Virtus Therapeutics, LLC
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https://virtustherapeutics.com

Industry: Biotech

Management:
Choukri Ben Mamoun, PhD, Yale University, Founder
Jessica Regan, PhD, Scientific Director

Advisory Board:
Stephen Chang, PhD, Mirimus
John Puziss, PhD, Dartmouth

Finance:
Accounting/Tax:
Marcum, LLP
ADP
BK:Rose Wang, MBA

Funding to Date:
- PITCH: $736K
- Blavatnik: $300K
- Foundations: $150K
- NIH SBIR: $799K

Funding Sought**:** $5-10M
For:
- IND enabling safety and toxicity
- Phase I/II clinical trials

IP:
Yale 63/043,534 valid until 2040

Legal:
Evan Kipperman |Wiggin and Dana, LLP

Company Description / Background:

Virtus Therapeutics is a spin-off of the Ben Mamoun Laboratory at Yale University. Virtus is developing small molecule therapies for the treatment of the rare, pediatric disease, Pantothenate Kinase-Associated Neurodegeneration (PKAN).

Market Opportunity/Unmet Need:

PKAN is a devasting, rare, pediatric neurodegenerative disease. Patients suffer debilitating cognitive and developmental deficiencies, motor impairment, and often death in early adolescence. There is currently no approved disease-modifying treatment for PKAN, leaving patients and their families with limited options and a poor quality of life. PKAN has an incidence of 1-3 per million births with approximately 1,200 cases in the US and Europe. As a rare orphan disease, our compounds will have an accelerated regulatory pathway, commercial exclusivity, and an estimated cost per patient per year of $300K in the US. Our groundbreaking approach has the potential to not only address PKAN but also to pave the way for similar treatments in other neurodegenerative diseases, expanding the market opportunities.

Product:

Virtus Therapeutics is developing first-in-class small molecule compounds, termed VTACs, which target the core pathological processes of PKAN, offering a more precise and efficacious treatment compared to existing approaches.

Commercial/Technical Milestones:

Preclinical *in vitro* and in vivo data have demonstrated that VTACs are able to target the underlying cause of the disease and bypass the genetic defect found in PKAN patients to restore normal cellular physiology. *In vivo* efficacy studies are underway to determine whether VTACs are capable of extending survival of PKAN mice. Our upcoming milestone includes IND-enabling safety and toxicity studies to advance VTACs to clinical trials with first in human studies beginning by 2028.

Competition / Competitive Advantage:

CoA Therapeutics (BridgeBio) has developed a small molecule targeting PKAN; however, they were unable to identify an effective therapeutic window due to inhibition by acetyl CoA, and thus no therapy emerged. Travere Therapeutics has developed a metabolite supplement; however, a Phase III clinical trial was terminated with no disease modifying therapy emerging. ApoPharma developed an iron chelator, deferiprone, for which Phase II trials were completed, however, no effective therapy emerged. Our compounds are the only molecules currently under development which are not inhibited by acetyl CoA and target the underlying cause of the disease to slow disease progression rather than to only treat symptoms.

Financial Forecast:

	Year 1	**Year 2**	**Year 3**	**Year 4**	**Year 5**
Net Revenue	$7.6M	$15.1M	$37.8M	$75.6M	$126M
Gross Profit	**$**6.7M	**$**12.8M	**$**33.9M	**$**69.8M	**$**115.5M
Gross Margin	88%	85%	90%	92%	92%