

Mpex Pharmaceuticals, Inc.

2010

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Mpex Pharmaceuticals Overview

- Aeroquin (proprietary aerosol formulation of levofloxacin) a reduced risk clinical program; 100% of rights retained
 - Compelling results from Phase 2b trial in Cystic Fibrosis
 - Preparing to initiate Phase 3 trial in Cystic Fibrosis
 - Market opportunity for Aeroquin forecasted to ~\$350 million/year by a globally recognized consulting firm
- Separate broad technology platform focused on efflux pump inhibitors (EPIs) for overcoming bacterial resistance
 - Collaboration with GSK fully funds this program
- Experienced management team
- Strong IP/exclusivity protection
- < 40 employee private company with supportive VC investors
- Well-funded; completed a \$40 million series D in early 2009

Important pharmacological and physical properties of new aerosol antibiotics for lung infections

- Broad Spectrum Activity: Cover all relevant bacterial pathogens found in the diseased lung
- Good PK/PD Profile: Achieve sufficient lung levels to not only have maximum killing but also to reduce the potential to cause resistance
- Activity in Lung Environments: Retain activity in biofilms and anaerobic environments and not be deactivated in sputum
- Compliance: Short nebulizer delivery time, infrequent dosing and portability of device
- Safety and Tolerability: Low systemic exposure and side effects

Aeroquin is well positioned to satisfy all of these requirements

Aeroquin is a proprietary formulation of levofloxacin developed for aerosol use



- Levofloxacin's combination of potency, spectrum, solubility, and physico-chemical properties make it an ideal antibiotic for aerosol therapy
- Novel formulation masks bitter taste associated with inhaled fluoroquinolones
- Specifically formulated for use with PARI eFlow high efficiency nebulizer
- Provides high C_{max} and increases AUC in the lung (to improve efficacy)
- Concentrated solution enables 4-6 minute administration time (to improve compliance)
- Manufactured in ready to use blow-fill-seal polyethylene ampules



Aeroquin Intellectual Property

- Patent applications filed to cover multiple aspects of Aeroquin are expected to provide patent exclusivity to 2026
- Exclusive license in place to use patented Pari eFlow nebulizer with Aeroquin for relevant indications
 - Drug/Device specific co-labeling – Aeroquin will only be approved for use with branded eFlow device which provides additional regulatory protections
- Orphan Drug Status granted for Aeroquin in CF
 - 7 year exclusivity in U.S.
 - 10 year exclusivity in Europe

Cystic Fibrosis is a high value indication with significant unmet medical need

- 60,000 patients in the U.S. and E.U.
- A combination of new drug therapies including inhaled antibiotics have been shown to significantly improve lifespan for CF patients
- Patients are chronically infected with bacterial pathogens (50% infected with *Pseudomonas*) which is associated with loss of lung function and subsequent death
- Aerosol antibiotics are the preferred approach to care because very high concentrations of drug can be delivered directly to the lung (site of infection)
 - Not feasible with oral or i.v. antibiotics
 - Max killing at site of infection with reduced resistance development
 - Lower systemic exposure and side effects
- Additional classes of inhaled antibiotics are needed (e.g. quinolones, beta lactams, etc.)

Significant unmet clinical need for new and improved inhaled antibiotic therapies for CF

- Recent market research study conducted by a globally recognized consulting firm has confirmed that there is still significant unmet clinical need for new and improved therapies for the treatment of CF

Key Needs:

- Additional inhaled antibiotic classes; provides additional options for cycling inhaled antibiotic
- New Antibiotics for treating patients intolerant to or experiencing diminished responses to existing therapies
- Broader spectrum to cover non-*Pseudomonas* CF pathogens
- Shorter dosage duration and infrequent dosing schedules to help reduce treatment burden

Goal for inhaled antibiotic treatment of chronic lung infections in CF is 12 months of treatment per year

- Clinical goal of continuous therapy is the reduction of pulmonary exacerbations and the prevention of the resulting decline in lung function
- In order to best achieve 12 months of inhaled antibiotic treatment, different classes of antibiotic should be cycled
 - e.g. aminoglycosides, beta lactams, fluoroquinolones
- Cycling different classes of antibiotics is expected to result in reduced resistance development, reduced class related side effects, and improved long term efficacy
 - These beneficial effects are not likely to be achieved by cycling different antibiotics of the same class (e.g. TOBI & Amikacin) or different formulations of the same antibiotic (e.g. TOBI & DPI TOBI)

Aeroquin's Successful Phase 2 CF Trial

Objective:

- To evaluate the efficacy, safety and tolerability of Aeroquin administered daily for 28 days, compared to placebo in CF patients infected with *Pseudomonas*

Study Design:

- Randomized, double blind, placebo controlled
- 51 clinical sites in the US and Europe
- Enrolled 151 patients
- Patients required to have at least 3 courses of inhaled antibiotics in previous 12 months
- Patients were allowed to remain on other medication shown to improve lung function and reduce exacerbations

Aeroquin's Successful Phase 2 CF Trial (Cont'd)

- Aeroquin met the primary endpoint of reducing bacterial counts of *Pseudomonas aeruginosa* in sputum after 28 days of dosing versus placebo
- Clinically and statistically significant improvements versus placebo were also seen in a number of important clinical endpoints:

Respiratory Function:

- FEV1
- % predicted FEV1
- FEF25-75

Exacerbations:

- Time to need for anti-pseudomonal antibiotics

Aeroquin's Successful Phase 2 CF Trial (Cont'd)

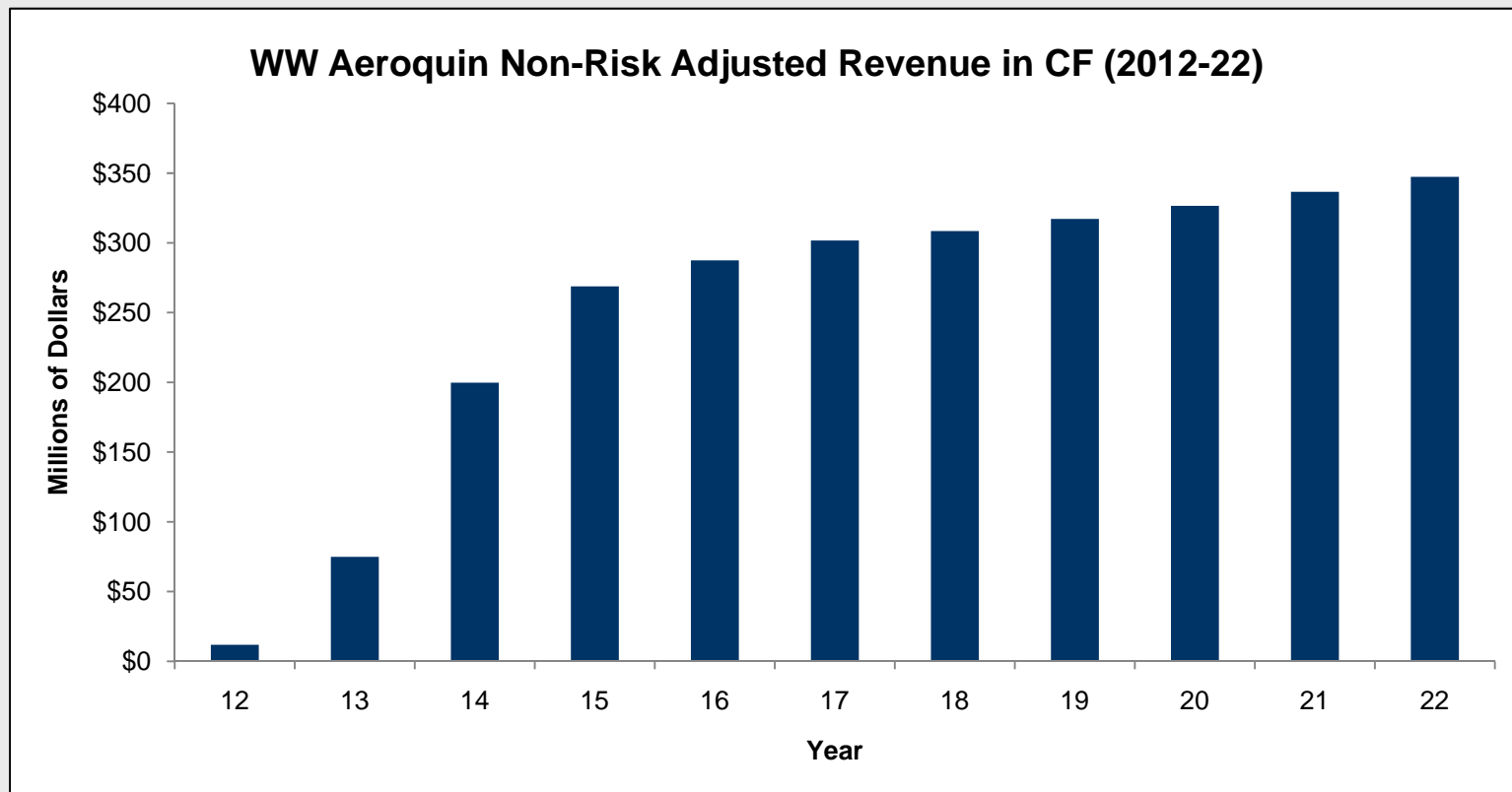
- Both once daily and twice daily dosing of Aeroquin showed activity in this trial, with higher doses showing improved responses
- Aeroquin was well tolerated
- No significant change in antibiotic resistance was observed in this study

Aeroquin was well received by CF clinicians

- A globally recognized consulting firm completed 51 physicians and 16 payor interviews in U.S. & Big 5 E.U.
- Physicians in both the U.S. and EU were excited about the use of Aeroquin as an inhaled maintenance therapy for CF patients with pulmonary infections
- According the consulting firm Aeroquin received unusually high attractiveness ratings from surveyed physicians
 - An average of ~5.9 out of 7 on an attractiveness scale
- Key benefits of Aeroquin are the reduction of exacerbations, dosing and treatment duration, and broad spectrum of activity

Globally recognized consulting firm forecasts revenue of ~\$350 million for Aeroquin in CF

- Completed Q3, 2009; U.S. & Big 5 E.U., 51 physicians & 16 payor interviews

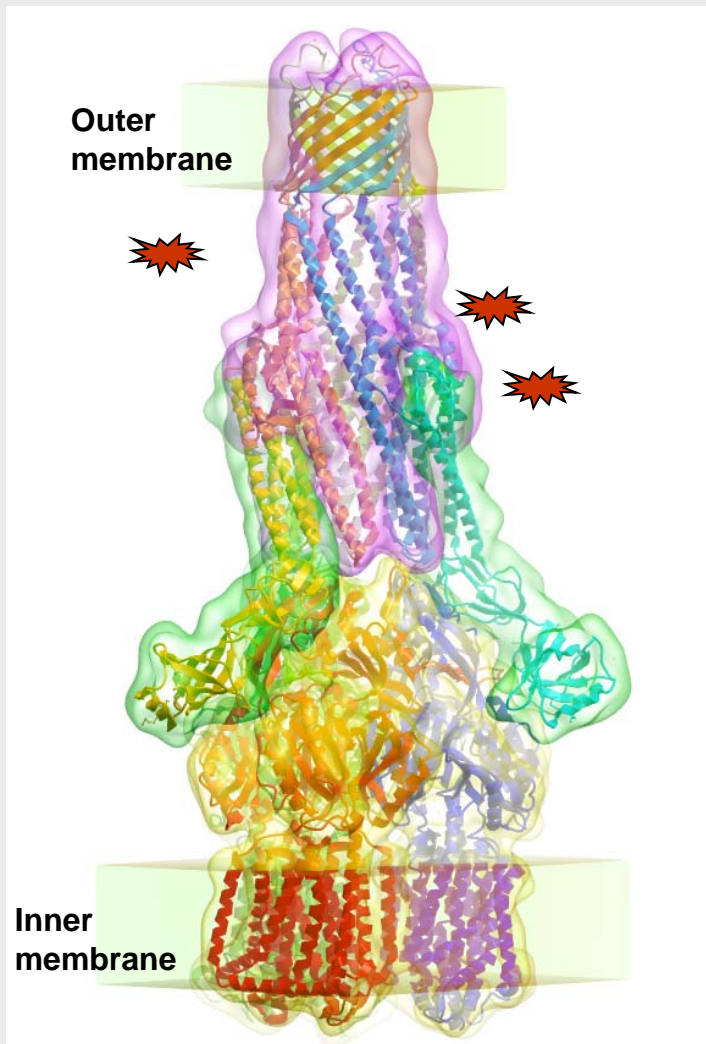


* Assumes ~45% share of CF patients are treated with approximately 3-4 courses of Aeroquin per year

Aeroquin Summary

- Potential best in class in CF
- Addresses high unmet medical needs
- Reduced risk development program
- Statistically and clinically significant CF Phase 2b data
- Phase 3 start mid 2010
- Strong IP/exclusivity protection
- Global product rights retained

Overcoming efflux pumps benefits many classes of antibiotics



➤ **Efflux Pumps**

- Reduce intracellular concentrations of antibiotics
- Responsible for multi-drug resistance in gram-negative pathogens
- Largely responsible for inability to develop new classes of antimicrobial agents

➤ **Efflux Pump Inhibitors (EPIs) as Drugs**

- Reverses efflux-mediated multi-drug resistance and increases antibiotic susceptibility
- Suppresses the emergence of resistance
- Enhances antibiotic effectiveness
- Can improve the “developability” of novel classes of antimicrobial agents



Mpex partnered this program with GSK

- Collaboration on combinations with both existing commercial antibiotics and novel GSK antibiotics
- \$15 million upfront in cash and equity funds lead optimization activities
- Early milestones cover all costs of Mpex taking compounds through Phase 2 proof of principle
- Up to 7 different products planned
- Milestones of \$200 – 250 million per product; \$1.5 billion in total
- Attractive royalty rates

The Mpex management team is highly experienced in all aspects of drug development

- **Dan Burgess – President and CEO, Director**
 - COO / CFO, Hollis-Eden Pharmaceuticals
- **Mark Wiggins – Chief Business Officer**
 - EVP Global Business Development, Biogen Idec
- **Dr. Mike Dudley – SVP of R&D and Chief Scientific Officer**
 - VP Preclinical and Clinical Sciences, Diversa Corporation
- **Dr. Jeff Loutit – Chief Medical Officer**
 - VP Clinical Science, InterMune, Inc.
- **Liz Morgan – VP Clinical Operations**
 - VP Clinical Operations, Nereus Pharmaceuticals
- **Dana Krzyston, CPA – VP Finance & Admin**
 - Controller, Tensys Medical Inc.
- **Dr. Olga Lomovskaya – VP Biology**
 - Associate Director of Biology, Essential Therapeutics
- **Dr. Scott Hecker – VP Chemistry**
 - VP Chemistry, Metabasis

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