[348] MITOCHONDRIA AUGMENTATION THERAPY FOR RARE MITOCHONDRIAL DISEASES

Natalie Yivgi Ohana[1], [1] Minovia Therapeutics

- **Investment Rational**

- Minovia's proprietary technology aims to save the lives of millions of people suffering from devastating diseases with no cure. The company is developing a biological platform technology called Mitochondria Augmentation Therapy: transplanting healthy mitochondria in diseased cells. The involvement of damaged mitochondria in the aging process suggest this method may be effective in treating many age-related diseases.
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- **Business Strategy**
  Minovia's go to market strategy is to focus on a group of rare mitochondrial diseases, affecting mainly children with no available treatment. There are several advantages to entering the rare disease market: faster regulatory track, lower costs, shorter time to market and high treatment price. Moreover, drug approval for such diseases yield Priority Review Vouchers, sold for more than $100M
- **Core Technology**

  Our products are compositions of functional mitochondria isolated in a proprietary process from placentas or from white blood cells and frozen, yielding off-the-shelf therapeutic products. We developed a therapeutic process based on autologous bone marrow transplantation, using patient's own cells as carriers of normal mitochondria. We have seven submitted patent applications, four PCT's, all privately owned by Minovia.

- **Product Profile/Pipeline**

- We have recently conducted mitochondrial therapy in three patients with a rare mitochondrial disease (Pearson Syndrome). Preliminary results show safety of the treatment and accumulating evidence of efficacy. Data collection is still on-going. Successful pre-pre IND meeting with the FDA's Center for Biologics Evaluation and Research, encouraged to advance IND submission.
  Clinical manufacturing processes defined and under development.

  What's Next?

- Minovia is currently preparing for IND submission, followed by a formal clinical trial expected to begin in Q4 2018 to expedite drug approval. The results are expected to support the efficacy of mitochondrial therapy, allow new IND submissions and to facilitate clinical trials for other mitochondrial and age-related diseases.