

## Corporate Overview

Sequella is a private, clinical-stage pharmaceutical company that addresses the challenge of antibiotic-resistant bacterial diseases by discovery, development, and commercialization of first-in-class antibiotics with novel mechanisms of action. Our lead drug candidate identified from our current >150,000 proprietary compound library, SQ109, is in Phase 2 clinical trials for treatment of both tuberculosis (TB) and gastritis (*Helicobacter pylori* infections), diseases complicated by growing resistance to existing drugs. In 2013, we acquired a new clinical-stage (Phase 2) oxazolidinone, sutezolid, for treatment of TB and other resistant bacterial diseases. In addition to new pathogen-specific antibiotics, we also have a vigorous research program to discover novel broad spectrum antibiotics that target the enzyme translocase-1, an essential enzyme in all bacteria.

Our product portfolio addresses global health threats with significant market opportunity in disease areas of known or suspected infectious etiology:

- TB (*Mycobacterium tuberculosis*)
- Duodenal ulcers (*H. pylori*)
- Gastric carcinomas (*H. pylori*)
- CDI (*Clostridium difficile*)
- Crohn's Disease (*M. avium paratuberculosis*)

## Business Model

Leverage core competencies in chemistry, microbiology, molecular biology, infectious diseases, and anti-infective R&D to discover, develop, and commercialize promising new antibiotics with excellent market potential.

## Target Markets

Infectious diseases affect millions of people worldwide. Increasing bacterial drug resistance is threatening to return the world to pre-antibiotic times when epidemics and pandemics decimated whole populations. New and novel antibiotics are critically needed to regain control of these diseases.

- Annual worldwide commercial market for a new MDR-TB drug is estimated at \$500 million, with an annual U.S. and E.U. market of \$400 million for treatment of uncomplicated and latent TB.
- The U.S. market for a new and better drug to eradicate *H. pylori* is estimated to be >\$1 billion annually. *H. pylori* is the etiological agent of more than 90% of duodenal ulcers, 80% of gastric ulcers, and most gastric carcinomas.
- The U.S. market for a new drug to eradicate *C. difficile* is estimated to be \$200-\$300M. Each year in the U.S., >400,000 cases result in \$4 billion in excess healthcare costs.
- The U.S. market for a drug to eliminate one cause of Crohn's Disease, *M. avium paratuberculosis*, is estimated at >\$500M. Crohn's Disease patients number 600,000 in the U.S.



## Sequella Background

<b>Incorporated:</b>	1997, Delaware corporation
<b>Address:</b>	9610 Medical Center Drive, Suite 200 Rockville, MD 20850 USA
<b>Therapeutic Focus:</b>	Antibiotic-resistant Infectious diseases
<b>IP Portfolio:</b>	92 patents issued and 29 pending in U.S., E.U., Japan, and selected countries of the ROW
<b>Clinical-Stage Drug:</b>	SQ109 (Phase 2)
<b>Employees:</b>	12 FTE
<b>Financing To Date:</b>	\$78 million: \$28 million in common equity, and \$50 million in grants and in-kind services.
<b>Website:</b>	<a href="http://www.sequella.com">www.sequella.com</a>

## Management

**Carol A. Nancy, Ph.D., CEO, Board Chair and Founder.** Prior to founding Sequella, Dr. Nancy was Chief Scientific Officer at Anergen, acquired by Corixa (CRXA-NASDAQ); Executive Vice President and Chief Scientific Officer at EntreMed (ENMD-NASDAQ); President of the American Society for Microbiology; and career scientist and science manager, Walter Reed Army Institute of Research.

**Lisa Beth Ferstenberg, MD, Medical Director.** Prior to joining Sequella, Dr. Ferstenberg was Chief Medical Officer (CMO) at Accelovance, Inc., Chief New Product Officer at Aeras Global TB Fdn, Founder, and CEO of Collective Therapeutics, and CMO of StemCo Biomedical, Inc. She is on the faculty of the Johns Hopkins Carey Business School.

**Leo Einck, Ph.D., CSO and Founder.** Prior to joining Sequella, Dr. Einck was Vice President for Research Operations, EntreMed (ENMD-NASDAQ); Director of Operations, HEM (now Hemispherix Biopharma, Inc, HEB-AMEX); and Scientist at the National Institutes of Health.

**Alan Klein, MBA, EVP Corporate Development.** Prior to joining Sequella, Mr. Klein managed over \$900 million in transactions, was VP Business Development at GeneLogic (GLGC-NASDAQ); Senior Director, Business Development at Curagen (CRGNNASDAQ); Senior Director within the Anti - Infectives, Allergy/Respiratory, and Immunology Business Units at Quintiles Transnational.

## Key Value Drivers

### Antibiotic Pipeline:

#### Lead diamine small molecule antibiotic, SQ109

- Discovered at Sequella from proprietary 63,238 small molecule library: novel mechanism of action, extraordinarily low induced drug resistance rate;
- Phase 2 human clinical trials for TB in 7 sites in Africa ongoing, funded in part by a grant from the E.U.;
- Licensed to Infectex for completion of TB clinical trials and commercialization in Russia and CIS, Phase 3 in MDR-TB ongoing (November 2012)
- IND for *H. pylori* gastritis indication 2010; Phase 2 study in Texas, USA demonstrated significant efficacy signal (ongoing).
- Has both Fast Track status at the FDA and Orphan Drug status at the FDA and EMEA for TB.

#### Lead oxazolidinone small molecule antibiotic, sutezolid

- In-licensed from Pfizer July 2013
- Has activity on *M. tuberculosis* and Gram positive bacteria, including methicillin-resistant *Staphylococcus aureus* (MRSA) and vancomycin-resistant *Enterococcus* (VRE)
- Successfully completed Phase 2a Early Bactericidal Activity trial in TB patients
- Has Orphan Drug status at the FDA and EMEA for TB.

#### Lead dipiperidine small molecule antibiotic, SQ609

- Discovered at Sequella from proprietary 35,000 library
- Acts specifically on *M. tuberculosis*: new mechanism of action
- Ready for IND-directed preclinical safety studies

#### Lead Translocase-1 inhibitor derived from capuramycin, SQ641

- Compound library in-licensed from Daiichi-Sankyo
- Lead compound identified from 7000 capuramycin analogs
- In formulation development for treatment of gastrointestinal infections caused by *Clostridium difficile* and *M. avium paratuberculosis*

### Additional Assets:

To complement its new antibiotics, and to manage their product lifecycle, Sequella invented and is developing two additional devices:

#### Event Marker System (EMS)

- Wristwatch device to transdermally detect a fluorescent tracer (event marker) incorporated into a medication ingested by a patient to ensure that the medication is taken appropriately;
- Ingestion signal sent electronically and wirelessly to healthcare provider;
- Improper or intermittent use of an antibiotic facilitates development of bacterial resistance to drugs;
- US patent issued in 2004: transdermal detection of a labeled pill, independent of label. Priority Date December 1998.

#### B-SMART™ Antibiotic Sensitivity Assay

- Novel technology to use sensitive nucleic acid testing to detect phenotypic drug resistance in bacteria
- Assess resistance to any drug, including those for which the resistance mechanism/gene is unknown;
- First test kit in development for MDR-TB, license to LabCorp for sales in U.S.
- U.S. patent issued in 2011

#### FDA Priority Review Voucher (PRV)

- FDA awards PRV to sponsor of newly approved drug or vaccine targeting neglected tropical diseases, including TB.
- PRV entitles bearer to a priority FDA review for a future new drug application, reducing FDA approval time by 4-12 months. Economists at Duke University calculate value to Pharma at \$100-\$300 million.
- Two Sequella drugs in development qualify for a PRV.

## Plans to Reach a Major Value Inflection Point

Recent events clarified the path to create the highest enterprise value for Sequella: our new TB drug registered for marketing and sales. On December 28, 2012, the FDA announced accelerated approval of J&J/Janssen's new MDR-TB drug, bedaquiline, after a Phase 2b clinical study. This approval is highly significant: bedaquiline is the first new TB drug approved in 40 years, so we now know exactly the FDA regulatory pathway for MDR-TB drugs; accelerated approval sets the parameters for new antibiotics addressing drug resistant infections; and Cowen & Co analyst report on bedaquiline documents the market for a new MDR-TB drug, 5% of worldwide TB, at \$300-500 million, substantiating Sequella's internal market assessment.

SQ109 is currently in a registration Phase 2/3 clinical trial in Russia for MDR-TB with our corporate partner, Infectex, and a Phase 2b study in Africa for drug sensitive TB. Sutezolid has successfully completed its Phase 2a study in TB patients. Sequella can now plan a global Phase 2b clinical trial with sufficient geographic diversity and appropriate patient populations to statistically validate efficacy of SQ109 and sutezolid in MDR-TB and use this trial to file two NDA for accelerated approval in the U.S. and E.U. in 2016/2017 timeframe.

## Capital Raise in 2013

Sequella intends to raise \$38 million in a mezzanine financing in 2013. This capital raise will support company infrastructure for 3-4 years and fund the following high-value activities:

Global SQ109 and sutezolid MDR-TB clinical trial for accelerated approval in U.S. and E.U. and two awarded PRV; SQ109 Phase 2b clinical trial in *H. pylori* infections to achieve a corporate partner; accelerate development of pipeline drugs SQ609 and SQ641 to the clinic; and general corporate purposes.