SpliSense: Transformative RNA Based Treatments for Orphan and Large Unmet Pulmonary Diseases

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ABSTRACT TEMPLATE: CHECKLIST AND INSTRUCTIONS

Company name SpliSense Ltd.* Website splisense.com *

CEO name Dr. Gili Hart* Cell phone number 054-2424554 *

CATEGORY: Biotech/Pharma

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

- Executive Summary
  - SpliSense is a clinical stage company focused on the development of RNA-based treatments for pulmonary diseases. Our platform harnesses Antisense Oligonucleotides (ASOs) for treatment of rare CF mutations and complex pulmonary diseases; muco-obstructive diseases (asthma, COPD, NCFB) and rarer diseases (IPF). The company has raised over $40M and is currently running a Phase 2 clinical trial for its lead product.
- Core Technology
  - SpliSense's technology is based on clinically validated ASO technology, delivered by inhalation. Using its proprietary algorithms for optimizing its ASOs, SpliSense aims to target the genetic cause of the disease by restoring/reducing protein function. ASO technology allows a precise organ targeting approach, with demonstrated proper lung and cells distribution and safety, which will hopefully lead to lung function improvement.
- Product Profile/Pipeline
  - SPL84 is a treatment for CF patients carrying a specific splicing mutation (3849), designed to correct the aberrant splicing of the CFTR mRNA
  - SPL5AC is a treatment for muco-obstructive diseases, designed to lower the over-expression of the mucus gene, MUC5AC

    - SPL5B is a treatment for IPF, designed to lower the over-expression of the mucus gene, MUC5B
- Business Strategy
    - The company is advancing its core programs with the aim of bringing them into the clinic with the long-term goal of marketing approval. In the short-term, the company is in discussions with regulatory authorities about accelerated approval for its rare disease programs.

- What's Next?
    - The company’s most advanced program, SPL84, is being evaluated in an ongoing global phase 2 study, with the goal of accelerated approval. It is expected that the SPL5AC program for muco-obstructive diseases and SPL5B program for IPF will advance to the clinic in 2025-6.