

Problem

Cost/difficulty of production of GM-CSF

Technology Overview

Chemically synthesized GM-CSF mimetic/GM-CSF receptor agonist

IP Status

- ▶ PCT application pending
- ► Includes composition claims
- ▶ Available for Licensing

Value Proposition

- ► Lower production costs
- ▶ Restarts IP protection

Market Attractions

- Existing indications for the use of Leukine (GM-CSF)
- ► New indications including:
 - Alzheimer's disease
 - Neurodegenerative diseases
 - o Cognitive decline
 - Down Syndrome

Development status

Tested *in vitro* and *in vivo* on mice.

Contact

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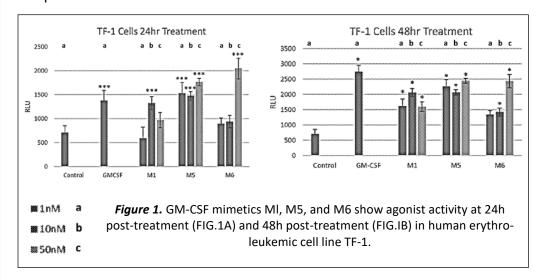
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Granulocyte Macrophage Colony Stimulating Factor (GM-CSF) Mimetic

<u>Problem</u>: Dr. Huntington Potter at the University of Colorado Anschutz Medical Center has shown that GM-CSF (recombinant human Granulocyte-Macrophage Colony-Stimulating Factor such as the commercially available Leukine) can slow or reverse Alzheimer's disease (AD). In a mouse model of AD, Leukine reduces amyloid deposition by more than half and restores normal cognition after a few weeks of treatment. Clinical trials are currently being carried out, but continuous treatment of AD patients with Leukine would be expensive at its current cost (>\$200,000/year/patient), and if effective, it would be difficult to produce sufficient quantities given production challenges associated with the biologic. Thus, more cost-effective options are needed.

<u>Technical Solution and Key Value Propositions:</u> Dr. Potter has developed new GM-CSF peptides that act as GM-CSF mimetics. The mimetics are smaller in size and easier to synthesize than GM-CSF itself. They also show improved bioavailability, improved stability, easier administration, improved blood brain barrier permeability, and improved oral bioavailability. The GM-CSF mimetics should also avoid development of GM-CSF autoantibodies when administered to a patient.



Key Documents and Sources:

- PCT application WO 2018227142A1. GM-CSF mimetics and methods of making and using same
- Boyd et al. *GM-CSF upregulated in rheumatoid arthritis reverses cognitive impairment and amyloidosis in Alzheimer mice*. J Alzheimer's Dis. 2010; 21(2):507-18.
- Jim et al. Granulocyte Macrophage Colony Stimulating Factor Treatment is Associated with Improved Cognition in Cancer Patients. Brain Disord Ther. 2012;1(1).