UC San Diego Health
Sanford Stem Cell Clinical Center

Product Development Stages
Sanford Stem Cell Clinical Center

Updated 3-20-2015

Stage	Expected Regulatory Status
Early exploratory research/discovery	• Research material • Exploratory studies • **No communications with the FDA**
Early feasibility *n.b., for the 2014-2015 call-for-applications, Early-Feasibility-Stage applicants must provide a strongly persuasive argument for the unique scientific merit or provide the bulk of funding.*]	• Research grade product • Non-GLP feasibility studies and initial model characterization • Cursory look at regulations and guidance documents • **May have early informal communications with FDA** • Identify potential regulatory path (device/biologic/drug/cell) • Develop understanding of Mechanism of Action (MOA)
Early product development/translation. [*n.b., for the 2014-2015 call-for-applications, Early-Stage applicants must provide a strongly persuasive argument for the unique scientific merit or provide the bulk of funding.*]	• Early Good Documentation Practices • Identify applicable regulatory path and early regulatory requirements • Research grade product transitioning to more clinically compliant reagents • Characterize disease models, including utility for evaluating product under development • Conduct Non-GLP feasibility, Proof-of-concept (POC), and safety studies with increasing rigor and animal numbers to support later study design and statistical analysis. • Develop characterization methods (QC assays) for identity, purity (and possible contaminants), safety, potency • Develop delivery method • Assemble source donor and derivation documentation (or get assurances/MTA from owner) • **Pre-pre-IND meeting(s)** for cell source and derivation acceptability (i.e., compliance with Good Tissue Practices); disease model(s), early pre-clinical and clinical study design
Late product development/translation	• Use Full Good Documentation Practices • Develop regulatory plan • Create early Chemistry, Manufacturing, and Controls (CMC) section and master files (or obtain permission letters from file holders) o Identify source material and derivation o Document reagent/material sources (animal source, GMP grade, reagent grade, USP, etc.) o Provide manufacturing-facility description, maintenance and

	certification (demonstrate environmental controls) o Identify and install critical equipment, plan maintenance, calibration, standardization and use in the process (IQ, OQ, PQ) o Create Manufacturing process flow diagram and description o Define In-process and final product release testing methods and acceptance criteria • Prepare information on pre-clinical testing to date o Include supportive publications o Model characterization o Non-GLP safety and POC studies o Formal reports with all individual data included in attached tables • Prepare definitive IND enabling study plan o Detailed plan and study descriptions o Justify model selection o Describe statistical plan and justification for animal numbers o GLP safety, toxicity and tumorigenicity studies o GLP-like POC/efficacy studies • Prepare clinical plan and draft clinical protocol and consent form • **Pre-IND meeting**
IND Submission	• Perform definitive IND enabling studies using GMP manufactured product • GLP Tox/Safety and GLP-Like POC studies in well characterized and justified models • Follow up Pre-IND meeting(s) as studies progress • **IND submission** and summary of communications with FDA including issues and plan to address issues
Clinical Hold	• **Clinical Hold** o Letter from FDA placing IND on hold with issues to address to release hold and non-hold issues to address at year 1 annual report o Plan and status of hold response o Summary of communications regarding hold
Clinical Trial Initiation	• **Approved IND** o Letter of approval with any restrictions • IRB Approval (send FDA any updates to Clinical Protocol and Consent required by IRB