



BEYOND BATTEN DISEASE
FOUNDATION

Reviewer Critique Form

REVIEWER PROJECT SUMMARY

Please write a summary of the proposal summarizing the strengths, weaknesses and investment potential that informed your overall score.

SIGNIFICANCE

Does the proposed work address a significant question or a critical barrier to progress in NCL research? If the aims of the project are achieved, how will our collective knowledge, technical capability, and/or translational capacity be improved?

INNOVATION

Does the application challenge and seek to shift current research paradigms by utilizing novel theoretical concepts, approaches or methodologies? If yes, novel to NCL research or novel in a broad sense to be applied to NCL investigation?

APPROACH

Are the overall strategy, methodology, and analyses well-reasoned and appropriate to accomplish the specific aims of the project?

APPLICANT'S QUALIFICATIONS AND POTENTIAL FOR A RESEARCH CAREER IN NCL (BATTEN DISEASE)

Please consider degrees, awards, positions, current and past research, other grants and how these experiences support the current application

FACILITIES, RESOURCES AND EQUIPMENT RELEVANT TO THE FELLOWSHIP AND THE APPLICANT'S PROPOSED LINE OF INQUIRY

Are the facilities, resources and equipment necessary and sufficient to execute the proposal? Do these resources represent the state of the art or are there more advanced or resources with higher resolution that should be accessed? Remember, the question being posed is whether this proposal, as written, is a true investment for the foundation; not whether the proposal does/does not have merit.

EXPECTED OUTCOMES, POSSIBLE PITFALLS AND ALTERNATIVE STRATEGIES

Has the investigator(s) planned appropriately including justification for his/her plan of action? Are potential problems, alternative strategies and benchmarks for success presented?

BIBLIOGRAPHY AND REFERENCES CITED

Does the applicant demonstrate a command of the pertinent literature in NCL (Batten disease), methods proposed, and line of inquiry s/he is pursuing?

BUDGET

Is the budget in line with the work proposed including the time and effort of salary support requested? Should any of the funds requested be paid under indirect costs versus a line item within this budget? The BBDF does not pay administrative, travel or publication fees.

ASPECTS ESPECIALLY WORTHY TO BE FUNDED

This section provides applicants and reviewers the chance to advocate for the work proposed. All too often review scores aggregate between fundable and not fundable suggesting the work proposed is neither good nor bad but just "ok." The association has lofty goals that cannot be reached by funding work that is "ok." Are you advocating that this work be done to an association dedicated to finding treatments and cures for Batten disease?

DRUGGABILITY/POTENTIAL FOR TRANSLATION

The importance of a protein/pathway in the pathology of Batten disease is not sufficient to make it a successful drug target. Did the applicant assess the druggability of his cellular target(s)?

If the applicant is using an FDA-approved or other compound as a functional probe or for pre-clinical assessment as a therapy, did s/he include its approval status, describe the clinical pharmacology, safety profile and efficacy profile of the compound? The purpose of this question is to determine whether the applicant understands what he is working with. The increasing availability and subsequent investigation of approved and emerging compounds holds the potential to accelerate treatment-oriented research. However, this can only happen if material rests in the hands of individuals who understand the full measure of these resources.

Of course, not all research leads to the development of drugs. Gene therapy, stem cell biology and other advancements warrant exploration. If the applicant is investigating these or similar avenues, does s/he demonstrate an understanding of the therapeutic potential, approval status and current clinical uses associated with this approach.

OVERALL SCORE

Provide an overall score to reflect the overall scientific merit of the proposal together with the proposal's investment potential. Has the applicant chosen a fundamental question that is compelling and can be solved? Should this proposal be considered an investment strategy for an association dedicated, not to exploring compelling scientific questions in NCL (Batten disease) but investing in compelling, meritorious research leading to the development of effective treatment strategies? The scoring system uses a 9 point scale. A score of 1 indicates an exceptionally strong application with no scientific weaknesses and high translation potential. A score of 9 has serious substantive scientific weaknesses with little to no translation potential.

Score:

SUGGESTED REVISIONS/CONDITIONAL APPROVAL

This s a "new" section within the BBDF research application providing reviewers the opportunity to make suggestions on how to improve the application. The purpose is not to re-write the proposal but to improve or advance an already-meritorious proposal.

TRIAGE INSTRUCTIONS

In the event that you think the proposal contains too many fatal flaws to warrant discussion or the topic area is outside of BBDF's mission, you may triage it. We ask that you explain your reasoning below understanding that the applicant may receive a copy of these statements if it appears s/he needs to hear them for research decision-making purposes.

Please contact BBDF as soon as you know you would like to triage this proposal. If all of the reviewers agree, the teleconference review of this proposal will be canceled.