

TSC Self-Report Portal

- This portal permits the collection of patient-reported outcomes (PROs) on a variety of topics related to TSC
- In the case of individuals with TSC that are unable to speak or report for themselves, PROs can be submitted by their parent, guardian, or caregiver
- The type of PROs will vary by study and studies will be continually added into the portal
- **Purpose:** to help the TSC Alliance and TSC researchers better understand the perspective of those affected by TSC so that we can begin to track changes and measure improvement in areas most important to you whether that be in the context of general care, treatment, or something else.

Patient-Reported Outcomes (PROs)

- A PRO is:
 - directly reported by the patient without interpretation of the patient's response by a clinician or anyone else
 - pertains to the patient's health, quality of life, or functional status associated with health care or treatment
 - not monitored in real-time; not to be used for reporting emergencies
- These outcomes can be measured in absolute terms (e.g., rating severity of pain on a numerical scale) or used to report changes from a previous measure (e.g., new symptom onset after starting a new drug)



How much of a problem has it been *over the last month?*

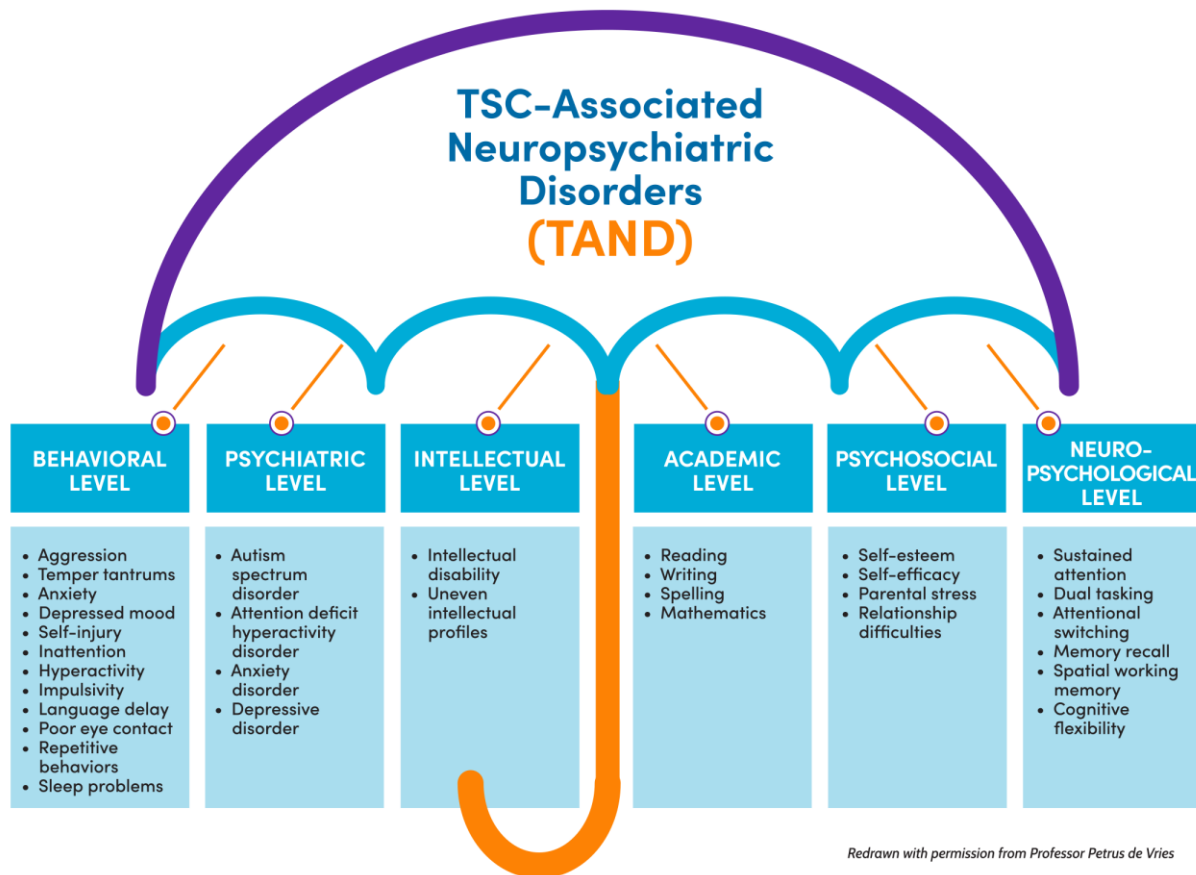
0 - Not at all	1	2	3	4	5	6	7	8	9	10 - Extremely
----------------	---	---	---	---	---	---	---	---	---	----------------



Patient-Reported Outcomes (PROs)

- PROs may be helpful for regulatory bodies such as the FDA to assess risk-benefit measures when evaluating a drug or medical device or clinical trial
- Some considerations:
 - Are the endpoints and measures relevant to people who have the disease?
 - How clinically meaningful is the benefit to patients?
 - How do the side effects impact the tolerability of the treatment?
 - Are emotional or social well being impacted?
 - Should we be measuring something else as an outcome measure?

TAND Patient-Reported Outcomes



- For many TAND symptoms, there are no objective outcome measurements and observation is subjective. PROs could be utilized as an outcome measure in a clinical trial if the correct instrument is validated.
- A PRO instrument (i.e., a questionnaire plus the information and documentation that support its use) is a means to capture PRO data used to measure treatment benefit or risk in medical product clinical trials.

TSC Self-Report Portal to Feature TAND-SQ Checklist

TAND-SQ

English version

Self-report, Quantified TAND Checklist (2021)

Tuberous Sclerosis Complex (TSC) is associated with a range of neuropsychiatric disorders that we refer to as TAND (**T**SC-**A**ssociated **N**europsychiatric **D**isorders). All people with TSC are at risk of having some of these difficulties. Some people with TSC have very few, while others will have many of these difficulties. Each person with TSC will therefore have their own TAND profile, and this profile may change over time.

- Portal is built in StudyTrax – participants in Natural History Database consent to participate and connect self-reported data with existing ID number
- Similar to the NHD, no identifiers will be used allowing de-identified data to be exported for research projects
- Time to complete checklist is estimated to be between 20-30 minutes

Questions about the Self-Report Portal? Email

grushing@tsalliance.org

