Marc O. Salzberg, MD
President & CEO

AIRWAY THERAPEUTICS

innovative lung solutions

Jan S. Rosenbaum, Ph.D.
Chief Scientific Officer

AIRWAY THERAPEUTICS

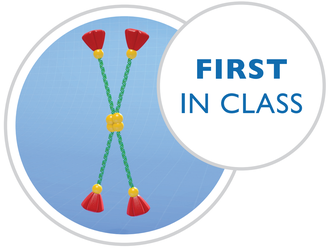
- Spin-out of **Cincinnati Children's** Hospital Medical Center (CCHMC)
- More than **$12 million raised to date.**
- **Mission:** to develop interventions for the prevention and treatment of major lung diseases
- **Initial focus: developing AT-100** (rhSP-D) for prevention of bronchopulmonary dysplasia (BPD)

WHAT IS BPD?

- **Most common studied complication of prematurity**; no effective treatment or preventive therapy is available
- **160,000 babies** at high risk to develop BPD each year in the US and Europe, and **1.2 million babies** in Asia
- **Definition:** Oxygen and ventilator support at 36 weeks gestational age
- Ventilation and oxygenation leads to inflammation, infection, decreased alveolar septation and **ultimately arrested lung development**.

VALUE PROPOSITION

A large percentage of surviving babies affected by BPD will suffer from lifelong chronic symptoms. **AT-100 is the first drug to prevent BPD.**



AT-100 preventative therapy has a mechanism of action that **mitigates risk by targeting multiple clinical endpoints**.

With **anti-infective and anti-inflammatory properties,** it reduces time on a ventilator, reduces time in the ICU, and improves survival.

Orphan designation is obtained, granting an additional 12/7 years market exclusivity (EU/US).

BPD patent is valid until 2032.

AT-100 PIPELINE

Potential for treatment of other lung conditions:

- **Asthma**
- **CF**
- **RSV**
- **COPD**

SALES POTENTIAL

Upon approval in the US & Europe, Airway estimates the peak year sales potential for prevention of BPD to be **at least $1B.**

AT-100 TODAY

- Manufacturing (CMC) **development ongoing** with a proprietary expression system
- **Successful Pre-IND meetings** with FDA and Scientific Advice with EMA in 2015
  - Preclinical and clinical development programs endorsed
  - Program endorsed by FDA and by EU regulatory authorities experienced in neonatology and lung diseases

PRIMED FOR CLINICAL SUCCESS

- **High confidence** in the right target for BPD
  - Strong link between role of SP-D, inflammation and BPD
  - Strong link between role of SP-D in surfactant homeostasis and critical need for surfactant in proper lung development
  - Clinical evidence that babies who develop BPD have lower levels of appropriately functioning SP-D
- Clinical trial design provides **multiple options to show benefits.**
  - Strong link between role of SP-D in inflammation and infection, and the incidence of both conditions in very pre-term infants
- SP-D is an innate protein; **safety profile of SP-D is well understood.**
  - Patients with certain lung diseases have elevated levels with no adverse effect.
  - These levels provide benchmarks for safety index.