

# Facial Angiofibroma Related to Tuberous Sclerosis Complex and the use of Topical Rapamycin in the United States: A Survey of Caregivers and Individuals with TSC

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## Introduction

- Facial angiofibroma (FA), a predominant cutaneous manifestation of tuberous sclerosis complex (TSC), typically starts appearing between 2–5 years of age and worsens with advancing age.<sup>1,2</sup>
- A major limitation in the US is lack of an FDA-approved treatment for FA, although the effectiveness of topical rapamycin was evident from several studies.<sup>3–8</sup>
- This analysis was aimed to study the impact of FA on those living with TSC and the management of FA, focusing on use of topical rapamycin (as a compounded formulation) in the US using the data from an international survey.

## Methods

- The TS Alliance conducted an online International Drug Development Survey between 15 May 2017 and 14 June 2017 by distributing a link to individuals living with TSC and caregivers through various channels including social media.
- Caregivers responded on behalf of their child or dependent adult living with TSC. Of the 420 caregivers and 133 individuals with TSC aged ≥13 years who responded from the US, 336 (80.0%) caregivers and 98 (73.7%) patients who reported cutaneous manifestations (FA, ungual fibromas, etc.) were included in this analysis

## Results

- Of the 336 caregivers, 11 (3.3%) and 10 (3.0%) caregivers were taking care of 2 and ≥3 patients, respectively.

Figure 1. Most frequently reported age of TSC individuals at the time of survey was 18–26 years

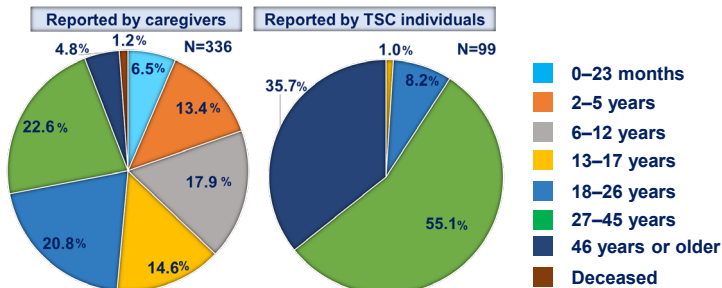


Figure 2. Most frequently reported age of TSC individuals at the time of diagnosis of TSC was 0-23 months

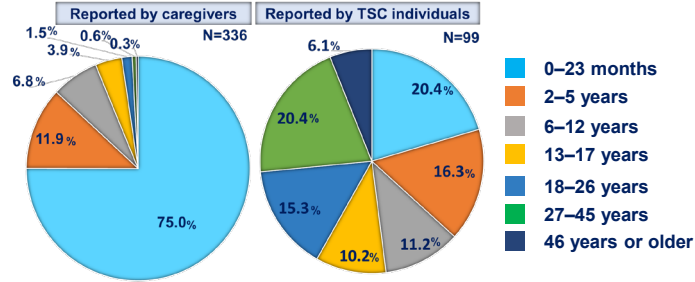


Figure 3. More than half of the responders reported a minor, moderate, or severe impact on lifestyle

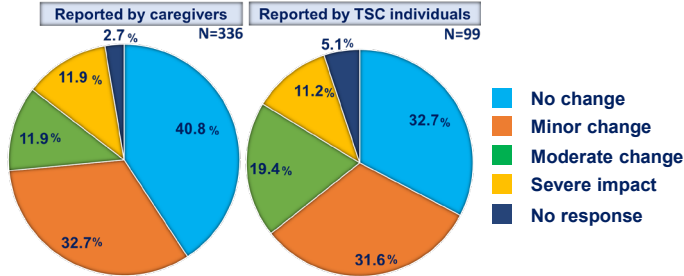


Figure 4. Topical rapamycin was reported to be moderately or very effective by 82.9% of caregivers and 73.9% of TSC individuals

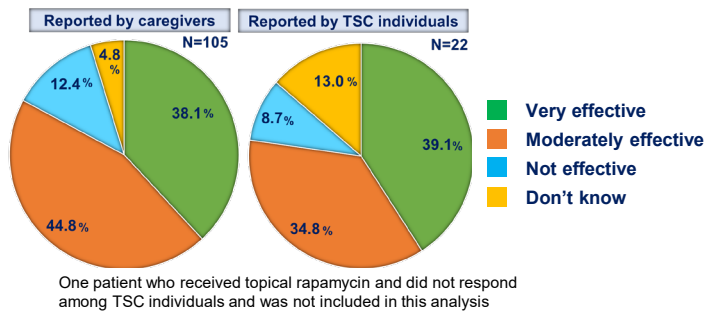


Figure 5. Use of topical rapamycin for the management of FA was reported by 105 (31.3%) caregivers and 23 (23.5%) patients

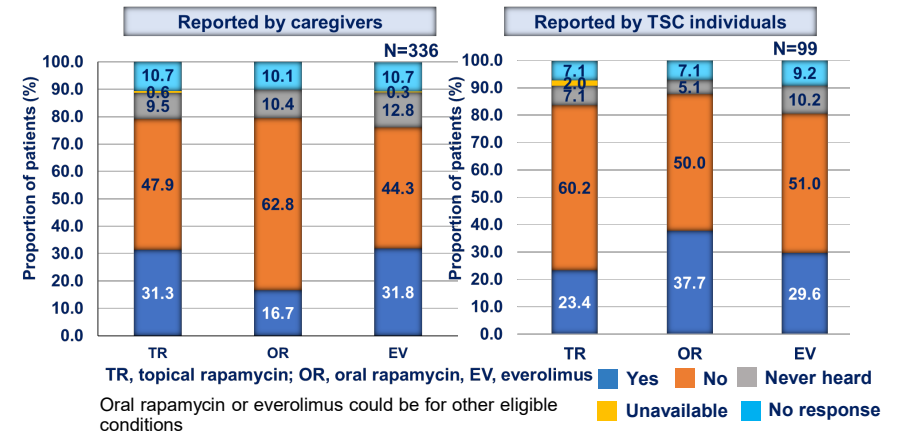
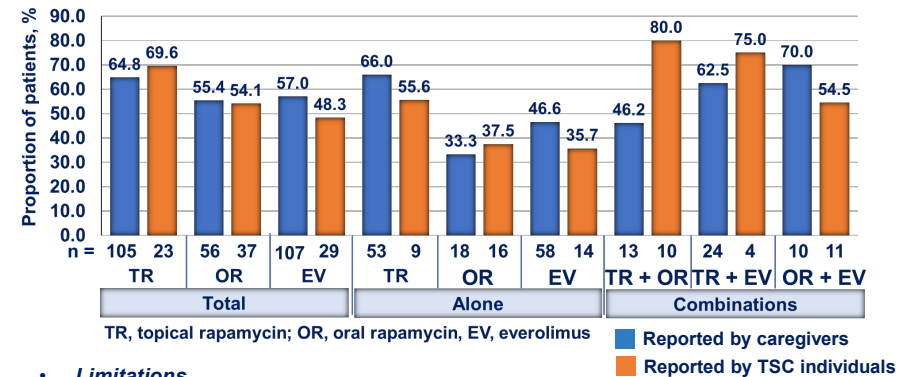


Figure 6. Higher proportion of TSC individuals using topical rapamycin either alone or in combinations reported improvement in the cutaneous condition



### Limitations

- Major limitation:** This is survey-based analysis which not prospectively designed to characterize the FA related to TSC.
- The analysis was limited to only patients who responded in the TS alliance survey.

TSC, tuberous sclerosis complex

- Use of topical rapamycin for management of FA was reported by 105 (31.3%) caregivers and 23 (23.5%) patients, despite the lack of an approved topical rapamycin formulation.
- Improvement in FA was more common with topical rapamycin than other treatment approaches, and most (>70%) caregivers and patients rated topical rapamycin as moderately effective or very effective.
- Access to an FDA-approved topical rapamycin formulation could benefit even more people living with FA and TSC.