CapCell Biologics, Inc.
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Industry: *Biotechnology*

Product: *Implants to treat rare disease using cell encapsulation technology developed by NASA.*

Funding Sought: *$150,000 for implant prototyping and small-mammal studies for 18 months. Follow-on raise of $1.2M to complete pre-clinical research.*

Funds to date: *$10,000 (Loyal VC)*

Solution Management:
Ben Young, CEO
Jon Zaikowski, President
Dr. Gorka Orive, Advisor
Cyndi Frank, Patient Advisor

Company Focus: CapCell Biologics is developing cell therapies for patients with lysosomal storage diseases. The current standard of care for these diseases is a biweekly infusion requiring 1.5-6 hours per session. Our first target is Fabry disease, which produced $855M in therapeutic sales in 2018. CapCell's proprietary cells produce therapeutic proteins and are implanted into patients inside a carbon nanotube macrostructure. The carbon nanotubes form a porous mesh that allows gas exchange to occur while preventing an adaptive immune system reaction to the cells. In addition, the carbon nanotubes themselves have low immunogenicity and can be controlled to reduce fibrosis and promote vascularization.

Market Opportunity: Lysosomal storage diseases are a group of approximately 50 rare and ultra-rare metabolic disorders with $6.4B of therapeutic sales predicted for 2019, according to Thomson Reuters. The majority of these diseases have no treatment available and our platform technology is well-suited to meet this need. Further targets include cancer, Crohn's, colitis, diabetes, Parkinson's, rheumatoid arthritis, and more.

Product/IP: The process to manufacture carbon nanotube capsules is protected by a NASA originated methods patent. We are finalizing negotiations with NASA for an exclusive license to lysosomal storage diseases and will develop derivative IP as we refine the procedure to manufacture capsules. We are also collaborating with Weill Cornell Medical College to develop proprietary cell lines.

Competitive Advantages: The current standard of care for lysosomal storage diseases is enzyme replacement therapy, requiring patients to undergo lengthy infusions every two weeks. There is universal dissatisfaction among patients with this treatment due to its **time-consuming** nature, **painfulness**, and **sub-optimal clinical outcomes**. We expect to capture a significant portion of the market as 78% of patients interviewed indicated they would switch to implants if available.

Commercial Milestones: NASA has performed safety/toxicity studies in rabbits that yielded no negative results. Initial cell engineering at Weill Cornell has also proven successful with a first generation expected by June 2019. We anticipate completion of capsule prototyping and first safety studies in 2020, with GLP studies in 2022 and a BLA application in 2023.

Financial Projections (Unaudited): Lysosomal storage diseases are a growing market. The following numbers are from Persistence Market Research, Thomson Reuters, and Bernstein:

- Annual therapeutic sales are currently $6.4B with CAGR estimated from 7.0%-10.2%
- This results in a **TAM between $11.8B and $15.3B** by 2028 (estimated approval year)
- COGS in this space are low, typically 11-14% of revenue

Purpose of Presentation: Investment of $150,000 for prototyping and first safety studies.