VIRTUS THERAPEUTICS

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Industry:
- Biotech (pre-clinical stage)
- Small-molecule therapeutics

Management:
Executive leadership:
- Choukri Ben Mamoun, PhD (Founder)
- Anasuya Pal, PhD (Scientific Director)
- Seeking CEO

Board Members:
- Choukri Ben Mamoun, PhD
- Stephen Chang, PhD
- John Puziss, PhD

Scientific Advisory Board:
- Stephen Chang, PhD
- Aaron Nilsen, PhD
- Jaime Grutzendler, MD
- Mark Plummer, PhD

Number of employees: 1

Finance:
Auditor:
- Rose Wang, MBA

Investments: $1.76M
- $768K: Program in Innovative Therapeutics for Connecticut Health (2017)
- $300K: Yale Blavatnik Fund (2021)
- $698K: NIH Phase I SBIR (2023)

Financing Sought: $5M
- $3.5M: Completion of pre-clinical studies
- $1.5M: IND-enabling studies

Legal:
IP contact:
John Puziss, PhD

Business Description / Company Background:
Established in 2020, Virtus Therapeutics is a Yale-University spin-out dedicated to developing first-in-class compounds for treatment of rare and life-threatening metabolic diseases. Breakthrough research by Dr. Choukri Ben Mamoun, Yale professor and company founder, led to the discovery of VTACs (Virtus Therapeutic Activator Compounds)—a class of small molecules that potently activate a critical enzyme in the biosynthesis pathway for Co-enzyme A (CoA). CoA is an essential metabolic cofactor involved in several vital pathways, including cellular energy production. Diseases stemming from genetic defects in CoA metabolism result in significant pediatric mortality and morbidity and currently comprise 47% (28/60) of the conditions on the U.S. Recommended Uniform Newborn Screening Panel. Despite such aggressive diagnosis, virtually all these diseases lack any approved therapies. Virtus Therapeutics aims to transform patient outcomes by bringing VTACs to market as a safe, effective, and convenient oral therapy for these diseases.

Market Opportunity / Unmet Need:
One of the first indications for VTACs will be for propionic acidemia, a rare genetic disease in which the body cannot properly break down protein and fats, resulting in toxic metabolite accumulation. Propionic acidemia affects 1 in 100,000 newborns in the U.S, leading to acute life-threatening metabolic crises, and lifelong complications such as intellectual disability (55% of patients), seizures, cardiomyopathy, vision loss, and kidney failure. Death by young adulthood is prevalent among patients. Outside of drastic dietary and surgical interventions, which incur a lifetime cost of $1-1.5M, there is currently no FDA-approved therapy for propionic acidemia. There exists a significant unmet need for a targeted, disease-modifying treatments. The global market for this disease was recently valued at $1.3B (Straits Research), growing annually by 3.3% over the next decade due to improved diagnosis and clinical management.

Products / Services – Launched & Pipeline:
Two compounds, VTAC-1 and VTAC-2, have been designated as early leads based on superior activation of the enzyme target, exceptional safety in human cell lines, and favorable physio-chemical properties for drug development. In mice, pharmacokinetic studies on VTAC-1 and VTAC-2 showcase substantial plasma and brain exposure, commendable half-lives, and no apparent toxic effects. Pre-clinical experiments testing efficacy of early leads in cell line and animal models of disease are currently underway to identify a late lead drug for chemical structure optimization.

Commercial and Technical Milestones:
- 2017-2023: Raised nearly $2 million in non-dilutive funding to-date
- 2020: Patented filed for VTACs (Yale IP 63/043,534 valid until 2040)
- 2021: Completed chemical screen resulting in target and early lead identification
- 2022: Commenced pre-clinical studies in mice
- 2023: Opened physical lab space in Groton, CT; hired first employee
- 2027: Completion of pre-clinical and IND-enabling studies.

Competition / Competitive Advantages / Customer Benefits:
Currently, there is no FDA-approved therapy for propionic acidemia, and no small molecule treatment for this disease is in active clinical trials. Moderna has developed an mRNA-based enzyme replacement therapy presently in Mixed Phase 1/2 clinical trials for propionic acidemia. However, the safety risk of chronic mRNA administration is not yet known, and early clinical reports have revealed an alarming number of serious adverse events among trial participants. Compared to small molecules, such mRNA therapy is more expensive to manufacture and can only be administered by IV infusion, requiring hospital visits 1-2X/month. VTACs are expected to capture 75% of patient market based on predicted efficacy, safety, and relative ease of administration (oral pill/liquid taken 1x/week).

Financial Forecast:
Priced at $350K per patient per year, VTACs are estimated to generate an annual revenue of $200-350M upon FDA-approval. Due to orphan and pediatric disease status, VTACs are expected to receive up to 12 years of FDA-granted exclusivity for protection against generics.

($ in millions)	Year 1	Year 2	Year 3	Year 4	Year 5
Revenue	$28.6	$148.6	$233.1	$244.7	$257.9
Gross Profit	$22.9	$124.9	$200.4	$215.3	$232.1
Gross Margin	80%	84%	86%	88%	90%