



## **Identification of a Myeloid Precursor Cell for Modulations of Immune Responses** (Yacov Ron, RWJ 98-08) *Therapeutic*

### **Background**

Pluripotential stem cells (PSC) are found in the bone marrow and spleen in mice and are cells that are capable of self-renewal and differentiation into all lineages of the hematopoietic system. Only one in 2,000-5,000 cells in bone marrow are PSC and are present within a narrow subset (Thy-1.1lo LinSca-1+) of bone marrow cells. The current dogma concerning the kinetics of hematopoiesis is that only primitive pluripotential bone marrow stem cells can support hematopoiesis, whereas lineage-committed stem cells can support only a particular lineage. The present invention provides long-lived myeloid-committed stem cell population that can replenish the mature myeloid lineage.

### **Description of the Technology**

Researchers at UMDNJ have identified long-lived myeloid-committed stem cells in spleen which replenish the mature myeloid lineage for at least 12 months. Evidence for these findings is provided by the discovery that these cells do not home back to the bone marrow. These stem cells can be targeted with a retroviral vector following LPS stimulation of T-cell depleted spleen cells. The ability to introduce exogenous genes into myeloid lineage has several advantages over current methods of retroviral-mediated gene transfer techniques using bone marrow stem cells. For example, the efficiency of gene transfer using bone marrow stem cells is very low (5%), while the efficiency with the present method is extremely high. Another disadvantage of using bone marrow cells includes expression of the exogenous gene in all hematopoietic cell lineages.

**Gene transfer using the cells identified in the present invention results in the targeted expression of the exogenous gene in the desired subset of the myeloid compartment.**

### **Applications**

- Gene therapy-based treatment of genetic disorders linked to myeloid lineage such as Gaucher's disease
- Selective immune response against tumor or viral antigens.

### **Patent Status**

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