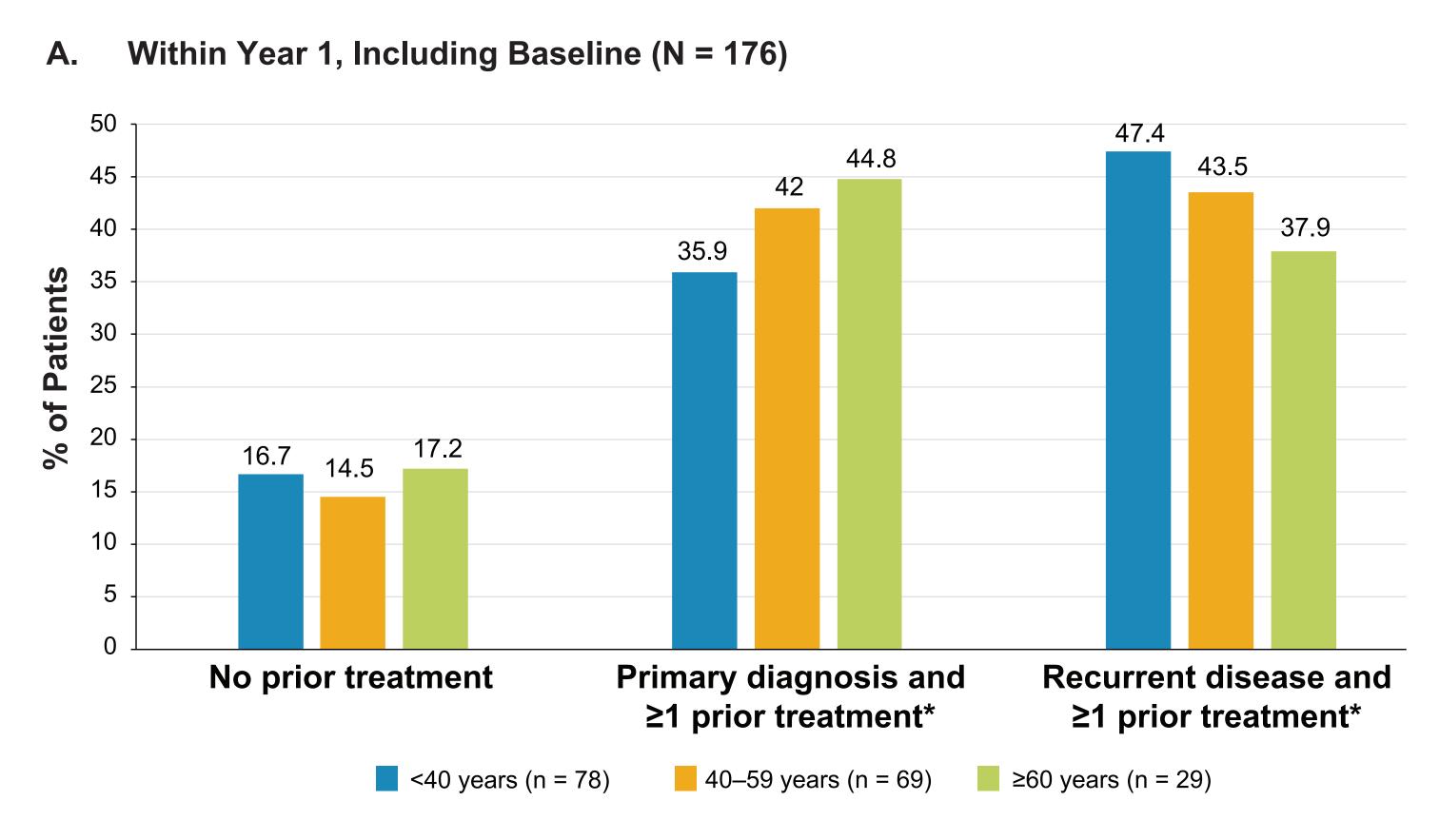
A total of the 183 patients (Baseline Analysis Set [BAS]) entered the study, of which 176 were included in the Full Analysis Set (FAS) (**Figure 2**)

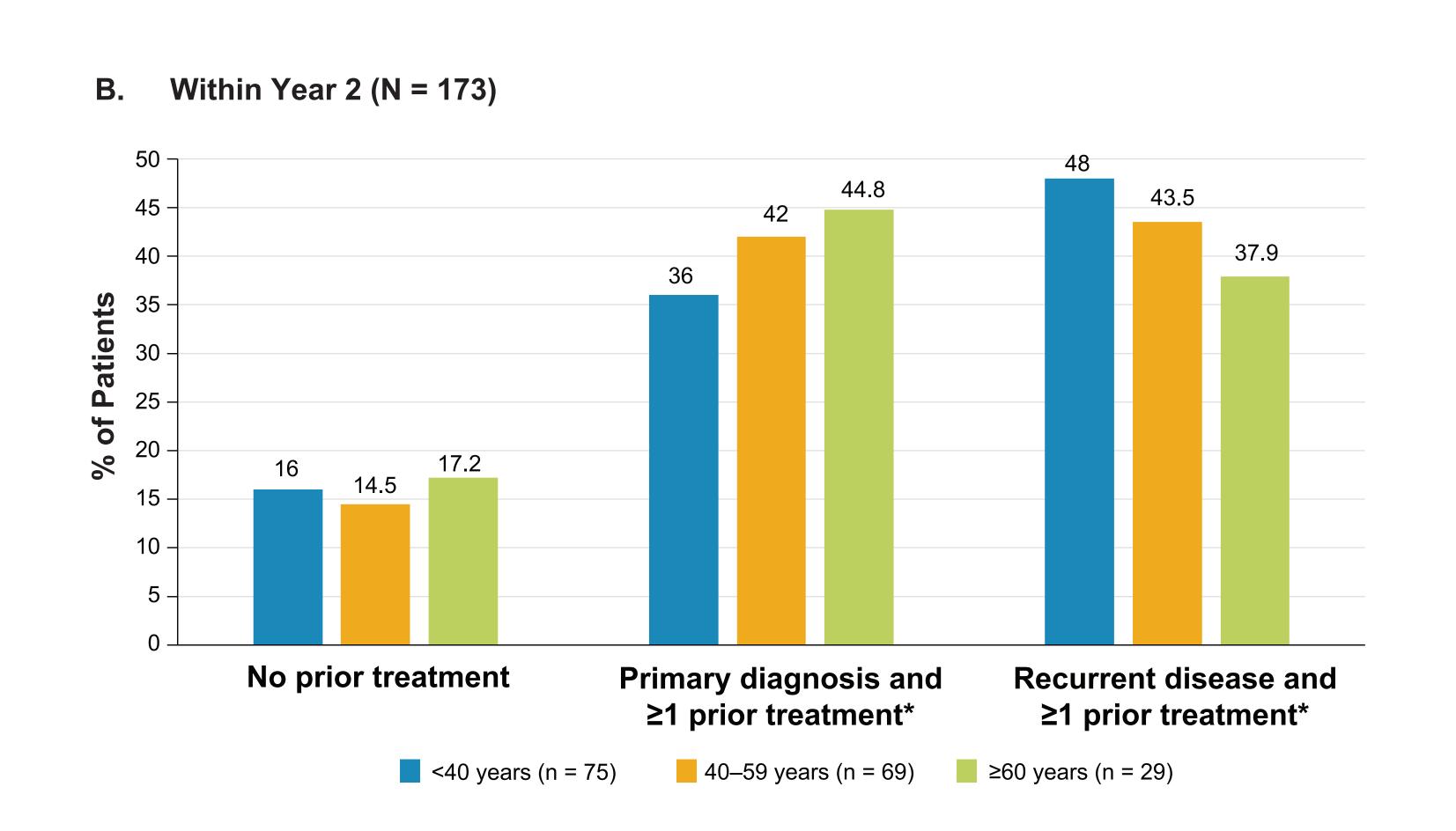
Figure 2. Patient Eligibility



The most common tumor status was recurrent disease and ≥ 1 prior treatment in patients <40 years (Within Year 1, 47.4%; Within Year 2; 48.0%) and patients 40–59 years (Within Year 1 and Within Year 2; 43.5%). For patients ≥60 years, primary diagnosis and ≥ 1 prior treatment was the most common tumor status (Within Year 1 and Within Year 2; 44.8%) (Figure 3A-B)







*Prior treatments included: Only Surgery. Only Systemic (pexidartinib, imatinib, nilotinib, investigator study medicine), Surgery + Other Treatment (radiotherapy or ⁹⁰Yttrium, or systemic treatment), and Other Treatment (radiotherapy or ⁹⁰Yttrium).

Methods

- TOPP was conducted at 12 sites (10 EU sites [7 European countries] and 2 US sites) that included adult patients with dt-TGCT seen in sarcoma referral centers
- Captured PRO measurements were assessed at Baseline, 6 months, 1 year,
- 18 months, and 2 years, and calculated as medians (Figure 1)
- PRO endpoints were:
- Brief Pain Inventory (BPI) Pain Interference, BPI Pain Severity, Worst Stiffness, EuroQol-5 Dimension Visual Analog Scale (EQ-5D VAS), EQ-5D, and Patient-Reported Outcomes Measurement Information System-Physical Function (PROMIS-PF)
- Patients were categorized by age: <40 years, 40–59 years, or ≥60 years

Table 1. Patient Demographics

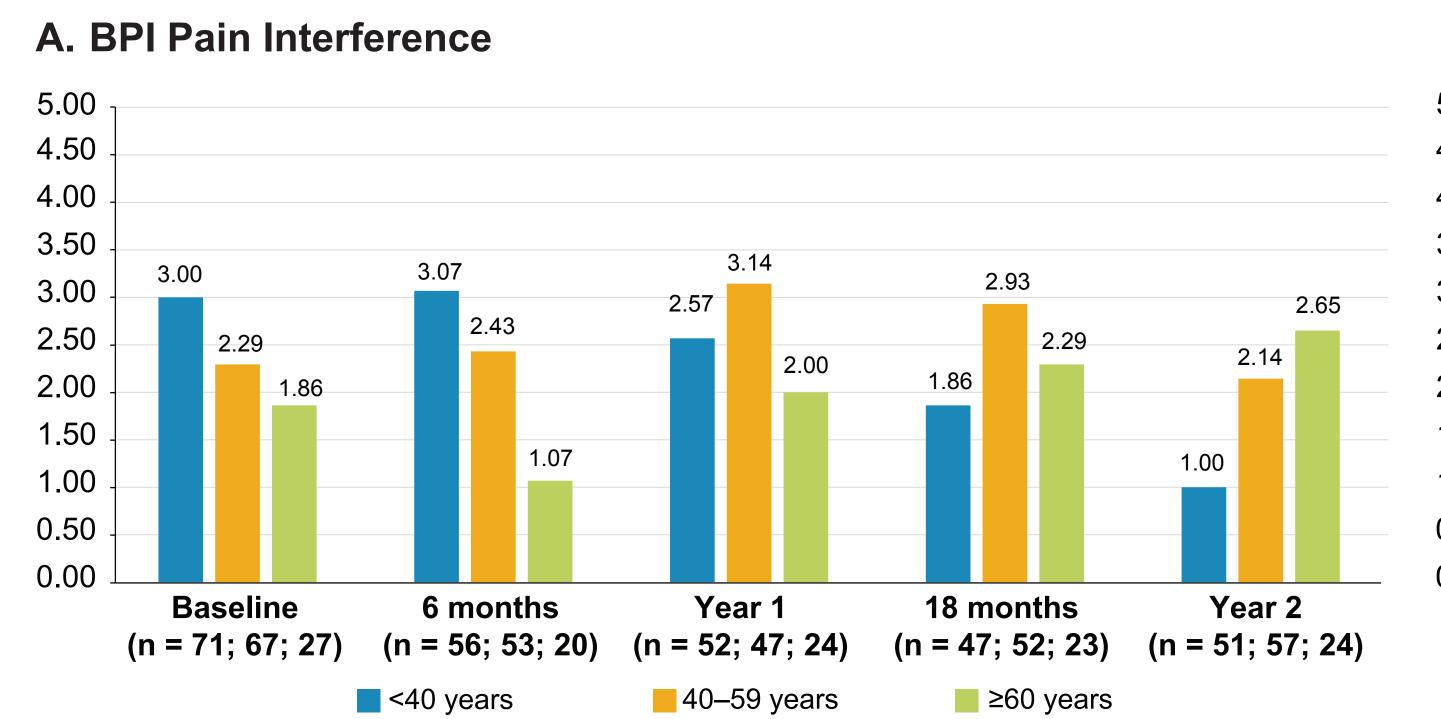
 Summary of Baseline demographics, tumor status, and treatment strategy of the FAS population (N = 176) (**Table 1**)

Features	(N = 176)
Age, mean, years (SD)	43.5 (±14.29)
Age, n (%)	
<40 years	78 (44.3)
40–59 years	69 (39.2)
>60 years	29 (16.5)
Tumor Status, n (%)	
Naïve (no prior treatment)	28 (15.9)
Primary diagnosis and ≥1 prior treatment*	70 (39.8)
Recurrent disease and ≥1 prior treatment*	78 (44.3)
Treatment Strategy, n (%)	
Only Surgery	43 (24.4)
Only Systemic	45 (25.6)
Surgery + Other	4 (2.3)
Radiotherapy	5 (2.8)
Wait-and-See	79 (44.9)
*Prior treatments included: Only Surgery, Only Systemic (pexidartinib, imatini study medicine), Surgery + Other Treatment (radiotherapy or ⁹⁰ Yttrium, or sys	

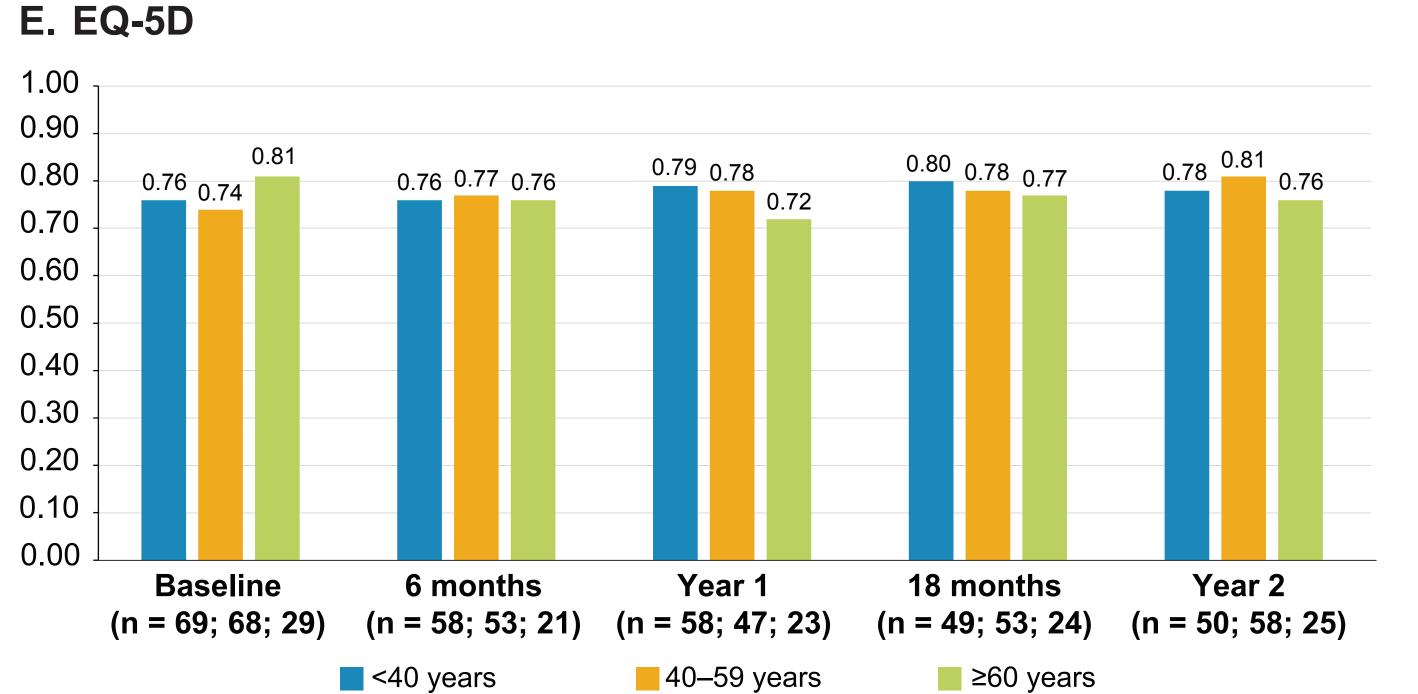
Treatment (radiotherapy or ⁹⁰Yttrium) SD = standard deviation.

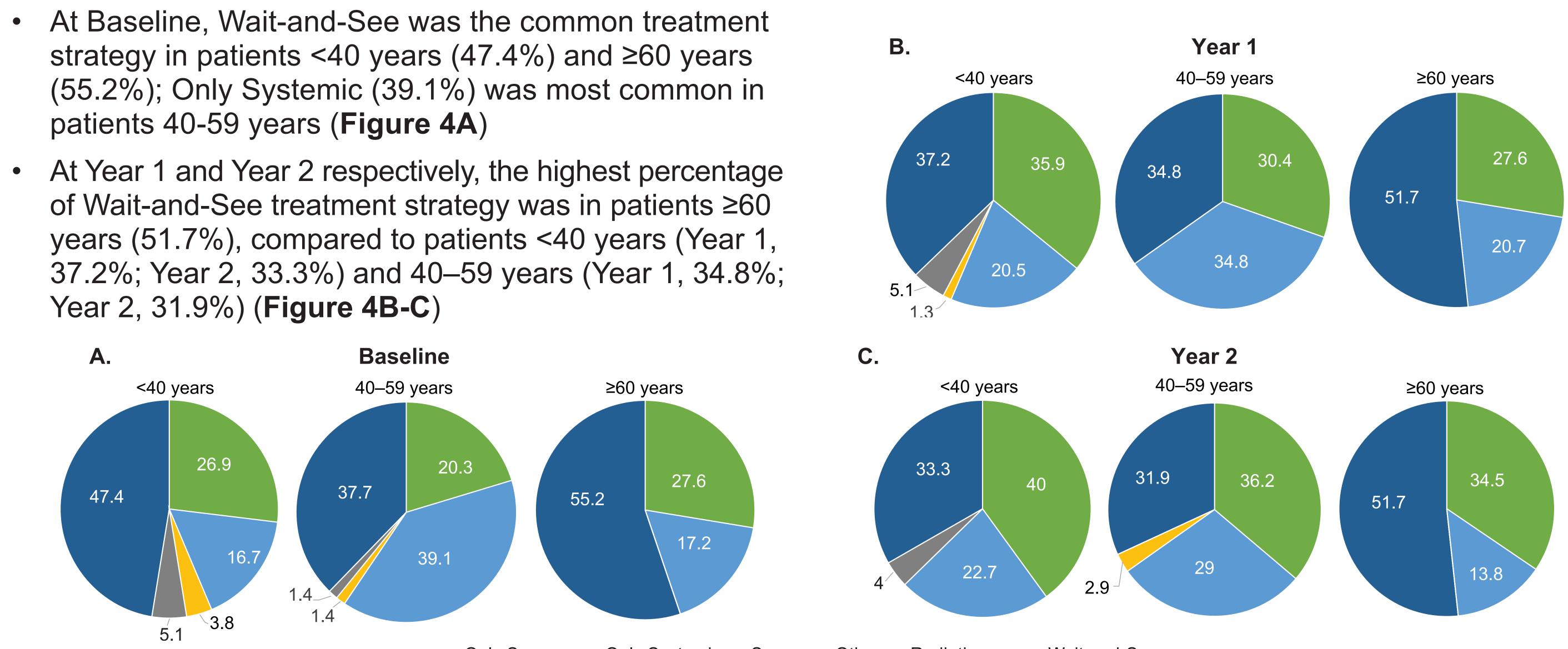
C. Worst Stiffness

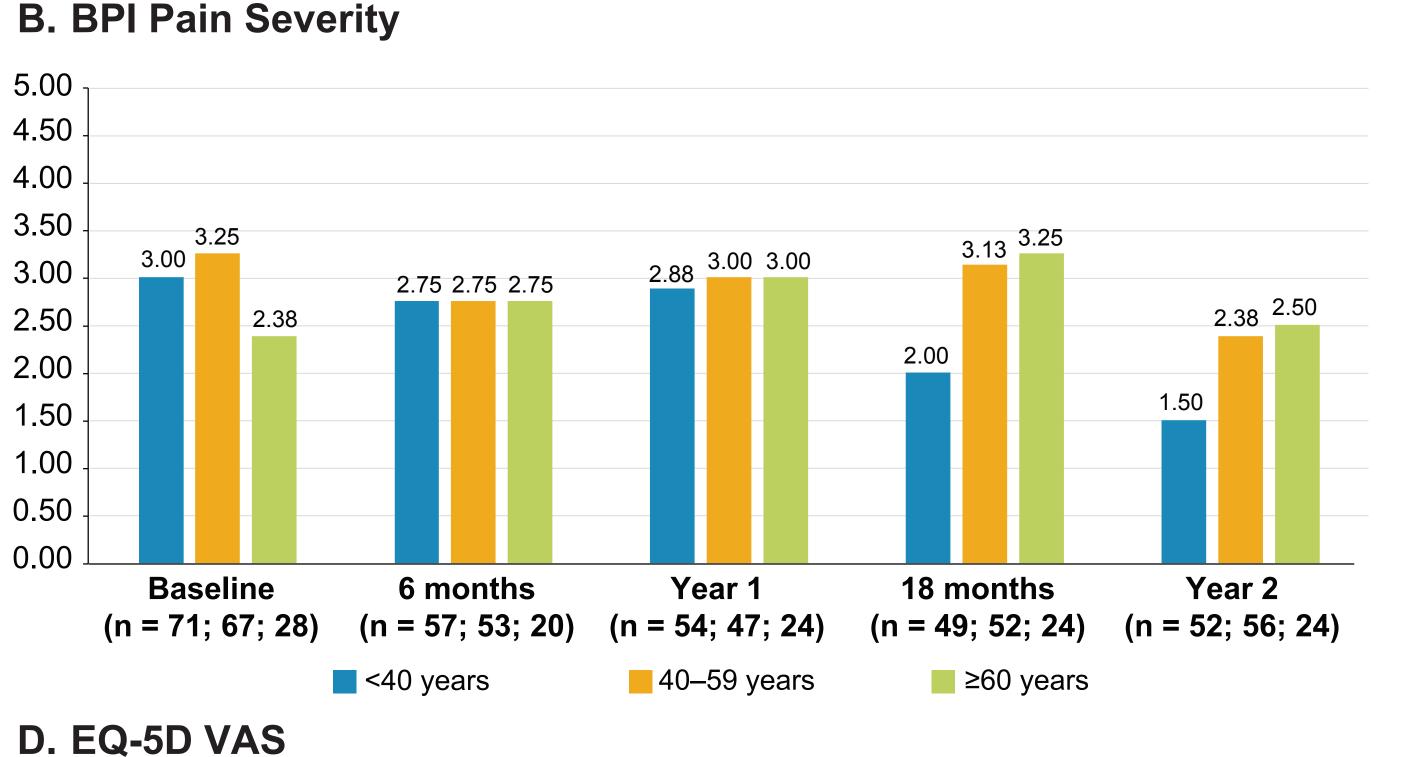
Figure 5. Patient-Reported Outcomes (Median Scores) by Age During 2-Year Observational Period



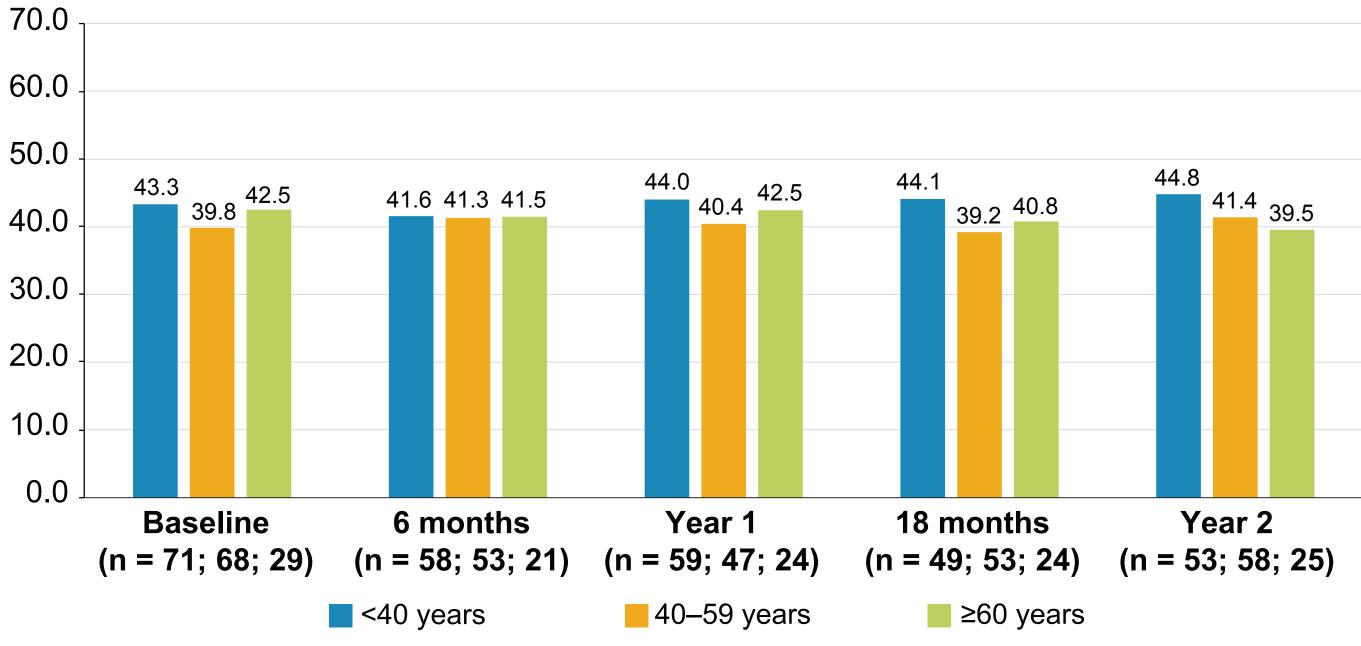
6.00 5.00 5.00 4.00 3.00 2.00 1.00 6 months 18 months Year 1 Year 2 (n = 58; 53; 21) (n = 57; 48; 24) (n = 48; 53; 24) (n = 52; 56; 25) (n = 71; 66; 27) ≥60 years <40 years</p>



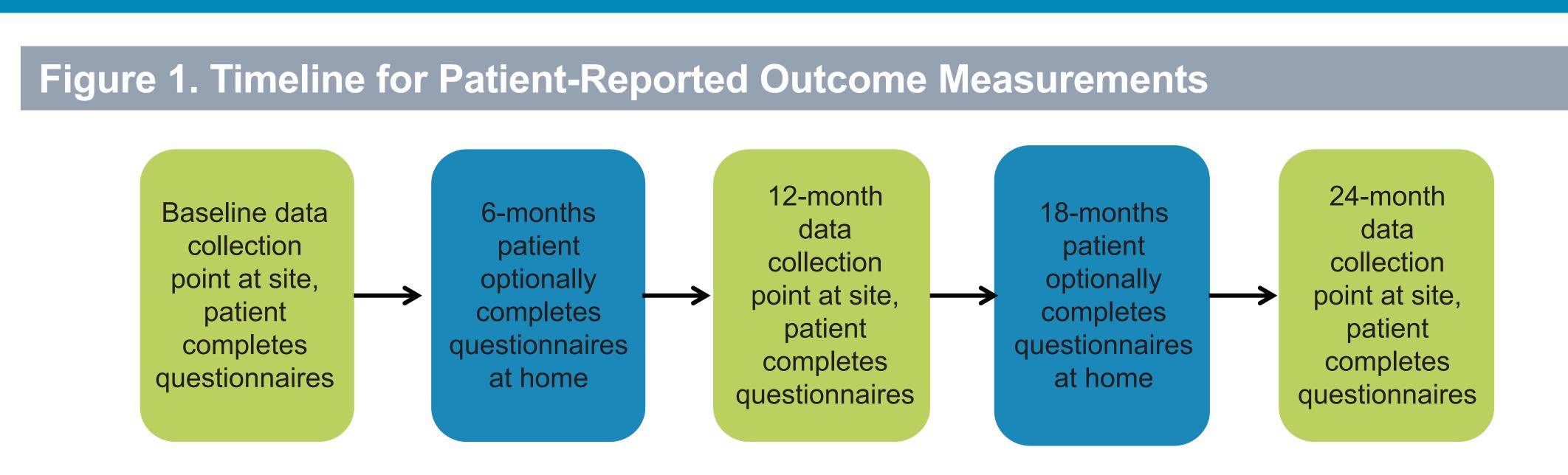




6 months Baseline Year 1 (n = 71; 68; 29) (n = 58: 54: 21) <40 years</p> F. PROMIS-PF



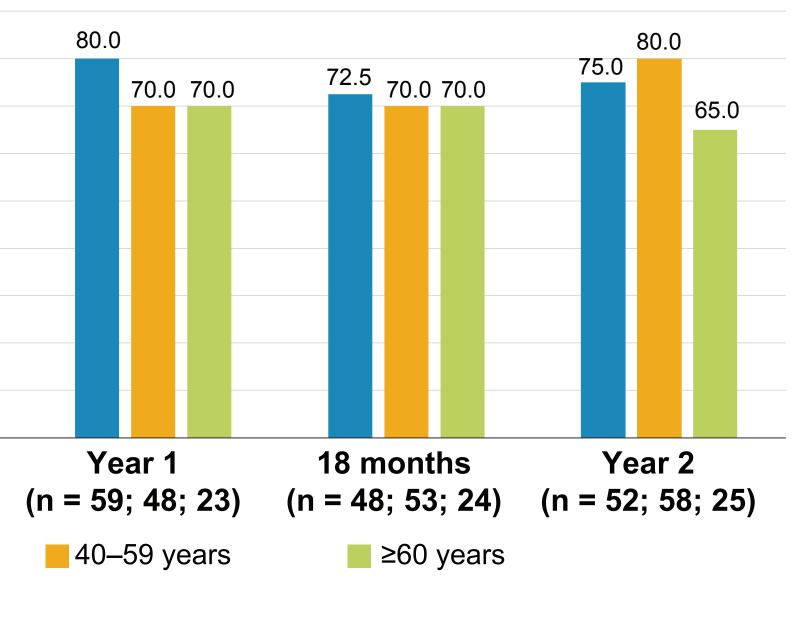
BPI = Brief Pain Inventory; EQ-5D VAS = EuroQoI-5 Dimension Visual Analog Scale; PROMIS-PF = Patient-Reported Outcomes Measurement Information System-Physical Function.



*Additional data collection points may occur at any time the patient visits the site, even if it is outside this schedule

Figure 4. Treatment Strategies by Age During the 2-Year Observational Period

Only Surgery Only Systemic Surgery + Other Radiotherapy Wait-and-See



- BPI Pain Interference scores improved in patients <40 years (Baseline, 3.00; Year 2, 1.00), whereas scores worsened in patients ≥60 years from Baseline (1.86) to Year 2 (2.65) (Figure 5A)
- BPI Pain Severity scores improved in patients <40 years from Baseline (3.00) through Year 2 (1.50) and in patients 40–59 years (Baseline, 3.25; Year 2, 2.38); there was no change in the group ≥ 60 years (**Figure 5B**)
- Worst Stiffness scores decreased in patients <40 years from Baseline (5.00) to Year 2 (3.00), and worsened in patients ≥ 60 years from Baseline (3.00) to Year 2 (4.00) (Figure 5C)
- EQ-5D VAS scores deteriorated over time in patients \geq 60 years: Baseline (80.00) to Year 2 (65.00), compared to a numerical increase in patients <40 years: Baseline (70.00) to Year 2 (75.00), and 40–59 years: Baseline (70.00) to Year 2 (80.00) (**Figure 5D**)
- No numerical differences were observed in EQ-5D (Figure 5E) and PROMIS-PF (Figure 5F) scores

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Disclosures

For a complete list of author disclosures, scan the QR code on this poster.

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