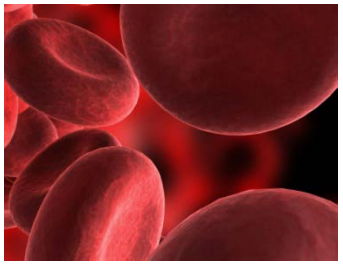
CIRM Disease Team MPP Grants

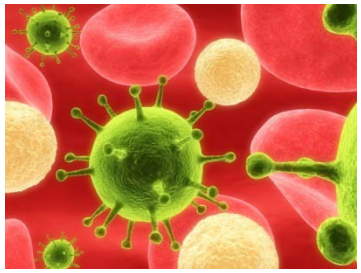


Ann N. James, Ph.D., J.D.

IRB/SCRO Meeting

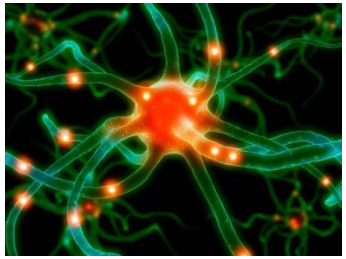
March 9, 2010

What is a Multiple Project Protocol (MPP)/Umbrella?



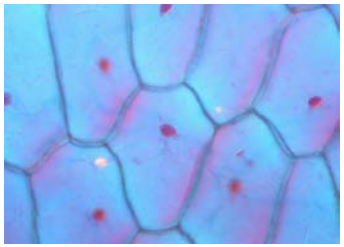
- States the overall goal of the grant
- Defines the various activities and the related collaborators (both as definite and as possible additions)
- Permits Stanford to track participation, compliance documents, progress, and regulatory issues under one Multiple Project Protocol, without change, expansion or modification to the defined protocols as approved by the appropriate oversight bodies.

What Are We Being Asked to Approve Today?



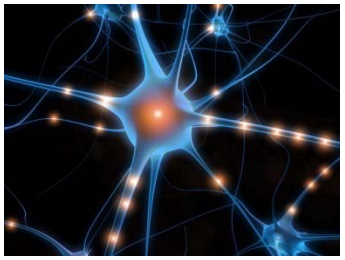
- A conceptual framework
- A list of individual protocols
- NOT individual new protocols or any expansion or revision to approved protocols
- Individual protocols must be submitted, reviewed and approved as they evolve
- NO new IRB/SCRO work will begin prior to individual protocol approval.

CIRM Disease Team Background



- 4-year grants totaling $250 Million
- Grant is expected to result in a new drug (IND) filing with FDA
- All protocols are pre-clinical; (IRB protocols for obtaining tissues)
- Most protocols include animal studies but cannot include clinical trials for human subjects
- Collaborations with academic and for-profit entities for the creation of therapeutics.

Stanford Disease Team Grants:



Epidermolysis Bullosa (Alfred Lane, PI)

iPs Cell-based Treatment of Dominant Dystrophic Epidermolysis Bullosa

Sub-cortical Stroke (Gary Steinberg, PI)

Embryonic-derived Neural Stem Cells for Treatment of Motor Sequelae following Sub-cortical Stroke.

Myeloid Leukemia (Irving Weissman, PI)

Development of Therapeutic Antibodies Targeting Human Acute Myeloid Leukemia Stem Cells

Epidermolysis Bullosa (Alfred Lane, PI)

- Human participants will be asked to donate tissues for this study
- Composite human skin grafts will be used on immunocompromised mice
- No human treatment will happen under this grant
- Focus is on the creation of patient-specific iPS cells to correct genetic defects to eventually make autologous sheet grafts.

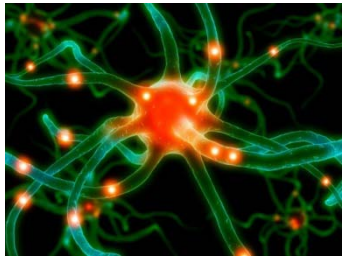
Sub-cortical Stroke (Gary Steinberg, PI)

- Stem Cell transplantation in rats, mice, possibly other animals; no human use
- Utilizes SD56, a derivative of WiCell H9 (hESC formally approved by the NIH)
- Strategies to aid in recovery of animal brain function following cerebral ischemia
- Investigation of migration, survival and integration of transplanted cells in animal brain
- Correlation of behavioral outcomes to the fate and actions of transplanted cells.

Myeloid Leukemia (Irving Weissman, PI)

- Human participants will be asked to donate tissues for this study
- No hESC involved
- Adult stem cells will be purified from cord blood and/or bone marrow
- Comparison of human acute myeloid leukemia stem cells to their normal counterparts to identify genetic and molecular differences
- Xenotransplantation into immunodeficient mice
- Focus is on identifying cell surface molecules preferentially expressed on the leukemia stem cells that can be targeted with monoclonal antibodies.

What Are We Being Asked to Approve Today?



- A conceptual framework for each Disease Team grant
- A list of individual protocols
- NOT individual new protocols
- Individual protocols must be submitted, reviewed and approved as they evolve
- NO new IRB/SCRO work will begin prior to individual protocol approval.