Unlocking protein production with translational read-through for rare genetic diseases

Second Quarter 2018 Financial Results and Business Update
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 read-through 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; 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.
Eloxx Pharmaceutical Highlights

- Expanded Leadership Team
- Prepared to move to Phase 2 (FPFV in 2018)
  - Phase 1a SAD Complete; Phase 1b MAD 4th Cohort Complete (to enter 5th and final)
  - Final Approval CTA for Cystic Fibrosis Phase 2 in Belgium
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- Expect Top Line Phase 2 Data Readout in 2019
- ELX-02 Abstract accepted for North American Cystic Fibrosis Society Meeting in October
  - “Measuring mRNA Levels in Cystic Fibrosis Organoids with Nonsense Mutations Following Treatment with ELX-02”
- Successful 1st Batch Release of lyophilized dosage form of ELX-02 (projected commercial formulation)
- On Track for Progression of 2nd Novel Molecule in 2018
- Well Funded to 2020
  - Cash and cash equivalents of $63.4 million at June 30, 2018
Built a Highly Experienced US Leadership Team

- Robert Ward, PhD
  CHAIRMAN AND CEO

- Greg Williams, PhD
  COO

- David Snow
  CBO

- Greg Weaver
  CFO

- Neil Belloff, JD
  GENERAL COUNSEL

- John van Duzer, PhD
  VP CMC

- Neal Sharpe, PhD
  VP TRANSLATIONAL SCIENCE

- Barbara Ryan
  INVESTOR RELATIONS
Orphan Drugs – A Major Growth Opportunity

- Orphan drugs will rise to over 20% of WW Pharma sales by 2024 and surpass $120bn in 2018
- Sellside consensus forecasts suggest orphan drugs will average 11.1% YOY growth, nearly double that of non-orphan drugs
- Respiratory Therapies (mostly Cystic Fibrosis) represent a top 5 (non-Onco) Orphan category with double digit growth expectations over the next 5 years
- Nonsense mutations occur in a subset of individuals within the populations of over 1,800 Orphan Diseases

Rapid Uptake of Orphan Disease Modifying Therapies

Cystic Fibrosis

1st launch

2nd launch

3rd launch

Projection

Cystic Fibrosis Nonsense Mutation Patients Represent a Key Unmet Need Population

Global Cystic Fibrosis Patients by mutation/genotype

- **F508del Mutations**
- **Other Mutations**
- **Nonsense Related Mutations**

CFTR Nonsense mutation subtypes

- **G542X**
- **W1282X**
- **R1162X**
- **R553X**
- **Other Nonsense Mutations**

No Currently Approved Drugs To Treat CFTR Nonsense Mutations

Source: Eloxx Internal Research/CFTR2 database
A CF swelling assay on cystic fibrosis patient organoids

Patient Organoid without drug treatment: No Swelling of Organoids

Patient Organoid with drug treatment: Swelling of Organoids
Cystic Fibrosis Organoid Responsive to ELX-02, Enhanced by Combination

- As presented at the European Cystic Fibrosis Society 41st Conference, Belgrade, Serbia, June 2018
- 100 µg/mL ELX-02 As previously presented
Complex Heterozygous Nonsense Mutation (G542X:R1066C missense)

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ELX-02 + Potentiator & Corrector
Potentiator & Corrector
ELX-02*
Homozygous Deletion Mutation (F508del)

Cystic Fibrosis Organoids Without Nonsense Mutations are Not Responsive to ELX-02

- As presented at the European Cystic Fibrosis Society 41st Conference, Belgrade, Serbia, June 2018
- 100 µg/mL ELX-02 As previously presented
ELX-02 Clinical Development – Phase 1 Studies

CLINICALTRIALS.GOV
Identifier: NCT03292302
A Phase 1a, Randomized, Double-blinded, Placebo-Controlled, Single Dose Escalation Study to Evaluate the Safety, Tolerability, and Pharmacokinetics of ELX-02 in Healthy Adult Volunteers

TO DATE:
• No SAE Observed
• No renal or otoacoustic SAE
• Generally well tolerated

CLINICALTRIALS.GOV
Identifier: NCT03309605
A Phase 1, Randomized, Double-Blinded, Placebo-Controlled, Third Party Open, Multiple Dose Escalation, Single Center Study to Evaluate the Safety, Tolerability and Pharmacokinetics of Subcutaneously Administered ELX-02 in Independent Consecutive Cohorts of Healthy Subjects

Planned Enrollment: 45

Completed dosing of 4th Cohort
Clinical Update for ELX-02 Phase 2 in Cystic Fibrosis

- Clinical Trial Application (CTA) for Phase 2 Study received final approved by the FAMHP in Belgium
  - Protocol assigned a High priority ranking by the ECFS-CTN
  - Study initiation activities ongoing
- Engaging with investigators on a protocol for Phase 2 to insure rapid execution
  - Will evaluate changes in sweat chloride at ascending doses
  - Planned enrollment will focus on patients with G542X nonsense mutation on one (complex heterozygote) or both alleles (homozygote)
- On track for first patient first visit (FPFV) this year
  - Study will be posted on clintrials.gov
- Expect top line data in 2019
Clinical Update for ELX-02 Phase 2 in Cystinosis

- IND for ELX-02 in cystinosis is open in the US
  - Engaging Investigators on Phase 2 protocol
  - Will evaluate changes in cysteine levels in white blood cells
  - FDA granted ELX-02 orphan drug status in cystinosis
- On track for first patient first visit (FPFV) this year
  - Study will be posted on clintrials.gov
- Expect top line data on cysteine levels in 2019
- Data previously reported showed that ELX-02 decreases the cysteine content in cellular and animal models*

* Dr. Paul Goodyer at the 14th Annual WORLDSymposium on Lysosomal Diseases in a presentation titled “Translational read through of CTNS nonsense mutations and attenuation of CTNS nonsense-mediated mRNA decay by ELX-02”
On Track for Advancing 2nd Novel Development Candidate

Eloxx holds global rights on these library compounds
ELX-02 Composition of Matter 2031 without extensions
Library Composition of Matter from 2027-2038 or later
Library Use Patents Expire 2036 or later
## 2Q’18 Financial Summary

<table>
<thead>
<tr>
<th>Operating expenses:</th>
<th>Three Months Ended June 30,</th>
<th>Six Months Ended June 30,</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>2018</td>
<td>2017</td>
</tr>
<tr>
<td>Research and development</td>
<td>$4,150</td>
<td>$2,595</td>
</tr>
<tr>
<td>General and administrative</td>
<td>9,560</td>
<td>584</td>
</tr>
<tr>
<td>Reverse merger related expenses</td>
<td>(167)</td>
<td>—</td>
</tr>
<tr>
<td>Total operating expenses</td>
<td>13,543</td>
<td>3,179</td>
</tr>
<tr>
<td>Loss from operations</td>
<td>(13,543)</td>
<td>(3,179)</td>
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<tr>
<td>Other (income) expense, net</td>
<td>(137)</td>
<td>699</td>
</tr>
<tr>
<td>Net loss</td>
<td>$(13,406)</td>
<td>$(3,878)</td>
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<tr>
<td>Basic and diluted net loss per share</td>
<td>$(0.42)</td>
<td>$(1.04)</td>
</tr>
<tr>
<td>Weighted average number of common shares in computing basic and diluted net loss per share</td>
<td>31,823,766</td>
<td>4,205,277</td>
</tr>
</tbody>
</table>
Financial Summary

- $63.4 million cash as of June 30, 2018
- No debt
- Funded to 2020 and top line data from the Phase 2 trials in cystic fibrosis and cystinosis
- Shares outstanding totaled 34.9 million as of 6/30/18
- Completed public offering of 5,899,500 shares of common stock at a price of $9.75 per share on March 30, 2018, raising net proceeds of approximately $53.6 million
- Traded Nasdaq: ELOX
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Upcoming Investor Presentations

- Canaccord Genuity Growth Conference
  - August 8, 2018
- Citi Biotech Conference
  - September 6, 2018
- ISI Biotech Deep Dive Conference
  - September 27, 2018
- Oppenheimer Fall Life Science Summit
  - September 28, 2018
- Cantor Global Healthcare Conference
  - October 2, 2018
- 2018 Piper Jaffray Healthcare Conference
  - November 27-28, 2018
Thank you.

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