Small Molecules for Treating Cystic Fibrosis

From the Laboratories of Nael McCarty, Ph.D. Dept. of Pediatrics, Emory
Hanoch Senderowitz, Ph.D., Bar Ilan University

Cliff Michaels, Senior Licensing Associate
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Cystic Fibrosis

- Rare autosomal recessive genetic disorder caused by mutation in the CFTR

- Leads to thick viscous mucus in the lungs & repeated respiratory infections
- 30,000 cases in the US and approximately 1,000 new annually
Treating CF Symptoms

Traditional treatments –

1) Chest physiotherapy – Percussive Therapy, Ventilation

2) Easing breathing – Pulmozyme®, Hypertonic Saline

3) Treating infections - TOBI®

Do not treat the cause of CF, only address the symptoms
Therapies

**Kalydeco®** – Only drug that improves CFTR function
- Approximately $300,000/year per patient
- $464M in product revenue in 2014
- Works on specific CFTR mutation; 4-5% of cases

**Next Generation** – Lumacaftor (Vertex)
- CFTR ∆F508 mutation; 60% of cases
- Small but not significant improvement in Phase II

**Unmet Need** - No drug that improves CFTR function & cause of CF for a majority of patients
Our Technology

OSSK-2 & OSSK-3 – Our new small molecule drugs that potentiate the ΔF508 CFTR

1. Known Compound that binds CFTR
2. Virtual screen of over 7M compounds
3. ID best hits and synthesized compounds for testing
4. Tested compound on WT & mutant CFTRs
Testing for Activity

OSSK-2 & OSSK-3 improve function of the ∆F508 CFTR
Value proposition

- CF is an orphan condition with a large unmet need
- New compounds that potentiate the CFTR
- Early but promising proof of concept data
- CFTR a target for other diseases – COPD, diarrhea

Next Steps – Additional validation & POC data

IP Status – Provisional patent application filed

Commercialization – Actively seeking entrepreneur & funding
Thank you!