Genetically Modified Hematopoietic Stem And Progenitor Cells (HSPCs) And Mesenchymal Cells As A Platform To Reduce Or Prevent Metastasis, Treat Autoimmune And Inflammatory Disorders, And Rebalance The Immune Milieu And Dysregulated Niches

Summary (1024-character limit)
There is a marked increase in immunosuppressive myeloid progenitors and myeloid cells in tumors and at metastatic tissue sites, rendering these types of cells useful in cancer therapeutics – especially after genetic modifications to improve their anti-tumor properties. The National Cancer Institute (NCI) seeks research co-development or licensing for genetically engineered myeloid cells (GEMys) for use in cancer immunotherapy.

NIH Reference Number
E-053-2019

Product Type
• Therapeutics

Keywords
• Genetically Engineered Myeloid Cells, GEMys, Platform Technology, Cancer Immunotherapy, Metastasis, Adoptive Cell Therapy, Autoimmune Disease, Kaplan

Collaboration Opportunity
This invention is available for licensing and co-development.

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Description of Technology
Cancer cells can spread to various regions in the body in a process called metastasis which is associated with non-responsive to treatment and thus reduced survival. Identifying the markers of metastasis has been a major concern in the field of cancer diagnosis and therapy. Interestingly, research has shown that there is an increase in myeloid progenitors and myeloid cells at various stages of metastasis in an attempt by the immune system to suppress cancer cells. This presents a promising technology for cancer immunotherapy.

Researchers at National Cancer Institute (NCI) developed a platform to culture myeloid cells from

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murine bone marrow cells and apheresed human peripheral blood. Myeloid cells were modified to express IL-12 to enhance anti-tumor immunity, limit inflammatory response, recruit T cells to sites of interest and specifically target and kill tumors. The inventors used these genetically modified myeloid cells (GEMys) in a metastatic embryonal rhabdomyosarcoma tumor model. They observed an activation of immune system cells, such as mature T cells, at the site of metastases, as well as an increase in myeloid cell populations with anti-tumor properties. Importantly, treatment of this orthotopic tumor model with GEMys reduced the metastatic burden and significantly improved survival in mice. These cells can be combined with traditional immunotherapies and other cell-based strategies – such as chimeric antigen receptors – to further improve anti-tumor immunity.

The Pediatric Oncology Branch is seeking parties interested in licensing and codeveloping this invention to commercialize the GEMys for improved cancer immunotherapy.

**Potential Commercial Applications**
- Cancer immunotherapy by GEMys alone or by coupling with T cell-based strategies
- Treatment of metastatic or recurrent cancers
- Treatment of autoimmune diseases through limiting the inflammatory response

**Competitive Advantages**
- GEMys improved survival in an orthotopic metastatic cancer model
- GEMys can be further modified for enhanced functionality
- GEMys can be used alone or in combination with therapeutic T cells
- Potential FDA Orphan Drug Designation for pediatric and rare tumor indications

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**Development Stage**
- Pre-clinical (in vivo)

**Publications**

**Patent Status**
- **U.S. Provisional**: U.S. Provisional Patent Application Number 62/803,468, Filed 09 Feb 2019
- **PCT**: PCT Application Number PCT/US2020/17515, Filed 10 Feb 2020

**Therapeutic Area**
- Cancer/Neoplasm

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