Compound Nomination Process

TSC Preclinical Consortium

The TSC Preclinical Consortium engages academic and pharmaceutical industry to accelerate development of new therapies for tuberous sclerosis complex (TSC).

The Preclinical Consortium Steering Committee (SC) sets the long-term goals and Working Groups (WGs) decide the best models and experimental design for drug testing and provide oversight for rigorous quality control and interpretation of results. Compounds are handled and tested by experienced contract research organizations (CROs), and data are shared among the Consortium. Any researcher may propose compounds for testing and benefit from the shared data.

The process described here is for nomination of compounds for which experiments will be run at no cost to researchers, provided that all data will be shared publicly among members of the Consortium. The nominating principal investigator (PI) will be the scientific leader of the project, responsible for taking the lead on publication of results and designing subsequent experiments. Acknowledgment of the Consortium in any publications utilizing the results is required, but co-authorship of other Consortium members is not, unless the PI chooses to collaborate with one or more members. The PI will have a year to follow up on results and work toward publication before data will be released publicly, and the PI may request a six-month extension if necessary.

Additionally, Company Members may submit compounds for testing and retain confidentiality and ownership of the data by paying for experiments as defined in the Consortium Operating Agreement. Companies interested in nominating compounds and retaining confidentiality and ownership of the data should not utilize the nomination form and should contact Dean Aguiar, PhD, Vice President, Translational Research, directly (daguiar@tscalliance.org).

Compound nomination form

The PI must complete the short nomination form, available on the TSC Alliance Preclinical Consortium webpage. The form requests no more than two pages of text and/or figures.
to describe the rationale for the compound and its mechanism of action for TSC. The lead PI must also submit an NIH-style biosketch.

**Nominations will be accepted until Monday, August 1, 2022 and will be evaluated in mid-August 2022.**

**Selection process**

Nominations will be reviewed by the Consortium’s WGs as appropriate, who will prioritize the compounds and forward recommendations to the Consortium SC. The Steering Committee will prioritize compounds across the different models and mechanisms of action, and compounds will be tested as soon as possible based on the availability of animals, CRO time, and funds.

Criteria used to evaluate and prioritize the nominations will include, but are not limited to:

- **Significance**: rationale for relevance to TSC
- **Impact**: whether the compound is a “tool compound” or a clinical candidate; there should be a path forward toward patient benefit if the compound has activity.
- **Innovation**: novelty of the mechanism or superiority of the compound over others with similar mechanism of action.
- **Pharmacokinetics and pharmacodynamics**: the degree of target (receptor, enzyme, etc.) engagement should be known or predictable based on existing data to ensure an appropriate dose and route of administration can be chosen.

The PI may be contacted for additional information during the review process.

**Questions?**

Please contact Vice President, Translational Research, Dean Aguiar, PhD (daguiar@tscalliance.org) or Zoë Fuchs, Science Project Manager (zfuchs@tscalliance.org) with any questions.

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1 Suggested acknowledgment format: This work was partially supported by the TSC Alliance® TSC Preclinical Consortium.