Challenge 6: BADIPS: Generating human induced pluripotent stem cells (iPS cells) to study bipolar affective disorder (BAD)

Surgery Questions and Answers

Q. How do we overcome the different perceptions of basic researchers and industry of what constitutes validation?
A. The industry sponsors are happy to discuss this with potential applicants prior to the submission of applications to ensure all parties are thinking along the same lines. There will also be opportunity for the winners of contracts to make minor revisions to their applications based on the negotiations with the industry sponsors after the Challenge Panels and before the winners are announced.

Q. Generating 5 to 10 iPS cell lines from clearly defined BAD patients is very underpowered. Will this be a problem?
A. The industry sponsors believe 5 to 10 iPS cell lines are a good starting point as a pilot project and envision that the pilot would allow us to work out optimum conditions for reliable and reproducible iPS cell generation as well as differentiation. A bold statement of 100 cell lines could be unrealistic and potentially overwhelm prospective applicants.

Q. Should iPS cells be generated from any particular tissue type?
A. No, applicants are required to make the case in their applications for why iPS cells should be generated from a particular tissue(s) or cell type(s).

Q. Should potential solutions only develop the cell lines or should they also demonstrate utility through the drug discovery phase?
A. Applications which address both of these spectra are welcome, but the focus is generating cell lines that are well defined to facilitate further work and potentially drug discovery, and continue to add value over time.

Q. Would it be preferable to have a single or multiple storage and distribution facilities for the cells generated during the project?
A. Applications which favour either a single or multiple storage and distribution facilities approach are welcome; but it is essential that practices are consistent across multiple sites.

Q. What will the industry sponsors do with the iPS cell lines at the end of the project?
A. The industry sponsors are not interested in holding on to any cell lines generated during the course of the project, but would rather see these widely accessible to researchers in the field. The industry sponsors will also work with research labs to help develop a research plan that eventually leads to the utility of the cell lines for drug discovery applications.

Q. Is there a preferred approach for generating iPS cells?
A. No, applicants will need to make the case for a particular approach in their applications.

Q. What happens to the IP if development of the iPS cell lines leads to a diagnostic tool?
A. The IP remains with the contractor and subcontractors .

Q. Are the industry sponsors interested in using the generated iPS cell lines for biomarker development/discovery or just for drug discovery?
A. We envision a variety of uses of the iPS cell lines that could facilitate drug discovery and development. These include the full spectrum of studies that enable target identification/validation through generation of patient tailoring hypotheses and associated biomarkers. However, this does not stop applicants from using this funding and research to apply for funding from other sources to explore the full potential of the cell lines developed during the course of this project.

Q. Are the sponsors looking for a particular phenotype?
A. No, the sponsors are interested in a variety of phenotypic approaches, for example, family studies with familial matched controls, rare variants, etc. The sponsors are interested initially

in approaches that would narrowly define a phenotype in order to reduce the “noise” in the signal *in vitro*.

Q. Do applications need to include a plan for making the cell line ‘toolbox’ accessible to the wider research community?
A. No, but applicants are encouraged to look for collaborations and generation of a toolbox for utility by the wider research community.

Q. Do the industry sponsors have specific criteria for cell culture conditions?
A. No, but the culture conditions must be reliable and consistent between cell lines.