Development of a new drug to treat mitochondrial disease and Parkinson's disease through enhancement of cellular bioenergetics

EnePharma Inc. was incorporated in June 2019 for the development of a new therapeutic class of **ATP production enhancers** that have application for the treatment of **Parkinson's and mitochondria-related diseases** and have potential to impact the aging process. EnePharma aims to become the global leader in the development of its ATP enhancer technology, which has large market potential. Towards this end, in early phase clinical trials of EnePharma's proprietary ATP enhancing combination of febuxostat and inosine, we have demonstrated early evidence of activity and a strong therapeutic index.

EnePharma

EnePharma Inc.
www.enepharma.com
c/o Adrian Rich, esq., Perkins Coie LLP
3150 Porter Drive, Palo Alto, CA 94304, USA
E-mail: info@enepharma.com
Management team
Nao Kamatani, CEO
Yoshi Shima, Secretary
Founded: June 2019

Rationale: Energy (ATP) associated human genes comprise 8 % of all genes and many diseases are caused by "energy shortages". Because of their key importance in human disease, ATP enhancement therapies have the potential to treat a wide-variety of human maladies. Mounting evidence from basic and clinical data strongly suggests that many of the neurodegenerative disorders including Parkinson's disease are caused by energy shortage and among rare diseases, mitochondrial diseases such as CPEO, MELAS, MERRF, and Leigh encephalopathy are unequivocally caused by energy shortage. By the intensive analyses of the association between genes and diseases, we have succeeded in designing methods to enhance concentrations of the major purine compound ATP in humans by both providing an extra source of purines and inhibiting purine degradation, i.e. by the administration of our proprietary combination of febuxostat and inosine.

Safety and efficacy supported by clinical data: Febuxostat and inosine have been co-administered to 65 subjects (18 healthy subjects and 47 patients), and the data from these trials support the safety of this combination. ATP and hypoxanthine were markedly increased in healthy subjects by the co-administration of febuxostat and inosine for 2 weeks but not by administration of either compound alone. In patients with Parkinson's disease treated with our drug combination, significant improvement ($P = 0.0146$) of the MDS-UPDRS Part III score with more than a minimal clinically important difference (MCID = -3.25) was achieved in 26 patients treated for 8 weeks. Likewise, in two patients with mitochondrial disease, co-administration of febuxostat and inosine for 2 weeks dramatically improved a biomarker of cardiac failure (BNP) in a mitochondrial cardiomyopathy patient and a biomarker of diabetes (insulinogenic index) in a mitochondrial diabetes patient.

Current development status: EnePharma is now preparing for pre-IND meetings with the U.S. FDA for Parkinson's and mitochondrial diseases based on our preclinical and clinical data. In terms of corporate development, we are solidifying our corporate structure and enhancing our scientific advisory board, which is anchored by leading academic authorities.

EnePharma Inc. is seeking additional capital to continue the clinical development of its promising febuxostat and inosine combination for the treatment of major market human diseases for patients with limited therapeutic options and dismal prognoses.