#11544: Pediatric patients with tenosynovial giant cell tumor: Real-world results from an observational registry

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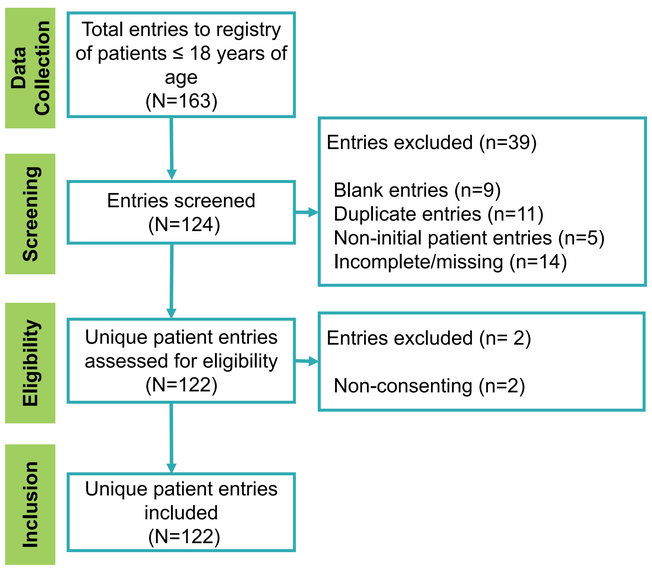
Background

Tenosynovial giant cell tumor (TGCT) is a rare, locally aggressive neoplasm that arises from the synovial lining of the joint, bursa, and tendon sheath. Pediatric TGCT is considered ultra-rare and is believed to mimic the adult form. However, pediatric patients are typically excluded from clinical trials, resulting in a lack of prospective data. Real-world evidence is essential to better understand the impact of TGCT on pediatric patients' quality of life and to characterize the disease in children compared to adults.

Methods

The TGCT Support Registry is an international, patient-reported registry initiated in 2022 by TGCT Support, a program of The Life Raft Group. Pediatric patients enrolled in the registry were selected to describe their clinical characteristics and their journey.



Results

Table 1. Characteristics of patients stratified by subtype (localized, diffuse, or unknown)

	Diffuse (n=89, 73.0%)	Localized (n=20, 16.4%)	Unknown (n=13, 10.6%)	Total (N=122)
Female sex, n (%)	59 (66.3)	14 (70.0)	9 (69.2)	82 (67.2)
Median age at Diagnosis, years (range)	14 (3 - 17)	15 (4 - 17)	14.5 (7 - 15)	14.5 (3 - 17)
Median age at enrollment, years (range)	16 (4-18)	17 (6-18)	15 (10-18)	16 (4-18)
Located in the US, n (%)	47 (52.8)	11 (55.0)	4 (30.8)	62 (50.8)
Location of disease, n (%)				
Knee	76 (85.4)	16 (80.0)	10 (76.9)	102 (83.6)
Hip	8 (9.0)	4 (20.0)	1 (7.7)	13 (10.7)
Ankle	5 (5.6)	0 (0.0)	0 (0.0)	5 (4.1)
Other[a]	0 (0.0)	0 (0.0)	2 (15.4)	2 (1.6)
Misdiagnosis, n (%)	56 (62.9)	10 (50.0)	10 (76.9)	76 (62.3)

Figure 1. Patient journey from diagnosis to treatment

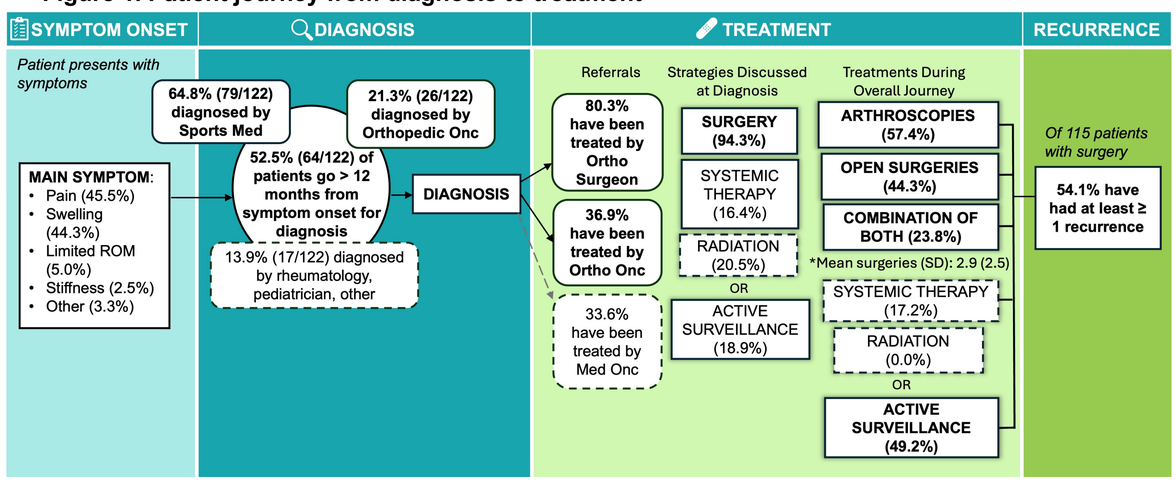
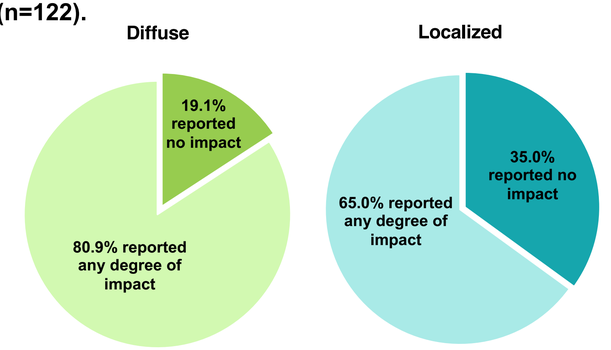


Figure 2. Proportion of pediatric patients with pain that interferes with day-to-day activities, social activities, enjoyment of life, and enjoyment in fun activities (n=122).



Future Directions

Pediatric patients with TGCT experience a significant disease burden and often follow a clinical course similar to that of adults, including high rates of misdiagnosis and local recurrence. However, therapeutic advancements have focused exclusively on adult populations. There is a critical need to prioritize multidisciplinary care and develop systemic therapies tailored to the pediatric TGCT population to reduce disease burden and enhance quality of life. In the context of ultra-rare diseases where clinical trials are often not feasible, patient registries play a vital role in generating meaningful data.

Acknowledgements

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