



Eloxx Pharmaceuticals Reports Fourth Quarter 2021 Financial and Operating Results and Provides Business Update

March 31, 2022

Expect topline data from cystic fibrosis (CF) Phase 2 expansion treatment arms evaluating combination with ivacaftor by the end of the first half of 2022

Cystic Fibrosis Foundation (CF Foundation) to provide an award of up to \$15.9 million for the ongoing ELX-02 clinical program

New development program for ELX-02 for the treatment of Alport syndrome, a rare kidney genetic disorder; initiation of proof-of-concept clinical trial expected in second half of 2022

On track to start First in Human Phase 1 study in 2022 with ZKN-013 that is being developed for the treatment recessive dystrophic epidermolysis bullosa (RDEB) patients with nonsense mutations cells

Treatment study of ZKN-013 in APC^{Min} mouse model of familial adenomatous polyposis (FAP) demonstrates significant reductions of 40% in polyp count and 50% in polyp burden leading to improved survival

WATERTOWN, Mass., March 31, 2022 (GLOBE NEWSWIRE) -- Eloxx Pharmaceuticals, Inc. (NASDAQ: ELOX), a leader in ribosomal RNA-targeted genetic therapies for rare diseases, today reported its financial results for the three months and year ended December 31, 2021 and provided a business update.

"The recently announced funding from the CF Foundation, together with positive topline clinical results from the monotherapy arm of Phase 2 of the ELX-02 clinical trial announced in November 2021, has increased our confidence in the potential of ELOX-02 to transform the lives of Class 1 CF patients with nonsense mutations," said Sumit Aggarwal, President and Chief Executive Officer of Eloxx. "In addition to the ELOX-02 CF program, we believe we continue to make significant progress across our portfolio of product candidates. With the addition of a new program in Alport syndrome, a rare kidney disease, we believe we are beginning to fully capture the potential of ELX-02 as a novel readthrough agent while at the same time, our oral RMAs continue to advance forward with encouraging preclinical data in RDEB and FAP that demonstrate the potential of ribosome modulation."

Fourth Quarter 2021 and Subsequent Highlights

Class 1 Cystic Fibrosis

- Eloxx recently announced additional funding from a Therapeutic Development Award of up to \$15.9 million from the CF Foundation to support the ongoing global Phase 2 ELX-02 clinical development of ELX-02 in CF. This award is in addition to the previously announced partial funding of the global clinical trial program. Following an upfront funding of \$7.0 million, the funding will be tranching based on the achievement of certain clinical milestones. Eloxx will pay the CF Foundation royalties tiered to the actual level of funding from the CF Foundation.
- Expansion arm of the ongoing Phase 2 clinical trials in CF patients affected by nonsense mutations in the CFTR (CF transmembrane conductance regulator) gene, which is designed to evaluate the safety of ELX-02 and assess short-term biological activity in patients, remains ongoing.
 - In preclinical studies, Class 1 CF patient organoids had a 2- to 3-fold higher swelling response with a combination of ELX-02 and ivacaftor than with ELX-02 as a monotherapy.
 - Topline results are expected by the end of the first half of 2022.
- Topline results from monotherapy arm of Phase 2 trials announced in November 2021. ELX-02 was well tolerated and achieved a statistically significant 5.4mmol/L reduction in sweat chloride in patients at the 1.5mg/kg/day dose.

- Evaluation of inhaled (nebulizer-based) delivery of the current subcutaneous formulation of ELX-02 remains ongoing. We believe this has the potential to further improve the activity of ELX-02 as a single agent and in combination with other drugs given potential for increased drug exposure in the lung versus plasma. We expect to submit an Investigational New Drug (IND) application in the second half of 2022.
- As previously announced, the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for ELX-02 for the treatment of CF patients with nonsense mutations. In addition, ELX-02 has also been granted Orphan Drug Designation for the treatment of CF patients with nonsense mutations by the FDA and orphan medicinal product designation by the European Commission.

Alport Syndrome

- Eloxx recently announced it has expanded the ELX-02 development pipeline to include the treatment of Alport syndrome, a rare kidney disease. Alport syndrome is a genetic disorder characterized by kidney disease with high levels of proteinuria, hearing loss and eye abnormalities caused by mutations in the genes (COL4A3, COL4A4, and COL4A5) needed for production of type 4 collagen.
- Eloxx believes there is a strong rationale to pursue clinical development of ELX-02 in Alport syndrome based on encouraging preclinical results demonstrating potentially therapeutic levels of readthrough, ability to deliver high drug concentrations in the kidney relative to plasma at clinically tolerable dose levels, and the clinical readthrough results seen in our Phase 2 cystic fibrosis trial.
 - ELX-02 is preferentially taken up in the kidney, resulting in an expected greater than 50-fold exposure in the kidneys compared to plasma.
 - In recently published preclinical studies, ELX-02 has demonstrated significant readthrough in COL4A5 mutations, which represent 85% of nonsense mutations in this population.
- Eloxx intends to initiate a proof-of-concept clinical trial in up to eight Alport syndrome patients with nonsense mutations in the second half of 2022. Patients will be dosed for two months with a three month follow-up. Trial primary endpoints include safety while secondary endpoints are reduction in proteinuria and induction of COL4A5 protein expression in the kidney. Initial topline results are expected in the first half of 2023.

Recessive Dystrophic Epidermolysis Bullosa and Junctional Epidermolysis Bullosa (JEB)

- We believe Eloxx remains on track to file an IND application start a First in Human (FIH) Phase 1 study in 2022 with ZKN-013 based on continued preclinical validation of ability to induce functional full-length Collagen 7A in RDEB patient cells and encouraging safety results in recently completed non Good Laboratory Practice (GLP) animal studies.
- Eloxx has conducted additional preclinical studies of ZKN-013. These demonstrated that ZKN-013 induces the production of functional, full-length COL7 in RDEB patient cells at levels comparable to high dose gentamicin. Induction of readthrough in multiple cell types and mutations from ZKN-013 was confirmed by research labs at University of Southern California and the Thomas Jefferson University, widely recognized leaders in RDEB.

Familial Adenomatous Polyposis and Targeted Oncology

- Eloxx continues to advance our preclinical oncology pipeline, which is focused on rare inherited cancers with driver nonsense mutations and cancers with driver mutations in the ribosome.
- Our most advanced program is for the treatment of patients with FAP with nonsense mutations. Eloxx has seen encouraging results for ZKN-013 in the APC^{Min} (multiple intestinal neoplasia) model for treatment of patients with FAP for ZKN-013. Eloxx completed an 8-week treatment study in this model to evaluate the potential of ZKN-013 to treat FAP. The APC^{Min} mouse is a translationally validated model for drug development for FAP.
 - The study demonstrated a significant 50% reduction in polyp burden and a 40% reduction in the number of polyps in ZKN-013 treated mice, including substantial reductions in both lesion area and area of adenoma with no progression to carcinomas. This led to an observed 50% survival benefit. These results compare favorably with treatment results with Celecoxib.
- Cancer cell line and xenograft mouse studies ongoing to evaluate the response to treatment with RMAs to advance onco-ribosome targeted inhibitors for oncology therapy.

- In September 2021, Eloxx entered into a debt facility with Hercules Capital, Inc., a specialty financing lender for life science and technology companies, that provides for borrowings of up to \$30.0 million, comprised of three tranches. On September 30, 2021, Eloxx drew the initial tranche of \$12.5 million and used the proceeds to repay in full amounts outstanding under the Eloxx' existing debt facility with Silicon Valley Bank. The remaining tranches under the new facility will be available to Eloxx based on achieving certain clinical and equity milestones during defined time periods.

Fourth Quarter 2021 Financial Results

For the three months ended December 31, 2021, we incurred a net loss of \$12.1 million, or \$0.14 per share, which includes \$1.6 million in stock-based compensation. For the same period in the prior year, we incurred a net loss of \$6.1 million, or \$0.15 per share. Since the closing date of Eloxx's acquisition of Zikani Therapeutics, Inc. in April 2021, the results of Zikani's operations have been included in the Eloxx' condensed consolidated financial statements.

Our research and development expenses (R&D) were \$7.9 million for the three months ended December 31, 2021, which includes \$0.6 million in stock-based compensation. For the same period in the prior year, R&D expenses were \$2.6 million. The increase was primarily related to expenses related to the continued development of ELX-02 due to the impact of the COVID-19 pandemic in the prior year period, an increase in salaries and other personnel related costs, and an increase in operational facilities.

Our general and administrative (G&A) expenses were \$3.7 million for the three months ended December 31, 2021, which includes \$1.0 million in stock-based compensation. For the same period in the prior year, G&A expenses were \$3.1 million. The increase was primarily related to an increase in stock-based compensation expense, an increase in salaries and other personnel related costs, as well as an increase in legal, accounting and other professional fees.

As of December 31, 2021, we had cash and cash equivalents of \$42.3 million, which combined with \$7.0 million from the CF Foundation, we expect will be sufficient to fund our operations into the second quarter of 2023.

About Nonsense Mutations

Nonsense mutations cause a premature stop codon in the mRNA resulting in less than full length or loss of function proteins. These remain highly underserved with no approved disease modifying therapies. An estimated 10-12% patients across over 8,000 inherited genetic rare diseases harbor nonsense mutations in one or both alleles harboring nonsense mutations.

About Eloxx Pharmaceuticals

Eloxx Pharmaceuticals, Inc. is engaged in the science of ribosome modulation, leveraging its innovative TURBO-ZM™ chemistry technology platform in an effort to develop novel Ribosome Modulating Agents (RMAs) and its library of Eukaryotic Ribosome Selective Glycosides (ERSGs). 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 clinical development, focusing on cystic fibrosis (US Trial NCT04135495, EU/IL Trial NCT04126473). Eloxx also has preclinical programs focused on select rare diseases, including inherited diseases, cancer caused by nonsense mutations, kidney diseases, including autosomal dominant polycystic kidney disease, as well as rare ocular genetic disorders.

For more information, please visit www.eloxxpharma.com.

Forward-looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements other than statements of present and historical facts contained in this press release, including without limitation, statements regarding our future financial results, the expected timing of trials and results from clinical studies of our product candidates and the potential of our product candidate to treat nonsense mutations are forward-looking statements. Forward-looking statements can be identified by the words "aim," "may," "will," "would," "should," "expect," "explore," "plan," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential," "seeks," or "continue" or the negative of these terms similar expressions, although not all forward-looking statements contain these words.

Forward-looking statements are based on management's current plans, estimates, assumptions and projections based on information currently available to us. Forward-looking statements are subject to known and unknown risks, uncertainties and assumptions, and actual results or outcomes may differ materially from those expressed or implied in the forward-looking statements due to various important factors, including, but not limited to: our ability to progress any product candidates in preclinical or clinical trials; the uncertainty of clinical trial results and the fact that positive results from preclinical studies are not always indicative of positive clinical results; the scope, rate and progress of our preclinical studies and clinical trials and other research and development activities; the competition for patient enrollment from drug candidates in development; the impact of the global COVID-19 pandemic on our clinical trials, operations, vendors, suppliers, and employees; our ability to obtain the capital necessary to fund our operations; the cost of filing, prosecuting, defending and enforcing any patent claims and other intellectual property rights; our ability to obtain financial in the future through product licensing, public or private equity or debt financing or otherwise; general business conditions, regulatory environment, competition and market for our products; and business ability and judgment of personnel, and the availability of qualified personnel and other important factors discussed under the caption "Risk Factors" in our Annual Report on Form 10-K for the fiscal year ended December, 2021, as any such factors may be updated from time to time in our other filings with the SEC, accessible on the SEC's website at www.sec.gov and the "Financials & Filings" page of our website at <https://investors.eloxxpharma.com/financials-filings>.

All forward-looking statements speak only as of the date of this press release and, except as required by applicable law, we have no obligation to update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

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ELOXX PHARMACEUTICALS, INC. AND SUBSIDIARIES
UNAUDITED CONDENSED CONSOLIDATED BALANCE SHEETS
(Amounts in thousands, except share and per share data)

	December 31, 2021	December 31, 2020
ASSETS		
Current assets:		
Cash and cash equivalents	\$ 42,268	\$ 24,668
Restricted cash	299	56
Prepaid expenses and other current assets	913	1,169
Total current assets	43,480	25,893
Property and equipment, net	216	133
Operating lease right-of-use assets	1,443	421
Other long-term assets	-	30
Total assets	\$ 45,139	\$ 26,477
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	\$ 1,379	\$ 481
Accrued expenses	4,196	2,924
Current portion of long-term debt	-	5,239
Advances from collaboration partners	3,723	805
Current portion of operating lease liabilities	657	389
Total current liabilities	9,955	9,838
Long-term debt	11,996	6,376
Operating lease liabilities	804	33
Total liabilities	22,755	16,247
Total stockholders' equity	22,384	10,230
Total liabilities and stockholders' equity	\$ 45,139	\$ 26,477

ELOXX PHARMACEUTICALS, INC. AND SUBSIDIARIES
UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS
(Amounts in thousands, except share and per share data)

	Three Months Ended December 31,		Year Ended December 31,	
	2021	2020	2021	2020
Operating expenses:				
Research and development	\$ 7,912	\$ 2,640	\$ 22,899	\$ 14,590
General and administrative	3,718	3,142	20,449	14,847
In process research and development	—	—	22,670	—
Restructuring charges	—	24	—	4,018
Total operating expenses	11,630	5,806	66,018	33,455
Loss from operations	(11,630)	(5,806)	(66,018)	(33,455)
Other expense, net	460	321	709	1,122
Net loss	\$ (12,090)	\$ (6,127)	\$ (66,727)	\$ (34,577)
Basic and diluted net loss per share	\$ 0.14	\$ 0.15	\$ 0.95	\$ 0.86

Weighted average number of common shares used in computing net loss per share, basic and diluted 86,386,335 40,153,552 69,962,843 40,124,953

Source: Eloxx Pharmaceuticals



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