WEBINAR – 15 April 2013
Clinical Trials: Moving Stem Cell based Therapies to the Clinic
Questions and Answers

Questions	Answers
1. Would the assessment of genetic changes be necessary when adult cells have been cultured only for 3 days and are intended for autologous transplantation?	**John Hyde:** The potential concern about the need to address genetic stability for specific products should be discussed with the FDA.
2. What is the best place to find information on rules and regulations of Cell Based Therapies?	**John Hyde:** There is a wealth of information available at the CBER website through fda.gov. Some links and contact information were provided in the last few slides of my talk. The following links should be of particular interest: The Office of Cellular, Tissue and Gene Therapy webinar course series (OCTGT Learn): http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/ucm232821.htm Guidance specific to cellular and gene therapies: http://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/Guidances/CellularandGeneTherapy/default.htm General guidance on biologic products: http://www.fda.gov/BiologicsBloodVaccines/GuidanceComplianceRegulatoryInformation/default.htm
3. What is the appropriate approach to reproductive tox testing?	**John Hyde:** The determination of potential reproductive/developmental toxicity may need to be addressed, depending on product type or target patient population. In general, such reproductive toxicology studies should be conducted prior to Phase 3 clinical trials. This issue should be discussed with the FDA Pharm/Tox personnel during the conduct of early-phase clinical trials.

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4. Problems and risks with using animal serum in cell production are well known. What is the FDA's position on using animal serum in GMP manufacturing cell therapy products?	**John Hyde:** The potential concern about the use of animal serum for a specific product should be discussed with the FDA.
5. Did you have any problems with enrollment in Phase 1? Was it as scheduled or slow? How is Phase 2 going? How many patients have been enrolled so far?	**Jonathan Glass:** No problem with enrollment in Phase 1. We had more patients than we could handle. Phase 2 is not yet enrolling - awaiting FDA approval.
6. Did you look at any migration from the injection sites? Did you label any of the stem cells?	**Jonathan Glass:** Cells not labeled and we were not able to assess migration.
7. How did you calculate the target dose and how far off were you in the Phase 1 trial?	**Jonathan Glass:** based on migration from injection site in pig studies. Dose include both number of injections and number of cells/injection-
8. How much leeway do the developers have after formal preclinical studies? It is generally understood that certain process changes after Phase 1 will require bridging studies? Is this similar if process changes are introduced after formal preclinical studies?	**John Hyde:** It will depend on the nature of the process change, but if the change is significant enough, it could bring into question the relevance of the preclinical data, so bridging might be needed.
9. Besides FDA approval and IRB, were these trials subject to any other regulatory reviews?	There were other oversight committees at the universities that evaluated the protocols. Each patient goes through a process before they enroll onto the trial of informed consent. In addition, each trial has an independent data safety monitoring committee to help ensure patient safety during the conduct of the trial.