Docket #: S13-065

TGF Beta Agonists for Neuroprotection and Treatment of Alzheimer's Disease

Prof. Tony Wyss-Coray and colleagues have identified a first-in-class small molecule with the potential for treating Alzheimer's disease and reversing some of its pathology. This compound was discovered due to its agonist effect on TGF-beta (a protein that has been shown to reduce ABeta accumulation in mouse models of Alzhemier's disease). Pre-clinical studies have demonstrated that oral administration of this agent can delay or reduce neurodegeneration and cognitive impairment in a mouse model of Alzheimer's disease. In addition, the molecule provided neuroprotection in a mouse model of neuronal damage.

Stage of Research
The inventors have performed a wide range of pre-clinical studies and are planning to apply for grants to support an IND filing. Studies to date include:

- characterization as orally active drug with minimal toxicity
- testing to verify activation TGF-beta signaling in the CNS
- demonstration of neuroprotective effects in vivo with models of acute neurodegeneration and Alzheimer's disease
- behavioral studies in mice to show the orally administered compound can delay or rescue memory deficits
- large animal toxicology

Applications

- **CNS therapeutic:**
    - to potentially improve cognition and slow progression of Alzheimer's disease

- to treat other neurological disorders

Advantages

- **First-in-class molecule** - unique mechanism of action that could both clear the underlying plaque pathology believed to cause brain damage in Alzheimer's disease and provide neuroprotection that enhances cognition
- **Unmet medical need** - Alzheimer's disease has no cure and currently available drugs afford only modest symptomatic benefits

Publications

- Benzyl Urea Derivatives for Activating TGF-Beta Signaling (PCT Application WO 2014152869)

Patents

- Published Application: WO2014152869
- Published Application: 20160039756
- Issued: 9,796,674 (USA)

Innovators

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