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Patient-reported Continued Benefits in Patients Treated With Pexidartinib for Tenosynovial Giant Cell Tumor Based on a Real-world Study in the United States

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Objective

- To assess changes in symptoms over time among patients who were treated with pexidartinib for tenosynovial giant cell tumor (TGCT) in a real-world setting

Conclusions

- Most respondents reported improvements in physical function, stiffness, and pain after pexidartinib treatment at the time of baseline survey, and the symptom improvements and treatment satisfaction were sustained after an additional year of follow-up among those remaining on pexidartinib treatment
- These findings indicated sustained long-term benefits for patients continuing treatment with pexidartinib

Plain Language Summary

Why did we perform this research?

Pexidartinib is the only systemic therapy approved by the US Food and Drug Administration (FDA) for the treatment of patients with TGCT based on the ENLIVEN trial. We conducted this research to understand how TGCT symptoms change over time for patients receiving pexidartinib in the real-world setting

How did we perform this research?

We conducted 2 surveys of adult patients enrolled in the Turalio® Risk Evaluation and Mitigation Strategy (REMS) program. Eligible patients were asked to provide information on demographics, medical history, symptoms, and treatment experiences

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

Most survey participants treated with pexidartinib experienced improvements in symptoms; on average, those benefits were sustained with continued pexidartinib treatment

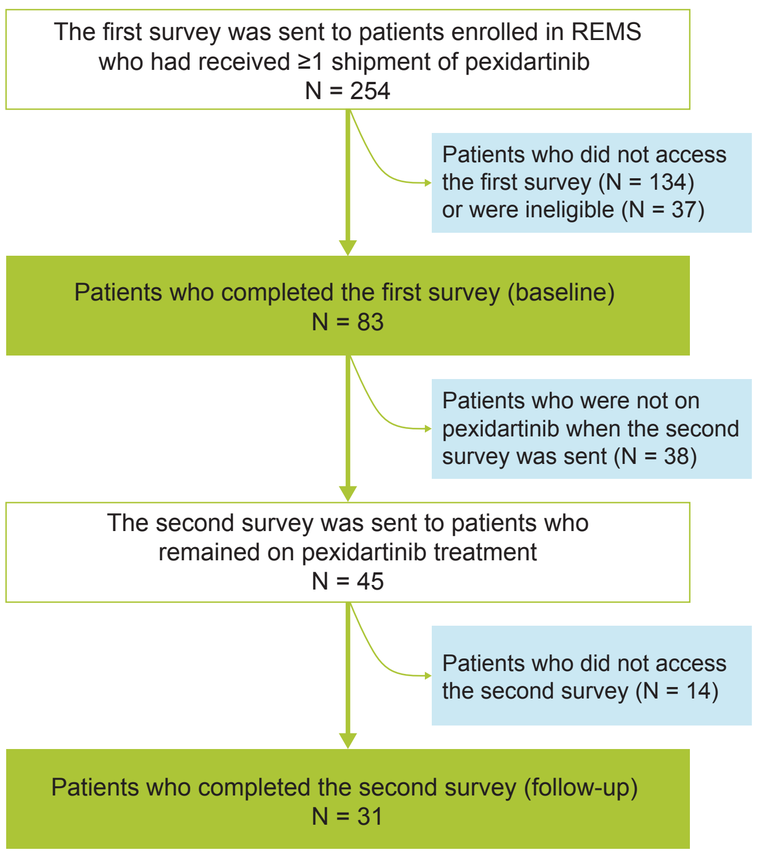
Introduction

- TGCTs are rare, nonmalignant but locally aggressive neoplasms characterized by inflammation of the synovial lining of the joints and tendons, which may cause significant symptom burden in affected patients[1]
- Pexidartinib (Turalio®) is the only systemic therapy approved by the FDA for the treatment of adult patients with symptomatic TGCT associated with severe morbidity or functional limitations and not amenable to improvement with surgery[2]
  - The approval was based on the double-blind, randomized, placebo-controlled, phase 3 ENLIVEN trial, which demonstrated a 39% overall response rate (ORR) and meaningful improvements in physical function and stiffness with pexidartinib at Week 25 in patients with advanced TGCT[3]
  - With prolonged follow-up of a median of 31.2 months in ENLIVEN patients, pexidartinib maintained clinical benefit, with an increase in ORR to 61% and no new safety signals observed after long-term treatment[4]
- Because of the risk of hepatotoxicity, pexidartinib is available to US patients who are registered in the Turalio® REMS program[2]

Methods

- Two web-based surveys were administered to adult patients in the Turalio® REMS program: the first wave was sent in 2021 (baseline) and the second wave was sent in 2022 (follow-up)
- **Inclusion/exclusion criteria:** Eligible patients were required to be taking pexidartinib before the baseline survey and to be on pexidartinib when accessing the follow-up survey. Patients were able to complete questionnaires in English and were not to be participating in any pexidartinib clinical trials
- Information on demographics, medical history, and patient-reported outcomes was collected

Figure 1. Patient Attrition



REMS, Risk Evaluation and Mitigation Strategy.

Results

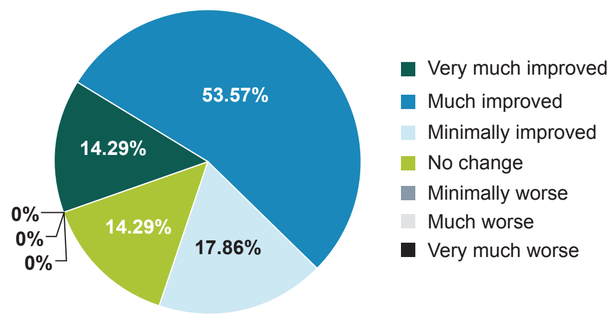
- Eighty-three eligible patients completed the first survey (baseline), and 31 patients completed the second survey (follow-up; **Figure 1**). Mean (standard deviation [SD]) time between baseline and follow-up was 1.02 (0.17) years, mean (SD) age at follow-up was 41.9 (13.70) years, and 67.7% of patients were female
- **Physical function:** At baseline, the majority (59.0%) of patients reported "much improved" or "very much improved" with pexidartinib treatment compared to life before pexidartinib treatment (**Table 1**)
  - From baseline to follow-up, the mean (SD) change in PROMIS-PF score was –0.43 (5.724), which was not significant using a statistical model adjusted for time since the first pexidartinib dose (**Table 2**), thus showing stability of physical function improvement over time while on therapy
- **Pain and stiffness:** At the baseline survey, improvement was reported during pexidartinib treatment, with a mean (SD) change of –3.2 (3.1) in worst stiffness numeric rating scale (NRS) and –3.0 (2.7) in worst pain NRS when compared to pain and stiffness prior to pexidartinib treatment (**Table 1**)
  - Results were sustained between baseline and follow-up, with a mean (SD) change in worst stiffness NRS of 0.8 (2.67) and a mean (SD) change in worst pain NRS of –0.1 (2.09), and neither of the changes were significant (**Table 2**)
- **Treatment Satisfaction Questionnaire for Medication (TSQM):** None of the TSQM scores changed significantly over time except for the TSQM Effectiveness Domain score, of which the mean (SD) was 68.64 (19.167) at baseline and 73.75 (17.749) at follow-up, with a significant increase of mean (SD) 7.28 (19.021; *P* = 0.04; **Table 2**)
- **Symptom improvement (Patient Global Impression of Change [PGIC]):** The majority (85.7%) of patients reported improved overall symptoms since the start of the study (**Figure 2**)

Table 1. Summary of PGIC in Physical Function Since Initiating Treatment With Pexidartinib, Worst Stiffness NRS, and Worst Pain NRS Prior to and During Pexidartinib Treatment[a] at the Baseline Survey

Physical function (N = 83)	**N**	**%**
Very much improved	25	30.1
Much improved	24	28.9
Minimally improved	16	19.3
No change	14	16.9
Minimally worse	1	1.2
Much worse	1	1.2
Very much worse	2	2.4
Worst stiffness NRS (N = 83)	**Mean**	**SD**
Score prior to pexidartinib treatment	6.2	2.8
Score during pexidartinib treatment	3.0	2.4
Change from prior to pexidartinib treatment	–3.2	3.1
Worst pain NRS (N = 83)	**Mean**	**SD**
Score prior to pexidartinib treatment	5.7	2.7
Score during pexidartinib treatment	2.7	2.4
Change from prior to pexidartinib treatment	–3.0	2.7

PGIC, Patient Global Impression of Change; NRS, numeric rating scale.
[a]Patients were asked to recall their worst stiffness and worst pain prior to and during pexidartinib treatment.

Figure 2. PGIC in Overall Symptoms Since the Start of the Study in the Follow-up Survey



PGIC, Patient Global Impression of Change.

Table 2. Patient-reported Outcomes, Including PROMIS-PF, Worst Stiffness NRS, Worst Pain NRS, and TSQM Scores at the Baseline and Follow-up Surveys

		Baseline	**Follow-up**	**Change**	***P* value[a]**
PROMIS-PF	**N**	31	27	27	
	Mean (SD)	44.29 (8.859)	43.06 (7.644)	–0.43 (5.724)	NS
Worst stiffness NRS	**N**	31	29	29	
	Mean (SD)	2.60 (2.610)	3.60 (2.460)	0.8 (2.67)	NS
Worst pain NRS	**N**	31	29	29	
	Mean (SD)	3.00 (2.890)	3.10 (2.750)	–0.1 (2.09)	NS
TSQM Effectiveness Domain score	**N**	31	29	29	
	Mean (SD)	68.64 (19.167)	73.75 (17.749)	7.28 (19.021)	0.04
TSQM Side Effects Domain score	**N**	31	29	29	
	Mean (SD)	60.89 (23.879)	59.27 (22.449)	0.86 (16.597)	NS
TSQM Convenience Domain score	**N**	31	29	29	
	Mean (SD)	65.05 (21.430)	63.98 (19.281)	1.34 (17.293)	NS
TSQM Global Satisfaction Domain score	**N**	31	29	29	
	Mean (SD)	69.12 (18.200)	71.18 (19.231)	4.19 (16.257)	NS

PROMIS-PF, Patient-Reported Outcomes Measurement Information System–Physical Function; NRS, numeric rating scale; TSQM, Treatment Satisfaction Questionnaire for Medication; SD, standard deviation; NS, not significant.
[a]*P* value was from a random slope regression model with time since first pexidartinib dose as the independent variable.

Limitations

- Surveys collected data from patients only; therefore, objective assessment of clinical outcomes was not possible
- The analysis was subject to recall bias and nonresponse bias
- The sample size of the second survey was relatively small, with only 31 participants
- Dosing information was not available to evaluate the correlation to outcomes

Disclosures

DD, FL, XY, and KT are employees of Daiichi Sankyo, Inc. and may hold stock/stock options. IP and KF are employees of United BioSource LLC, a research consulting firm that received funding from Daiichi Sankyo, Inc. to conduct this study. WT has received consulting fees from Daiichi Sankyo, Inc.

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