

Corporate Overview

Sequella is a private, clinical-stage pharmaceutical company that discovers, develops, and intends to commercialize novel first-in-class antibiotics for treatment of serious life-threatening infectious diseases. We initially created a 65,000 small molecule compound library from which we identified a novel antibiotic now in clinical development for treatment of tuberculosis (TB), SQ109. Following initiation of SQ109 human clinical trials in 2006, we expanded our small molecule library to >150,000 compounds and broadened our therapeutic focus to include other bacteria and fungi based on biologic activity of our proprietary library. 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 & Gastric carcinomas (*H. pylori*)**
- **Crohn's Disease (*M. avium paratuberculosis*)**
- **Pneumonias (*M. avium avium*)**
- ***Clostridium difficile* infections**

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.

Targeted Markets

Infectious diseases affect millions of people worldwide, with estimated U.S. annual treatment costs of \$20 billion.

- Annual worldwide market for a new TB drug is estimated at \$564 million, with an annual U.S. and E.U. market of \$400 million for treatment of TB and latent TB.
- The U.S. market for a new antifungal is estimated at \$350-\$400M. The addressable market is 575,000 patients in U.S., with a total worldwide market opportunity of \$8.7B.
- 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.
- 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 costs.

Sequella Background

Incorporated: 1997, Delaware corporation

Therapeutic Focus: Infectious diseases

IP Portfolio: 122 patents issued and pending in U.S., E.U., Japan, and selected countries of the ROW

Clinical - Stage Drug Candidate: SQ-109 (Phase 2)

Employees: 17 full-time

Financing To Date: \$74 million: \$24 in common dilutive funding and \$49 million in grants and in-kind services.

Website: www.sequella.com

Key Management

Carol A. Nancy, Ph.D., CEO, Chair of the Board and Founder.

Prior to joining Sequella, Dr. Nancy was Executive Vice President and Chief Scientific Officer at EntreMed (ENMD-NASDAQ); Former Chief Scientific Officer at Anergen, acquired by Corixa (CRXA-NASDAQ); Former President of the American Society for Microbiology; and Former career scientist and science manager, Walter Reed Army Institute of Research.

Marty Zug, MBA, CFO. Prior to joining Sequella, Mr. Zug was Vice President of the Washington Redskins where he managed three business units comprising over \$42 million in annual revenue; former Director of Financial Projects for Snyder Communications; and a former investment banker at International Finance Corp.

Gary Horwith, M.D., Chief Medical Officer. Prior to joining Sequella, Dr. Horwith was Vice President Clinical Research and Medical Affairs, NABI Biopharmaceuticals (NABI-NASDAQ); former Vice President of Clinical Research and Drug Regulatory Affairs at Genetic Therapy, Inc., a Novartis company (NYSE-NVS); and former Director of Clinical Research at The Liposome Company.

Leo Einck, Ph.D., CSO and Founder. Prior to joining Sequella, Dr. Einck was former Vice President for Research Operations, EntreMed (ENMD-NASDAQ); former Director of Operations, HEM (now Hemispherix Biopharma, Inc, HEB-AMEX); and Former 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); Former Senior Director, Business Development at Curagen (CRGN-NASDAQ); Former Senior Director within the Anti-Infectives, Allergy/Respiratory, and Immunology Business Units at Quintiles Transnational.

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:

- **Lead diamine antibiotic SQ109**
 - Discovered at Sequella from a 63,000 small molecule library
 - Phase 2a human clinical trials for TB in Africa (fully funded by a grant from the E.U.) completed enrollment
 - IND for *H. pylori* duodenal ulcer indication filed in 2010; Phase 2 studies begun in U.S. in Q4 2011
- **Lead dipiperidine antibiotic SQ609**
 - Discovered at Sequella from a 50,000 small molecule library
 - SQ609 (TB indication) is ready for IND-directed preclinical safety, pharmacology, and toxicology studies
- **Translocase-1 inhibitors derived from capuramycin**
 - Series in-licensed from Daiichi-Sankyo
 - Lead compound identified from 7000 semi-synthetic analogues
 - SQ641 is in lead optimization for infections caused by *Clostridium difficile*

Sequella lead drug candidate in Phase 2 clinical trials, SQ109, has both Fast Track status at FDA and Orphan Drug status at FDA and EMA.

Event Marker System (EMS)

- Wristwatch device that transdermally detects a fluorescent tracer (the event marker) incorporated in medication ingested by a patient
- Sequella US patent #6,663,846B1 issued in 2004 (non-U.S. applications pending): claims transdermal detection of a labeled pill, independent of the label. Priority Date is December 1998.
- Sequella believes this is a key enabling patent for any technology used to transdermally detect and monitor drug ingestion compliance using any event marker.

FDA Priority Review Voucher (PRV)

- FDA awards PRV to the sponsor of newly approved drug/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.

Infectious Disease Target	Research	Formulation	Pre-clinical	IND	Phase 1	Phase 2	Phase 3	NDA
Tuberculosis	SQ109							
	SQ609							
	SQ641							
<i>H. pylori</i> Infection	SQ109							
<i>H. pylori</i> - related Carcinomas	SQ109							
<i>C. difficile</i> Infections	SQ641							
Non - TB Pneumonias	SQ641							
<i>C. difficile</i> Infections	SQ109*							

*SQ109 based ethylenediamine analogs