



SARGRAMOSTIM IN THE MANAGEMENT OF FUNGAL INFECTIONS

SEPTEMBER 25, 2020

Sargramostim - Ready for Investigational Use Today



- FDA approved in 6 disease indications →
 - > 500,000 recipients
 - **Safe in pediatrics and adults**
- Leukine® 250 mcg/single-dose vials
 - Given by **nebulizer for investigational use**



Leukine®
sargramostim
250 mcg/single-dose vial
Refrigerate, 2-8°C (36-46°F)
Do not freeze or shake
Protect from light

NDC 71837-5843-1
Rx only Dosage: See insert
Mfd. by: Partner Therapeutics, Inc.
Lexington, MA 02421
RL10152
U.S. Lic. No. 2087 AW001-00

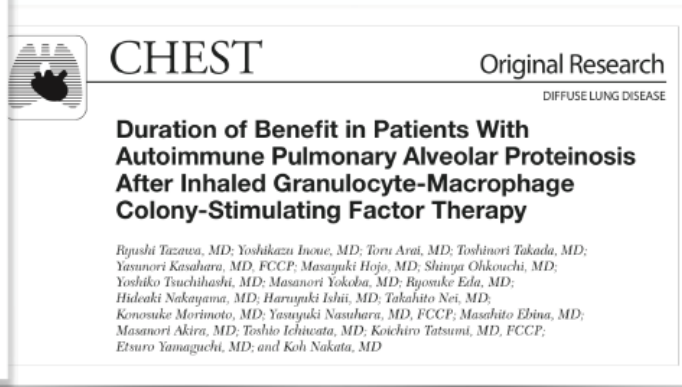
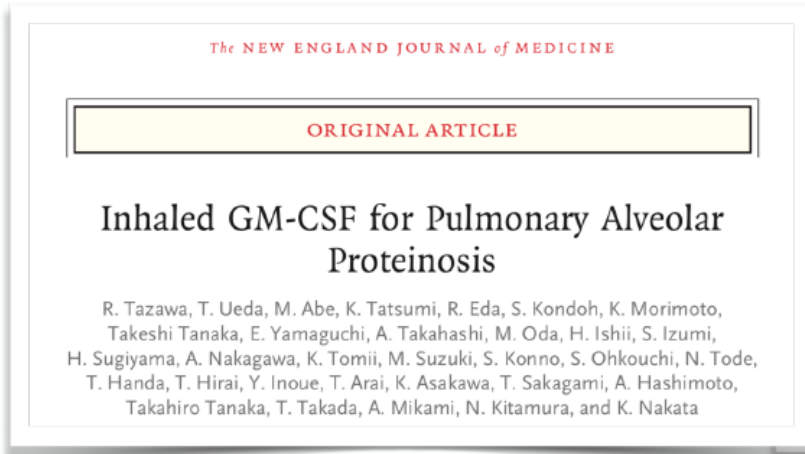
(01)00371837584316

Labeled indications:

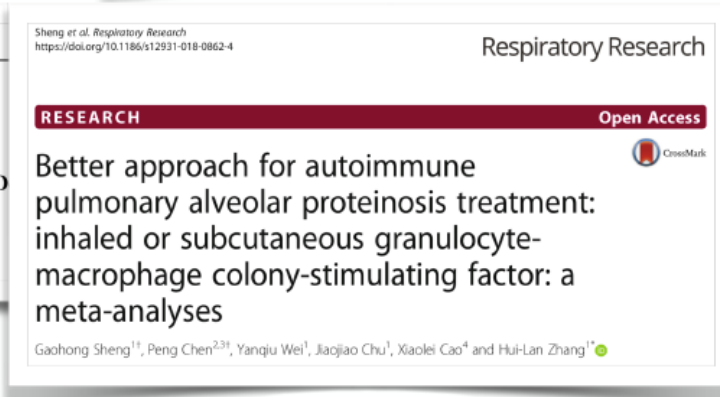
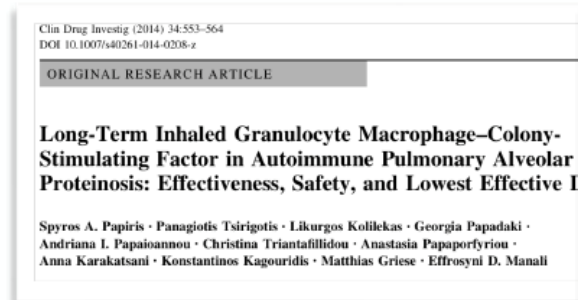
- 1) Shorten time to neutrophil recovery and reduce incidence of severe life-threatening, and fatal infections in adults over 55 years old after induction chemotherapy for acute myeloid leukemia
- 2) Mobilization of hematopoietic progenitor cells for autologous transplantation in adults
- 3) Acceleration of myeloid reconstitution after autologous stem cell transplantation
- 4) Acceleration of myeloid reconstitution after allogeneic stem cell transplantation
- 5) Treatment of delayed neutrophil recovery or graft failure after bone marrow transplantation in adults or children at least 2 years of age
- 6) To increase survival after acute exposure to myelosuppressive doses of radiation

Inhalation Safety Data Supports Investigational Use

Existing Product, Delivered with Standard Nebulizers



The Aerogen® Ultra
High-Performance Aerosol Drug Delivery



Chronic investigational nebulized therapy – feasible and tolerable

Anti-infectious, including antifungal, mechanisms of GM-CSF



- Increase proliferation of myeloid-derived cell, including neutrophils
 - Activate and differentiate myeloid cells, including macrophages and dendritic cells
1. Enhance neutrophil **oxidative burst** and antifungal activity via NADPH oxidase¹
 2. Limit dimorphic fungus survival in macrophages through **zinc sequestration** and ROS generation²
 3. Increase expression of pattern-recognition receptor **Dectin-1** in macrophages³
 4. Increase expression of **myeloperoxidase** in neutrophil extracellular traps⁴
 5. Increase **chitotriosidase** expression (cleaves *Candidal* chitin)⁵

Proof of Mechanism: murine *Aspergillus* model⁶



Summary – Sargramostim for Fungal Infections

1. Nebulized and systemic sargramostim **available now** for investigational use
2. Inhalational safety data support **combination testing** with antifungal agents
3. Nonredundant mechanisms demonstrate *in vivo* **proof of mechanism**

We Seek To Partner To Advance Antifungal Therapy

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