



## **Eloxx Pharmaceuticals Acquires Zikani Therapeutics**

April 1, 2021

*Combined Company to be Leader in Ribosomal RNA-Targeted Genetic Therapy Bringing Together Complementary Platforms*

*Maximizes Potential for ELX-02 for Cystic Fibrosis in Phase 2 Development*

*Adds Preclinical Stage Pipeline in Rare Diseases and Oncology Targeting RNA and Ribosomal Mutations*

*Expects to File IND for First Oral Drug to Treat Patients with Recessive Dystrophic and Junctional Epidermolysis Bullosa (RDEB and JEB)*

*Sumit Aggarwal, Zikani President and CEO, to Lead the Combined Company*

*Eloxx to Issue Approximately 7.6 Million Shares to Zikani Stockholders*

*Company to Host Investor Call at 8:30 a.m. ET, April 1*

WALTHAM, Mass. and WATERTOWN, Mass., April 01, 2021 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX) today announced it has acquired Zikani Therapeutics, Inc. in an all-stock transaction, with the potential to create a leader in ribosomal RNA-targeted therapies for treatment of rare diseases and oncology. Sumit Aggarwal, previously the President and Chief Executive Officer of Zikani, has been named President and Chief Executive Officer of Eloxx, and Vijay Modur, M.D., Ph.D., who was Zikani's Chief Scientific and Medical Officer, has been named Eloxx's Head of Research and Development.

"With the strength of our ELX-02 program for cystic fibrosis, this acquisition provides us with the opportunity to amplify the potential of our innovative science by developing a new class of therapies to treat diseases with limited to no treatment options under the stewardship of leaders with a proven ability to translate technology into treatments for patients," said Tomer Kariv, Eloxx Chairman.

"We are excited about the potential of ELX-02 and combining the companies opens the door to build a leadership position in genetic therapy by rapidly developing treatments that can restore functional proteins in patients with nonsense mutations in their RNA," said Aggarwal. "The combined capabilities of Eloxx and Zikani in chemistry, biology, regulatory and drug development, including Zikani's TURBO-ZM™ synthetic chemistry platform for designing macrolide-based Ribosome Modulating Agents (RMAs), along with a committed leadership team and talented employees, will further accelerate our ability to impact the lives of those who have rare diseases with the type of urgency and novel thinking that they deserve," added Aggarwal.

ELX-02 is currently in Phase 2 clinical trials in Cystic Fibrosis (CF) patients affected by nonsense mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. The investigational therapy has shown strong activity across a full range of mutations in CF preclinical models. In Phase 1 testing, ELX-02 was generally well-tolerated and demonstrated high bioavailability with consistent pharmacokinetics across both single and multiple-dose studies.

"The Phase 2 trials are designed to validate the safety of ELX-02 and assess its biological activity. We look forward to completing enrollment in the first four treatment arms by mid-year and reporting data from these treatment arms in the second half of this year," said Dr. Modur.

In addition to CF, the company plans to file an IND in 2022 for what could potentially become the first oral therapy for protein restoration for patients with nonsense mutations in Recessive Dystrophic Epidermolysis Bullosa (RDEB) and Junctional Epidermolysis Bullosa (JEB). RDEB is an incurable, extremely painful and often fatal skin blistering condition caused by a lack of collagen type VII that is estimated to affect more than 3,000 people worldwide. JEB is the most severe form of EB, with most patients dying in infancy.

By extending the application of ribosomal RNA modulation to the readthrough of nonsense mutations in tumor suppressor genes, the company is also rapidly advancing preclinical research for familial adenomatous polyposis (FAP), an inherited pre-cancerous colorectal disease frequently caused by nonsense mutations in the adenomatous polyposis coli (APC) gene.

Nonsense mutations cause approximately 10-12 percent of rare inherited diseases. ELX-02 along with the TURBO-ZM™ library of compounds are anticipated to significantly expand to include the treatment of many other rare diseases and certain cancers.

### **Acquisition Terms**

Under the terms of the merger agreement, stockholders of Zikani received approximately 7.6 million Eloxx common shares and own approximately 16 percent of the combined company.

### **Board and Management Changes**

In connection with the acquisition, Silvia Noiman, Ph.D., and Martijn Kleijwegt have stepped down from the Eloxx Board. Alan Walts, Ph.D., and Raj Parekh, Ph.D., who have both served as Zikani directors, were appointed to fulfill the vacancies and serve out the remaining terms of office.

"We're pleased to welcome Sumit and Vijay to the Eloxx leadership team. They demonstrated their ability to transform Zikani by following the science and pursuing the creation of a new class of therapies on behalf of patients with unmet medical need. We want to extend our thanks and appreciation to Dr. Greg Williams for his stewardship of Eloxx and his commitment to advancing the critical work of the company. We are pleased that Greg will

continue to advise Eloxx to facilitate a smooth transition,” said Kariv.

B. Riley served as financial advisor to Zikani.

### **Conference Call Information**

**Date:** Thursday, April 1, 2021

**Time:** 8:30 a.m. ET

**Domestic Dial-in Number:** (866) 913-8546

**International Dial-in Number:** (210) 874-7715

**Conference ID:** 8180169

**Live Webcast:** accessible from the Company's website at [www.eloxxpharma.com](http://www.eloxxpharma.com) under Events and Presentations or by clicking [here](#). A replay of this conference call will be available on the Eloxx and Zikani websites.

### **Leadership Profiles**

#### **Sumit Aggarwal**

Sumit Aggarwal served as Zikani's President and CEO. He has led the transformation of Zikani from an early-stage technology company to a development-stage rare disease and oncology-focused organization. Under Aggarwal's leadership, Zikani has concentrated its focus on demonstrating pre-clinical proof of efficacy across several disease states using its TURBO-ZM™ technology platform.

In his more than 20 years in pharmaceutical and biotechnology commercial operations, investment management and management consulting, Aggarwal has been successful in transforming companies by re-invigorating innovation, growth and profitability, and raising capital for promising technology companies.

Prior to joining Zikani, he reinvigorated growth and profitability at Progenity, raised \$125 million in capital and built a novel drug delivery-based GI pipeline. He also held leadership roles in healthcare and biotechnology at Adage Capital and as an Associate Partner at McKinsey & Company in its healthcare practice.

Aggarwal has an MBA with distinction from the Johnson School, Cornell University, and a Bachelor of Technology with Honors in Chemical Engineering from the Indian Institute of Technology, Kharagpur.

#### **Vijay Modur, M.D., Ph.D.**

Vijay Modur, M.D., Ph.D., served as Zikani's Chief Scientific and Medical Officer and has led the scientific efforts to transform medicines based on ribosomal modulation using Zikani's proprietary TURBO-ZM™ technology platform.

In his more than 20 years in pharmaceutical and diagnostic roles in R&D, he has successfully translated research discovery efforts into products that have impacted medical practice.

Prior to joining Zikani, Dr. Modur led the venglustat rare disease program at Sanofi across multiple rare disease indications into Phase 2 and Phase 3 clinical development along with leading other early development programs. Prior to Sanofi, he held leadership roles in HTG Molecular, Novartis Oncology and Merck Research Labs.

Dr. Modur obtained his MBBS from Karnatak University and his Ph.D. from the University of Utah. He was a resident in Clinical Pathology at Washington University School of Medicine where he also completed his post-doctoral fellowship.

### **About Eloxx Pharmaceuticals**

Eloxx Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company developing novel RNA-modulating drug candidates (designed to be eukaryotic ribosomal selective glycosides) that are formulated to treat rare and ultra-rare premature stop codon diseases. Premature stop codons are point mutations that disrupt protein synthesis from messenger RNA. As a consequence, patients with premature stop codon diseases have reduced or eliminated protein production from the mutation bearing allele accounting for some of the most severe phenotypes in these genetic diseases. These premature stop codons have been identified in over 1,800 rare and ultra-rare diseases. Read-through therapeutic development is focused on extending mRNA half-life and increasing protein synthesis by enabling the cytoplasmic ribosome to read through premature stop codons to produce full-length proteins. Eloxx's lead investigational product candidate, ELX-02, is a small molecule drug candidate designed to restore production of full-length functional proteins. ELX-02 is in the early stages of clinical development focusing on cystic fibrosis. ELX-02 is an investigational drug that has not been approved by any global regulatory body. Eloxx's preclinical candidate pool consists of a library of novel drug candidates designed to be eukaryotic ribosomal selective glycosides identified based on readthrough potential. Eloxx also has preclinical programs focused on kidney diseases including autosomal dominant polycystic kidney disease, as well as rare ocular genetic disorders. Eloxx is headquartered in Waltham, MA, with operations in Rehovot, Israel, and Morristown, NJ. For more information, please visit [www.eloxxpharma.com](http://www.eloxxpharma.com).

### **About Zikani Therapeutics**

Zikani Therapeutics is an emerging leader in the science of ribosome modulation, leveraging its innovative TURBO-ZM™ chemistry technology platform to develop novel Ribosome Modulating Agents (RMAs) as therapeutics for people with limited treatment options. Zikani's TURBO-ZM™ platform allows rapid synthesis of novel compounds that can be optimized to modulate the ribosome in a disease specific manner. As the company evolves its focus from early-stage to clinical-stage research, Zikani is actively moving into pre-clinical development to target select rare diseases including inherited diseases and cancers caused by nonsense mutations. For more information, visit [zikani.com](http://zikani.com).

### **Forward-Looking Statements**

*This press release contains forward-looking statements, which are generally statements that are not historical facts. Forward-looking statements can be identified by the words "expects," "anticipates," "believes," "intends," "estimates," "plans," "will," "outlook" and similar expressions. Forward-looking statements are based on management's current plans, estimates, assumptions and projections, and speak only as of the date they are made. We undertake no obligation to update any forward-looking statement in light of new information or future events, except as otherwise required by law. Forward-looking statements involve inherent risks and uncertainties, most of which are difficult to predict and are generally beyond our control. Actual results or outcomes may differ materially from those implied by the forward-looking statements as a result of the impact of a number of factors, including: the development of the Company's readthrough technology; the approval of the Company's patent applications; the Company's ability to successfully defend its intellectual property or obtain necessary licenses at a cost acceptable to the Company, if at all; the successful implementation of the Company's research and development programs and collaborations; the Company's ability to obtain applicable regulatory approvals for its current and future product candidates; the acceptance by the market of the Company's products should they receive regulatory approval; the timing and success of the Company's preliminary studies, preclinical research, clinical trials, and related regulatory filings; the ability of the Company to consummate additional financings as needed; the impact of global health concerns, such as the COVID-19 global pandemic, on our ability to continue our clinical and preclinical programs and otherwise operate our business effectively, including successfully integrating the combined companies; as well as those discussed in more detail in our Annual Report on Form 10-K and our other reports filed with the Securities and Exchange Commission.*

**Contact**

Investors

John Woolford

[john.woolford@westwicke.com](mailto:john.woolford@westwicke.com)

443.213.0506

Media

Laureen Cassidy

[laureen@outcomescg.com](mailto:laureen@outcomescg.com)

SOURCE: Eloxx Pharmaceuticals, Inc.

Eloxx Pharmaceuticals