Characterization of novel cellular therapeutics – safety and efficacy evaluations by flow cytometry

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Over the last two years, immunotherapies based on autologous gene modified T cells have reached the level of commercialized products and are in great demand to elicit cures of hematologic malignancies. At the same time, many other advanced cellular, tissue and gene therapies are currently being developed, translated into clinical applications and applied in early to late phase clinical trials. Many of these products are already showing promising results in the treatment of diseases that did not have any therapies available prior to the development of such novel cell based medicines. Among these therapies are stem cell products differentiated from pluripotent stem cells, such as embryonic stem cells or induced pluripotent stem cells; other products are comprised of adult type, multipotent stem cells. The Good Manufacturing Practice (GMP) facility at the University of California Davis has, for several years, been manufacturing a variety of these products for clinical trial applications. In this talk, the challenges of characterizing such cellular products using flow cytometry will be discussed. Two examples will be highlighted; in example 1, it will be discussed how allogeneic neuronal stem cells derived from pluripotent stem cells with excellent functionality in an in vivo model of Huntington's disease should be characterized to reliably demonstrate safety of such cell populations for human applications, and at the same time, should correlate to in vivo efficacy. In example 2, the characterization of autologous multipotent stem cells, CD34+ cells purified from bone marrow aspirates of patients and formulated for administration in the eye to treat blindness, will be discussed.