Abstract: Lempo Therapeutics - Pioneering CRISPR Gene Editing to Calibrate the Immune System and Eliminate Collateral Damages

Company name Lempo Therapeutics Ltd. * Website: www.lempo-tx.com *

CEO name Oren Glanz

CATEGORY: Biotech/Pharma

SESSIONS: Personalized Pathways; Genetic and cellular based Advances for Rare and Complex Disease

- **Investment Rationale**

We're developing CRISPR therapy targeting the immune system myeloperoxidase (MPO) for attenuating inflammatory and autoimmune diseases. Next year, we'll launch US clinical trials of MP001 for the $1B+ SSc-PAH market and plan expansion to large-scale indications later-on. We're funded through FIH studies including by NDF from CIRM. Our rapid progress stems from our work with truly outstanding renowned experts.

- **Business Strategy**

We will commercialize MP001 for SSc-PAH while developing an encapsulated in-vivo delivery version for larger-scale diseases including CVD, neurological, and renal diseases. Our near-term revenue will come from ex-vivo therapy for rare diseases ($2M per patient), while long-term growth will stem from the in-vivo version, targeting the broader inflammatory-derived and autoimmune disease market worth tens of billions.

- **Core Technology**

Our technology uses CRISPR/Cas9 gene editing to knockout MPO in hematopoietic stem cells, significantly reducing the immune system collateral damage while preserving immune function. Our approach is uniquely de-risked because MPO deficiency occurs naturally in humans without compromising immunity, addressing a root cause of tissue damage in multiple inflammatory conditions. Pre-clinical studies demonstrated excellent safety profile as well as efficacy.

- **Product Profile/Pipeline**

MP001 ex-vivo version targets SSc-PAH (5,400 US patients) with expansion potential to 30,000 broader PAH patients. Following positive FDA pre-IND feedback, our IND-enabling studies are underway, with IND submission for US trials planned for 2026.

MP001 in-vivo version is in development and will target large-scale indications including Alzheimer's disease, in which POC in animal model already showed the potential effect.

- **What's Next?**

We will complete IND-enabling studies and prepare for First-in-Human trials in 2026 with Professor Donald Kohn at UCLA. We plan to initiate local US presence for manufacturing and business development and are seeking strategic partners for the in-vivo delivery development and for advancing our ex-vivo commercialization. No additional capital is needed for our initial clinical trials.