MJFF PARTNERING PROGRAM

THE MICHAEL J. FOX FOUNDATION FOR PARKINSON'S RESEARCH

FPRT Bio Inc.
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Organization and Team Overview

FPRT Bio Inc. is a clinical stage biotech company formed to develop XPro1595 for the treatment of neurodegenerative diseases including Parkinson's disease. The management team is small, experienced and focus to allow efficient use of capital.

Opportunity Overview

FPRT Bio Inc. is raising Series A funds to support clinical development of XPro1595 in neurodegenerative diseases including Parkinson's disease. XPro1595 was in-licensed with a complete IND package. No additional pre-clinical work needs to be performed. All funds raised in the Series A will be used to support the clinical trials. XPro1595 will be tested using a unique Proof-of-Biology Phase I design that allows both safety and biologic efficacy to be tested. This strategy allows for accurate assessment of the value of the drug in the disease and lowers the risk of the development process. Clinical trials will start as soon as financing is available to support a clinical trial. We are anticipating closing the Series A in May 2014. Investors can expect a high value exit in less than 4 years.

Parkinson's disease has neuroinflammation and microglial activation as a prominent part of its pathology. Currently, there are no treatment strategies in development to treat this harmful inflammatory process. XPro1595 has a unique attributes that make it an ideal treatment for Parkinson's disease. XPro1595 is a selective inhibitor of soluble TNF (sTNF). sTNF is the master cytokine in the brain causing chronic neuroinflammation and is directly toxic to dopaminergic cells. By blocking sTNF, XPro1595 is neuroprotective disease modifying drug. Because XPro1595 also crosses the blood-bran-barrier (BBB), is given as a patient friendly once a week subcutaneous injection (like and insulin shot) and can be used with any drugs the patient is already on.

Details of MJFF Grant

The purpose of this study was to test if peripheral administration of XPro1595 was a viable option for treating patients with Parkinson's disease. Earlier work supported by the MJFF (in Malu Tansey's laboratory), demonstrated that DN-TNF analogs were effective in treating Parkinson's disease in the 6-OHDA model either as a direct infusion (XENP345) or as a viral vector. Because delivery of XPro1595 directly to the brain in patients was not an attractive treatment option, other alternatives were sought. This study, if positive, was to be last pre-clinical study before a clinical trial.

The title of the project is: ***Peripheral administration of XPro1595 in the rat 6-OHDA model of Parkinson's disease.*** The purpose of the study was to determine if peripheral administration of XPro1595 would alter the Parkinson like disease symptoms in the 6-OHDA model and determine the mechanism of that effect. That is, are the effects of XPro1595 due to a direct anti-inflammatory effect in the brain or an indirect effect due to a decrease in peripheral inflammation?

Results and Potential Next Steps

The interim results were presented in a poster format at the annual MJFF meeting in October. Briefly, XPro1595, when given early after the 6-OHDA lesion rescued a significant portion of nigral dopaminergic neurons (80% survival in treated vs 45% survival in vehicle treated animals, P=0.03). CSF levels of XPro1595 were 3 ng/ml. Overall, peripheral administration of XPro1595 crossed the BBB, gained entry to the CNS, reached therapeutic levels and had a positive impact on the disease pathology. The remaining work will focus on the mechanism of the rescue. That is how much of the benefit was due to peripheral immune effects, central immune effects or a combination of the two. The clinical benefit to the animals must also be determined. Finally, the mechanism of BBB permeability will be accessed. At the conclusion of this work, we should understand how XPro1595 rescued the dopaminergic neurons.

This work sets the stage for a clinical trial. We know that XPro1595 rescues dopaminergic cells in the setting of neuroinflammation. The goal of a disease modifying therapy of Parkinson's disease is to prevent progressive dopaminergic cell death. We also know that XPro1595 can be given as a patient friendly peripheral subcutaneous injection and show benefits. This is how the drug will be given in the clinical trials.

Intellectual Property Status

The composition-of-matter IP on XPro1595 and other DNF-TNF analogues has issued. There is a decade of protection remaining.

For additional information, please contact: ResearchPartnerships@michaeljfox.org