RWD170

Real-World Experience of Patients Newly Initiated on Pexidartinib for Tenosynovial Giant Cell Tumor (TGCT)

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Introduction

- TGCTs are rare, non-malignant, yet locally aggressive neoplasms, characterized by inflammation of the synovial lining of joints and tendons which may cause significant symptom burden in affected patients.[1]
- Pexidartinib (Turalio®) is the first and only systemic therapy approved by the US FDA for the treatment of adult patients with symptomatic TGCT associated with severe morbidity or functional limitations that are 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 of ENLIVEN patients, pexidartinib maintained its clinical benefit, with an increase in ORR to 61%, and no new safety signals were observed after long-term treatment.[4]
- Because of the risk of hepatotoxicity, pexidartinib is available to US patients who are registered in the Turalio Risk Evaluation and Mitigation Strategy (REMS) program.[2]

Rationale & Objective

- There are studies confirming the benefits of pexidartinib in improving symptoms and functional outcomes among patients with symptomatic TGCTs from the prevalent pexidartinib patients' perspective[5,6], yet the benefits on patients who newly initiated on pexidartinib need to be studied in the real-world settings.
- The objective of this study was to assess symptom change over time among patients who were newly initiated on pexidartinib for TGCT in a real-world setting.

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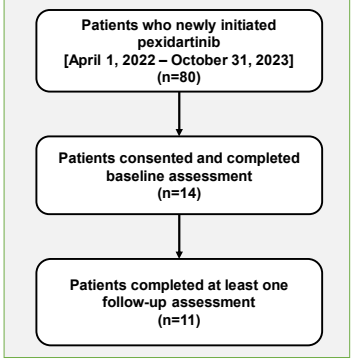
Methods

- Longitudinal, observational study on TGCT patients registered in Turalio REMS program in the US.
- Inclusion/Exclusion: At least 18 years of age, newly-initiating pexidartinib (never had pexidartinib before) in real-world clinical practice. No exclusion criteria for participation.
- Participants completed patient-reported outcome (PRO) assessments electronically at baseline when initiating pexidartinib and at least one follow up assessment within 12 months.
- Patient-reported outcome assessments:
  - Patient-Reported Outcomes Measurement Information System physical function (**PROMIS-PF**); represented in T-score.
  - 0-10 Numeric Rating Scale (**NRS**) for **Worst Stiffness** and **Worst Pain;** higher score denotes worse stiffness/pain.
  - Patients' global impression of change (**PGIC**) in overall symptom since initiation of pexidartinib.
  - Treatment Satisfaction Questionnaire for Medication (**TSQM**); total score = 100, higher score denotes higher satisfaction.
- Random slope regression models were developed using "time since first pexidartinib dose" as an independent variable to describe rate of change in patient-reported outcome measures from baseline to last follow-up.

Figure 1. Patient enrollment and attrition



Results

- Eleven (n=11) patients completed the baseline survey and at least one follow-up survey within 12 months of follow-up (**Figure 1**).
- Mean (SD) age at baseline was 42.2 (12.2) years, 54.5% were females, median (range) duration of follow-up was 8.2 (0.5-11.5) months, and 45.4% of tumors located at knees (**Table 1**).
- Overall, **72.7% (8/11) endorsed moderately high satisfaction with pexidartinib treatment** (i.e., scored at least 70 out of 100 points on TSQM). Among eight participants providing responses for PGIC, **87.5% (7/8) reported "much improved" or "very much improved" in overall symptoms** since initiation of pexidartinib (**Table 2**)
- **Majority of patients reported reduction in stiffness and pain since initiation of pexidartinib** (**Figure 2**). Two patients (Participant No. 2 & No. 11) who reported worsening of stiffness (+2 and +4 on NRS) also reported minimal worsening in physical function; however, both reported reduction in pain (−3 and −2 on NRS) and felt overall change in symptoms to be "Much improved" on PGIC (**Table 2**, **Figure 2**).
- The average rate of change (ARoC) derived from random slope regression models of the outcome measures revealed an **overall trend of reduction in Worst Pain NRS** (ARoC = −2.44) and **Worst Stiffness NRS** (ARoC = −1.82) and **improvement in PROMIS-PF** (ARoC = 6.33) during treatment with pexidartinib.

Table 1. Summary of characteristics of participants

Participant No.	Sex	Tumor Location	Follow-up (month)
1	Female	LE*	11.5
2	Female	Hip	11.0
3	Female	Knee	9.3
4	Male	Knee	9.4
5	Male	Knee	9.5
6	Male	Foot	8.2
7	Female	Knee	2.5
8	Male	UE**	0.5
9	Female	Knee	6.6
10	Female	Wrist	5.8
11	Male	Ankle	6.3

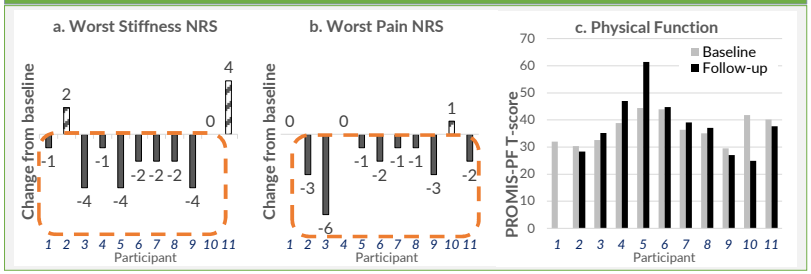
*Tumor in lower extremity; participant did not specify.
**Tumor in upper extremity; participant did not specify.

Table 2. Patient-reported outcomes on treatment satisfaction and change in disease state

Participant No.	PGIC	TSQM Global satisfaction	TSQM Effectiveness	TSQM Side Effects	TSQM Convenience
1	NR	28.57	50	37.5	77.78
2	Much improved	71.43	61.11	25	5.56
3	Much improved	71.43	61.11	100	55.56
4	Much improved	78.57	83.33	56.25	66.67
5	Very much improved	71.43	83.33	87.50	77.78
6	Very much improved	92.86	94.44	56.25	44.44
7	NR	85.71	66.67	37.50	50
8	NR	78.57	72.22	25	83.33
9	Much improved	71.43	55.56	56.25	33.33
10	Much worse	14.29	55.56	18.75	100
11	Much improved	50	83.33	100	72.22

PGIC=Patient Global Impression of Change; TSQM= Treatment Satisfaction Questionnaire for Medication; NR = Not Reported.

Figure 2. Symptoms Improvement Since Initiation of Pexidartinib



NRS=Numeric Rating Scale; PROMIS-PF=Patient-Reported Outcomes Measurement Information System–Physical Function.

Conclusions

TGCT is known to negatively impact the quality of life of working-age adults; however, most patients in this study reported improvements of symptoms that could be noticeable as early as a half-month following first dose of pexidartinib. Achieving clinically-meaningful reduction in pain is considered a major driver of patients' overall impression of change in disease status.

LIMITATIONS

- Survey study collected PRO assessments, which are subjective and represent patients' perspectives only.
- Response rate was 13.8% with an overall limited sample size of 11 patients. Representativeness of the total pexidartinib-treated population cannot be assessed.
- Dosing information was not available to analyze its association with outcomes (e.g., total daily dose, withholding dose, dosing interruptions).

DISCLOSURES

- This study was funded by Daiichi Sankyo, Inc. Dong Dai, Xin Ye and Kristen Tecson are employees of Daiichi Sankyo and may hold stock/options. Irene Pan is an employee of United BioSource LLC, a research consulting firm that received funding from Daiichi Sankyo to conduct this study. William Tap has received consulting fees from Daiichi Sankyo.

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