## 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 clinical stage drugs are SQ109 and sutezolid, and both are being independently advanced for the treatment of multi-drug resistant tuberculosis (MDR-TB). SQ109 completed a successful registration trial in Russia in 2016: an application for commercial approval will be filed in 2H 2017 and Sequella will receive product royalties in 2018. Both SQ109 and sutezolid are ready to enter pivotal clinical trials in the U.S./E.U. that, if successful, will lead to NDA filing for accelerated registration in both territories. Registration in the U.S. of either drug for MDR-TB will provide Sequella with a priority review voucher that has a market value of up to $350M. SQ109 has been evaluated in a clinical trial for treatment of gastritis (*Helicobacter pylori* infections). We have a chemical library of small molecule compounds that we can screen on other drug resistant bacteria.

Our product portfolio currently 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 control these diseases.

- Annual commercial market for a new MDR-TB drug in EME and BRICS is estimated at $824 million, with an annual market of $1.1 billion for treatment of MDR-TB, drug-sensitive TB and latent TB.

- The U.S. market for a new and better drug to eradicate *H. pylori* is estimated to be $3 billion annually. *H. pylori* is the etiological agent of more than 90% of duodenal ulcers, 80% of gastric ulcers, and most gastric carcinomas.

- The global market for a new drug to eradicate *C. difficile* is estimated to reach $2.4 billion by 2024. Each year in the U.S., >400,000 cases result in $4 billion in excess healthcare costs.

- The U.S. market for drugs to eliminate a cause of Crohn's Disease, *M. avium paratuberculosis,* is estimated at >$500M. U.S. Crohn's Disease patients number 600,000.

SEQUELLA Incorporated

### Sequella Background

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| **Incorporated:** | 1997, Delaware corporation |
| **Address:** | 9610 Medical Center Drive, St 200<br>Rockville, MD 20850 USA |
| **Therapeutic Focus:** | Drug-resistant bacterial infections |
| **IP Portfolio:** | 92 patents issued and 27 pending in U.S., E.U., Japan, and selected countries of the ROW |
| **Clinical-Stage Drugs:** | SQ109 (Ph 2b/3), sutezolid (Ph 2) |
| **Other Assets:** | Potential PRV worth up to $350M (average sale price $186M) |
| **Financing To Date:** | $95 million: $35 million in common equity sales, and $60 million in grants and in-kind services. |
| **Website:** | www.sequella.com |

### Management

**Carol A. Nacy, PhD, Chief Executive Officer, Founder, and Board Chair.** Prior to founding Sequella, Dr. Nacy was CSO at Anergen (ANRG), acquired by Corixa (CRXA); EVP/CSO at EntreMed (ENMD); President of the Am. Soc. for Microbiology; and scientist manager, Walter Reed Army Inst. of Research. She is on the faculty of George Washington University.

**Mark Rampy, Ph.D., Chief Business Officer.** Prior to Sequella, Dr. Rampy was CEO/Founder of Theraly Pharmaceuticals, CEO of CohBar, CBO/Founder of CoGenesys acquired by Teva (TEVA), VP/Head of BD at Teva, VP/Head of BD at Human Genome Sci Inc.(HGSI)

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

**David Mc Neeley, MD, MPH, Global Medical Director.** Prior to joining Sequella, Dr, Mc Neley was Global Program Medical Dir. at Novartis (NVS), Dir, Global Clin Dev at Tibotec (JNJ), Assoc Dir, Global Clin Res at J&JRD (JNJ)

**Leo Einck, PhD, Founder, and Director.** Prior to joining Sequella, Dr. Einck was VP Research Operations, EntreMed (ENMD); Director of Operations, HEM (now Hemispherix Biopharma, Inc, HEB-AMEX); and Scientist at NIH.

## Key Value Drivers

**Antibiotic Pipeline**
Sequella in-house drug discovery program identified antibiotics with activity against a variety of important bacterial pathogens. The Sequella product pipeline includes:

**SQ109 diamine small molecule antibiotic,**
- 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 funded in part by a grant from the EU (results in 2016);
- Licensed to Infectex for completion of TB clinical trials and commercialization in Russia and CIS. Sequella receives milestones and double digit royalties on sales.
- MDR-TB Russia registration trial complete**.**
- IND for *H. pylori* gastritis; Proof-of-Principle study in Texas, USA demonstrated significant efficacy signal.
- Has both Fast Track status at the FDA and Orphan Drug status at the FDA and EMEA for TB.

**Sutezolid oxazoldinone small molecule antibiotic**
- Licensed from Pfizer in mid-2013;
- Phase 1 study of 600mg BID for 28 days demonstrated that usual class safety issues were not induced;
- Phase 2a human clinical trial for TB in 2 sites in South Africa complete; good early bactericidal activity
- Has Orphan Drug status at the FDA and EMEA for TB.

**SQ641 Translocase-1 inhibitor**
- Compound library in-licensed from Daiichi-Sankyo;
- Lead compound identified from 7000 compound library;
- Excellent activity in animal models; stays entirely in GI tract (no systemic exposure); very specific for *C. difficile*.
- In IND-directed preclinical development for prophylaxis to avoid GI infections caused by *C. difficile*.

**SQ609 dipiperidine small molecule antibiotic**
- Discovered at Sequella from 35,000 compound library;
- Active on *M. tuberculosis*: new mechanism of action;
- Acts additionally to inhibit inflammation into TB lesions;
- Ready for IND-directed preclinical safety studies;
- Partnership with National University of Singapore to bring the drug into first-in-man studies.

**Additional Assets:**

**Chemical Compound Library** (>200,000 compounds)
In addition to these drug candidates, Sequella will screen its large proprietary new small molecule chemical library for activity against other important drug-resistant bacterial pathogens.

**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 priority FDA review for a future New Drug Application, reducing FDA approval time by 4-6 months.
- PRV's can be sold to other companies;
- Economists at Duke University calculate value to Pharma at $100-$300 million.
- Three Sequella drugs in development could qualify for a PRV (SQ109, SQ609, sutezolid).

## Plans to Reach a Major Value Inflection Point

Recent events clarified the development path to create the highest enterprise value for Sequella. 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 the exact 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 has completed a pivotal trial in Russia for MDR-TB with our corporate partner, Infectex, and a Phase 2b study in Africa for drug sensitive TB.
- Sutezolid, acquired from Pfizer in 2013, successfully completed its Phase 2a study in TB in Africa.
- Sequella has clinical development plans for both SQ109 and sutezolid and will be ready to start a global Phase 2b clinical trial within a year. The Phase 2b trial plan has sufficient geographic diversity and appropriate patient populations to statistically validate efficacy of SQ109 and sutezolid in MDR-TB and use this trial to file an NDA for accelerated approval in the U.S. and E.U. within several years.
- Registration of SQ109 and/or sutezolid in the U.S. for treatment of MDR-TB will qualify Sequella for receipt of a PRV for each drug registered. SQ109 PRV will be a financial asset of the company; sutezolid PRV is promised to Pfizer, a condition of the license agreement.