

Questions for categories: **Biotech/Pharma**

Investment Rational

SpliSense is an Israel-based company focused on transformative RNA-based treatments. SpliSense pioneering platform harnesses Antisense Oligonucleotides (ASOs) for treatment of genetic diseases such as Cystic Fibrosis and pulmonary diseases.

Business Strategy

SpliSense, proprietary algorithms for modulating and optimizing its ASOs properties and splicing activity to target sequences. This allows a precise targeting approach, enabling manipulation of specific exons without impacting other regions of the sequence or nonspecific genes. In addition, the company has established its ASO combined delivery system for proper lung delivery, distribution and cell penetration for patients benefit.

Core Technology

SpliSense aims to target the root cause of the disease, to overcome specific genetic mutations and restore protein function. Utilizing its transformative RNA-Based technology the company is developing treatments for unmet cystic fibrosis mutations (orphan indication) as well as pulmonary diseases, specifically mucus-obstructive diseases such as COPD, Asthma, PDC, IPF and others.

Product Profile/Pipeline

SpliSense most advanced program, SPL84-23 focus on CFTR splicing mutation 3849, is expected to enter phase 1-2 later this year and will be followed by an additional CF program (W1282X mutation) in early 2023, with an additional larger, non-orphan pulmonary programs for muco- obstructive diseases targeted for IND late 2023.

What's Next?

clinical studies

Questions for category: **Pharma**

- **Main Service:**

Treatment of unmet indications which are orphan

- **Key Strengths:**

Robust understanding of genetic pulmonary diseases and ASOs technology

- **Experience with Israeli Companies:**

CROs – animals models

- **Key Markets:**

EU, US and Asia

