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Office of Technology Management

THERAPY TO TREAT AND PREVENT RESPIRATORY DISEASE IN PRE-TERM AND AT-RISK NEONATES

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Technology Description

Researchers in Prof. S. Celeste Morley's laboratory have developed a long-lasting prophylactic therapy that could protect pre-term and at-risk infants from pneumonia, respiratory distress and bronchopulmonary dysplasia. Neonates born prematurely are at increased risk for lung disease, in part because their immune system is not fully developed. This invention could protect those children by giving them a boost of rhGM-CSF (recombinant human granulocyte macrophage colony stimulating factor) during a crucial phase after birth to enhance innate immune development in the lungs. Specifically, this local, intra-tracheal treatment augments alveolar macrophages (AM), showing sustained effects that are not seen with systemic GM-CSF administration. The AM are then able to defend against neonatal bacterial pneumonia and chronic lung disease without perturbing lung development. Because rhGM-CSF is an FDA-approved agent with an established safety profile, it could potentially be translated to clinical use in preterm infants to improve outcomes in this highly vulnerable patient population.

Stage of Research

In vivo studies - The inventors have used a mouse model for pneumonia susceptibility to demonstrate that GM-CSF administered intranasally on days 1-3 of life continued to protect the mice from bacterial lung infection 8 weeks later. These mice also had enhanced alveolar macrophage production that persisted into adulthood with no detrimental effects on surfactant metabolism or alveolarization.

Applications

- **Therapeutic to prevent or treat respiratory conditions**:
  - prophylactic therapy for **pre-term or term neonates** at-risk for pneumonia, respiratory distress or bronchopulmonary disease
  - potential for delivery in combination with surfactant treatment

Key Advantages

- **Long term protective effects –** mice that received neonatal treatment (1-3 days) with GM-CSF were protected from bacterial infection into adulthood (8 weeks)
- **Known safety profile:**
  - recombinant GM-CSF and biosimilars are FDA-approved drugs
  - local administration is likely to reduce systemic side effects

Patents

Provisional patent application filed

Website

- Morley Lab