ERAD Therapeutics Inc. Update

SYNOPSIS

ERAD Therapeutics Inc. is developing drugs to treat Gaucher Disease, Cystic Fibrosis, and potentially other genetic diseases that result from the creation of "mis-folded" proteins. Our unique drug platform is based on the finding that an inert component of cholera toxin called the *B sub-unit* (CTB) can be engineered to deliver enzymes and proteins to all cells throughout the body—including to brain cells, across the "blood brain barrier."

Our technology has allowed us to create two novel approaches to treating ERAD diseases.

1. Enzyme Replacement Therapy that Crosses the Blood Brain Barrier

CTB-linked Enzyme Replacement Therapy (ERT) will provide a superior treatment for patients with Gaucher Disease. By crossing the blood brain barrier (BBB), this should allow for prevention and/or treatment of previously unaddressed neurological conditions associated with all three forms of Gaucher Disease. While there are currently three FDA approved ERTs for Gaucher Disease, with $1.6 billion in annual sales, none of them can cross the BBB to address these often fatal neurological conditions.

Of critical importance to our company has been the emergence of an unanticipated link between Gaucher Disease and Parkinson Disease. While an absolute deficiency of a critical enzyme (glucocerebrosidase) causes Gaucher Disease, a relative deficiency of the same enzyme in the brain has recently been closely associated with all forms of Parkinson Disease. This discovery indicates that our ERT, which has unique access to brain cells, potentially offers a new therapeutic option for Parkinson's. This has a potentially significant impact on the value of our ERT product.

2. Modified Cholera Toxin Licensed from SickKids Hospital (Toronto, CA)

Patients with ERAD diseases produce mis-folded versions of certain proteins critical to cell function. The cell recognizes that these proteins are mis-folded and routinely degrades them, leaving patients with a debilitating and sometimes fatal protein deficiency. This is unfortunate, however, because some of these mis-folded proteins retain a degree of normal activity. Were a cell to preserve rather than degrade them, it would not develop a disease state.

Certain bacterial toxins, in particular the cholera toxin and verotoxin, can reversibly stop the intracellular pathway for the destruction of mis-folded proteins. "ERAD Blockade Technology" created at SickKids Hospital in Toronto, Canada employs CTB to deliver a genetically modified and harmless version of the active component of the cholera toxin (called mCT's). This formulation, by interfering with the degradation of these imperfect but functional proteins, prevents and/or treats certain ERAD diseases including Gaucher Disease and Cystic Fibrosis.

ERAD THERAPEUTICS INC. ACCOMPLISHMENTS TO DATE:

- Development of mCTs for ERAD blockade

- Demonstration of protein rescue and restoration of near normal cellular function in cell lines in both CF and GD Type 1 and also in GD Type 1 mouse model
- Raised ~ $1,000,000 for corporate and scientific development activities
- Third party validation of CF activity via Charles River Laboratories Cambridge, MA
- Incorporation in USA to access large capital pools
- **Filed new Intellectual Property surrounding the exploitation of CTB to transport therapeutic molecules across the BBB**
- Collaboration with NRC of Canada for the manufacture of our first two products

MOVING FORWARD

The company is focusing on two development programs:

(1) CTB-GCC - exploitation of the ability of the CTB subunit to deliver the deficient enzyme in Gaucher Disease across the BBB and thus address all three types of GD. This molecule may also prove valuable in the treatment of idiopathic Parkinson disease.
   a. Collaboration with the NRC of Canada to manufacture CTB-GCC and working with the Beyond the Blood-Brain-Barrier group to validate its delivery across the BBB and its activity in the lysosomes.

(2) mCT0 – use of mCT0 to rescue the del508CFTR protein in CF to restore cellular function.
   a. Collaboration with the NRC to manufacture mCT0 and validate its activity in restoring cellular function in CF cell lines and mouse model.

Upon completion of our current development programs by December 31, 2017, the company intends to have a clear path forward to clinical trials in both Gaucher Disease and Cystic Fibrosis. We are currently seeking $2.5 - $3 M USD to accomplish these goals.

In recent years, the Pharmaceutical industry has changed its acquisition strategies in two manners important to the company. First, there has been a dramatic shift to acquiring companies focused on Orphan Drug development. Second, there has been an equally dramatic shift to acquisition of pre-clinical companies or in-licensing their technologies. In 2016, acquisition or in-licensing deals in the pre-clinical stage represented 48% of all deal activity.

Examples of Pre-clinical Deals: the following are three examples of pre-clinical deals (from a list of 15), for platform technologies or single products, announced between early 2015 and 2016 between big pharma and small biotech:

1. GSK- Zymeworks[1]: in December, 2015 Zymeworks announced a collaboration deal with GSK to develop Zymeworks EFECT® platform (tailored Fc domains for immunotherapy) wherein GSK has rights to develop 4 products. Upfront & milestone payments of up to $110M USD per product.
2. Amgen – Advaxis[2]: in August 2016, Amgen in-licensed Advaxis' pre-clinical cancer immunotherapy candidate ADXS-NEO, for treatment of multiple cancers, in a deal with a $40 M USD up-front payment, plus a $25 M USD equity infusion with potential payments of up to $540 M USD
3. CSL-Behring2[2] – Calimmune: August 2017, CSL-Behring has paid an upfront fee of $91M USD with additional payments taking the deal up to or over $300 M USD for Calimmunes Hematopoietic stem cell therapies including two platform technologies aimed at addressing major challenges currently faced in stem cell therapies.

[1] Zymeworks website Dec. 3, 2015

[2] Fierce Biotech