



# Æterna Zentaris

**Improving Life... Transforming Value**

**May 9, 2017**

# Forward-Looking Statements

This presentation contains forward-looking statements made pursuant to the safe-harbor provision of the U.S. Securities Litigation Reform Act of 1995, which reflect our current expectations regarding future events. Forward-looking statements may include, but are not limited to statements preceded by, followed by, or that include the words “expects,” “believes,” “intends,” “anticipates,” and similar terms that relate to future events, performance, or our results. Forward-looking statements involve known risks and uncertainties, many of which are discussed under the caption “Key Information - Risk Factors” in our most recent Annual Report on Form 20-F filed with the relevant Canadian securities regulatory authorities in lieu of an annual information form and with the U.S. Securities and Exchange Commission (“SEC”). Such statements include, but are not limited to, statements about the timing of, and prospects for, regulatory approval and commercialization of our product candidates, statements about the status of our efforts to establish a commercial operation and to obtain the right to promote or sell products that we did not develop and estimates regarding our capital requirements and our needs for, and our ability to obtain, additional financing. Known and unknown risks and uncertainties could cause our actual results to differ materially from those in forward-looking statements. Such risks and uncertainties include, among others, our now heavy dependence on the success of Macrilen™ and the continued availability of funds and resources to successfully complete the submission of an NDA without undue delay with respect to Macrilen™ and, in the event the FDA approves Macrilen™, to successfully launch the product, the rejection or non-acceptance of any new drug application by one or more regulatory authorities and, more generally, uncertainties related to the regulatory process, the ability of the Company to efficiently commercialize one or more of its products or product candidates (including, in particular, Macrilen™), the degree of market acceptance once our products are approved for commercialization (including, in particular, Macrilen™), our ability to take advantage of business opportunities in the pharmaceutical industry, our ability to protect our intellectual property, the potential of liability arising from shareholder lawsuits and general changes in economic conditions. Investors should consult the Company’s quarterly and annual filings with the Canadian and U.S. securities commissions for additional information on risks and uncertainties. Given these uncertainties and risk factors, readers are cautioned not to place undue reliance on these forward-looking statements. We disclaim any obligation to update any such factors or to publicly announce any revisions to any of the forward-looking statements contained herein to reflect future results, events or developments, unless required to do so by a governmental authority or applicable law.

# Macrilen™ – Continued Progress

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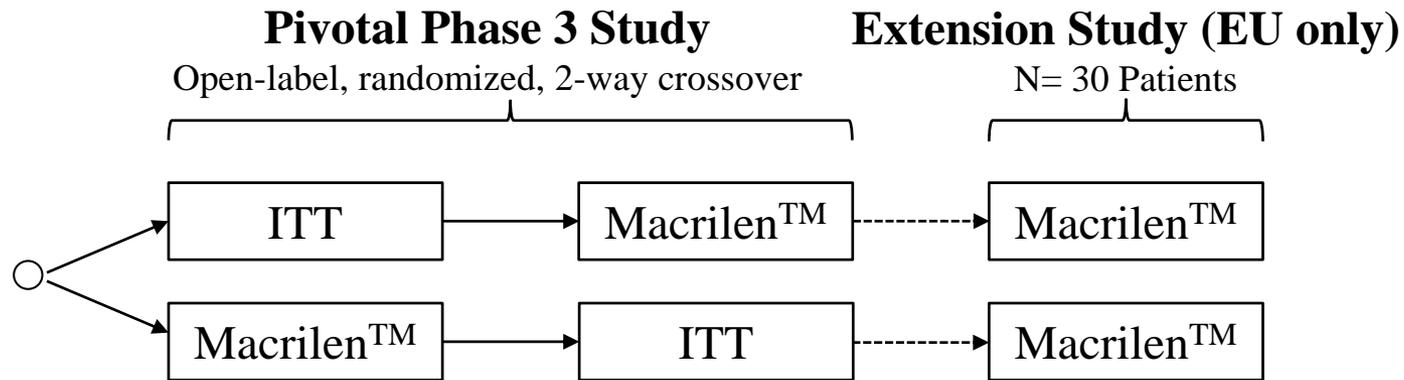
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**Press Release**  
For immediate release

## **Aeterna Zentaris Intends to File NDA with respect to Macrilen™ in Third Quarter of 2017**

**Charleston, S.C., March 30, 2017** — Aeterna Zentaris Inc. (NASDAQ: AEZS) (TSX: AEZS) (the “Company”) today announced that, following its meeting with the U.S. Food and Drug Administration (the “FDA” or the “Agency”) on March 29, 2017, the Company intends to file a new drug application (“NDA”) seeking approval of Macrilen™ (macimorelin) for the evaluation of growth hormone deficiency in adults (“AGHD”).

# Macrilen™ -- Confirmatory Phase 3 Trial Design



- **Primary Objective**
  - Validation of macimorelin for the diagnosis of AGHD, using the ITT as comparator
- **Co-Primary Efficacy Variables**
  - “Percent Negative Agreement” and “Percent Positive Agreement”
- **Patients**
  - Patients with suspected GHD (low ↔ high risk) and 25 healthy subjects
  - N = at least 110 ( $\geq 55$  with positive and  $\geq 55$  with negative ITT outcome)

# Macrilen™ Confirmatory Phase 3 Trial

## Results did not meet the pre-defined equivalence criteria for Macrilen™ success

- Negative Agreement: **93.94%** (CI: **85.20%**, **98.32%**)
- Positive Agreement: **74.32%** (CI: **62.84%**, **83.78%**)

Under the study protocol, performance of Macrilen™ was considered acceptable if the lower bound of the two-sided 95% confidence interval for the primary efficacy variables is

- 75% or higher for ‘Percent Negative Agreement’ and
- 70% or higher for the ‘Percent Positive Agreement’
- Secondary endpoint: (acceptance criteria not defined)
  - Overall agreement: 83.57 % (CI: 76.38%, 89.29%)

# Macrilen™ Confirmatory Trial – Conclusions

- **Macrilen™ stimulates the pituitary gland effectively to secrete growth hormone**
  - This stimulation was consistently more pronounced than the stimulation achieved with the ITT (in about 80% of all cases, peak growth hormone levels following the administration of Macrilen™ were equal to or higher than those observed during the ITT).
- **The Macrilen™ test performed well in the study:**
  - Sensitivity (87%) and Specificity (96%) of the Macrilen™ test were very good.
  - Data of the previous study (82% sensitivity, 92% specificity) could be reproduced.
  - The co-primary endpoint “negative agreement”, which is considered as the more relevant endpoint, was met, demonstrating that the Macrilen™ test provides medical benefit.
  - The co-primary endpoint “positive agreement” was not met.

# Macrilen™ Confirmatory Trial – Conclusions

- **In the repeatability extension part of the study, conducted upon request from the EMA, Macrilen™ results proved to be highly reproducible.**
  - 94% reproducibility (32 out of 34 cases at the pre-defined cut-off point)
  - Reproducibility of the ITT, which was not investigated in this study, appears worse than the Macrilen™ test as demonstrated by a high number of non-evaluable ITTs in the study
- **Study results can be further optimized by modulation of the pre-defined cut-off point of 2.8 ng/mL.**
  - Any cut-off point for Macrilen™ between 4.6 ng/mL and 8.2 ng/mL would have resulted in a positive study outcome in that both protocol-defined co-primary endpoints would have been met.
  - Increasing the cut-off point when comparing to the ITT outcome is justified by the more powerful stimulation of Macrilen™ as compared to the ITT (pre-defined cut-off point of 5.1 ng/mL)

# Macrilen™ Confirmatory Trial – Robustness

- **Macrilen™ was found to be a more robust test than ITT,** since only 1 out of 153 Macrilen™ tests needed to be repeated to become evaluable (compared to 28 out of 157 for the ITT)
  - Reproducible outcome of the Macrilen™ test upon planned repetition in the repeatability study (34 tests) is further evidence of its robustness
- **Macrilen™ stimulation of growth hormone release more pronounced than the ITT**
  - In about 80% of all cases, peak growth hormone levels following the administration of Macrilen™ were equal to or higher than those observed during the ITT

# Macrilen™ -- Target Market

- 40,000 annual AGHD stimulatory/confirmatory tests
  - Majority are patients with brain injury due to a tumor itself or treatment (surgery, radiation, chemo) thereof
  - Pediatric GHD patients transitioning into adulthood
  - Traumatic brain injury (TBI) due to accidents
  - Idiopathic causes
- Approximately 2,500 ENDOs
- 150 pituitary centers
- 200 to 300 additional large hospitals
- 13 Defense and Veterans Brain Injury Centers (DVBIC) providing TBI care for active and retired military

# Macrilen™ -- Target Market Dynamics

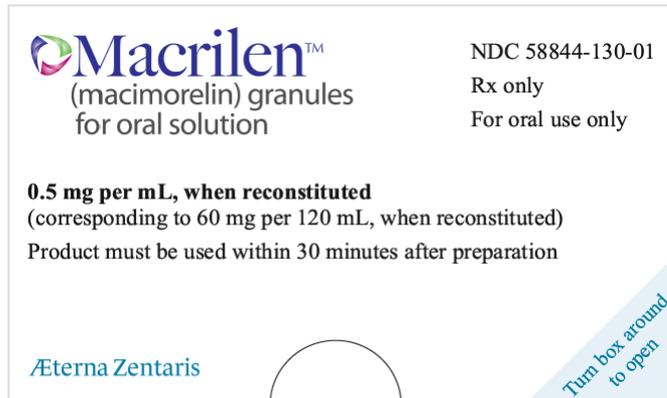
- The current unapproved AGHD stimulatory tests, including the “gold standard” Insulin Tolerance Test (ITT) and Glucagon Stimulation Test (GST) are injectable (IM/IV), take 3 to 5 hours to complete, are associated with potentially dangerous adverse events and/or offer questionable effectiveness (GST) or repeatability (ITT).
- Given their unapproved status, there is no commercial promotion of, nor support for, the ITT and GST tests, and no new competitor entrants in development.
- This results in the AGHD test market being a very profitable, albeit small commercial opportunity of approximately \$30-50M/annual. Significant additional market expansion opportunities exist in traumatic brain injury (TBI) and pediatric GHD.

# Macrilen™ -- Brand Differentiation

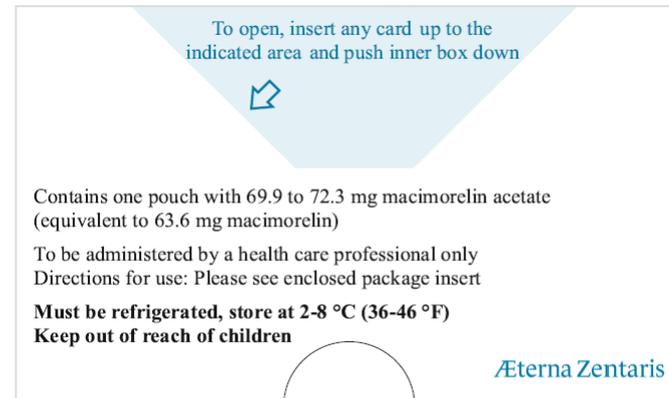
- A significant commercial opportunity to clearly differentiate Macrilen™, resulting in a value proposition that supports the establishment of Macrilen™ as the new preferred standard stimulatory test based upon meaningful clinical features from our phase III trial
  - Accuracy
  - Reproducibility
  - Potency
  - Safety
  - Convenience
  - FDA-approved
- Further health economics outcomes research (HEOR) will be conducted to document the improved “value” of Macrilen™ when compared to the true costs associated with implementing the ITT or GST stimulatory test procedures.

# Macrilen™

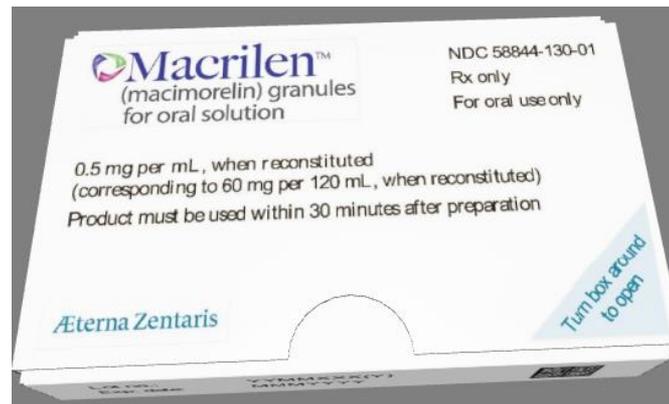
## Front



## Back



## 3D



# Q1 2017 Capital Overview

- Æ Adequate liquidity and resources to fund operations through expected Macrilen™ approval
- Æ ~\$17.8 million unrestricted cash and cash equivalents at end of Q1; no third party debt
- Æ Expected average operating cash use rate of between ~\$1.7 million and \$1.9 million/month for the remainder of 2017 (down approximately 22% compared to the first quarter)
- Æ Approximately \$4.3 million of gross proceeds raised from sales of Common Shares pursuant to various ATM programs during and subsequent to the first quarter
- Æ Approximately 14.3 million Common Shares outstanding



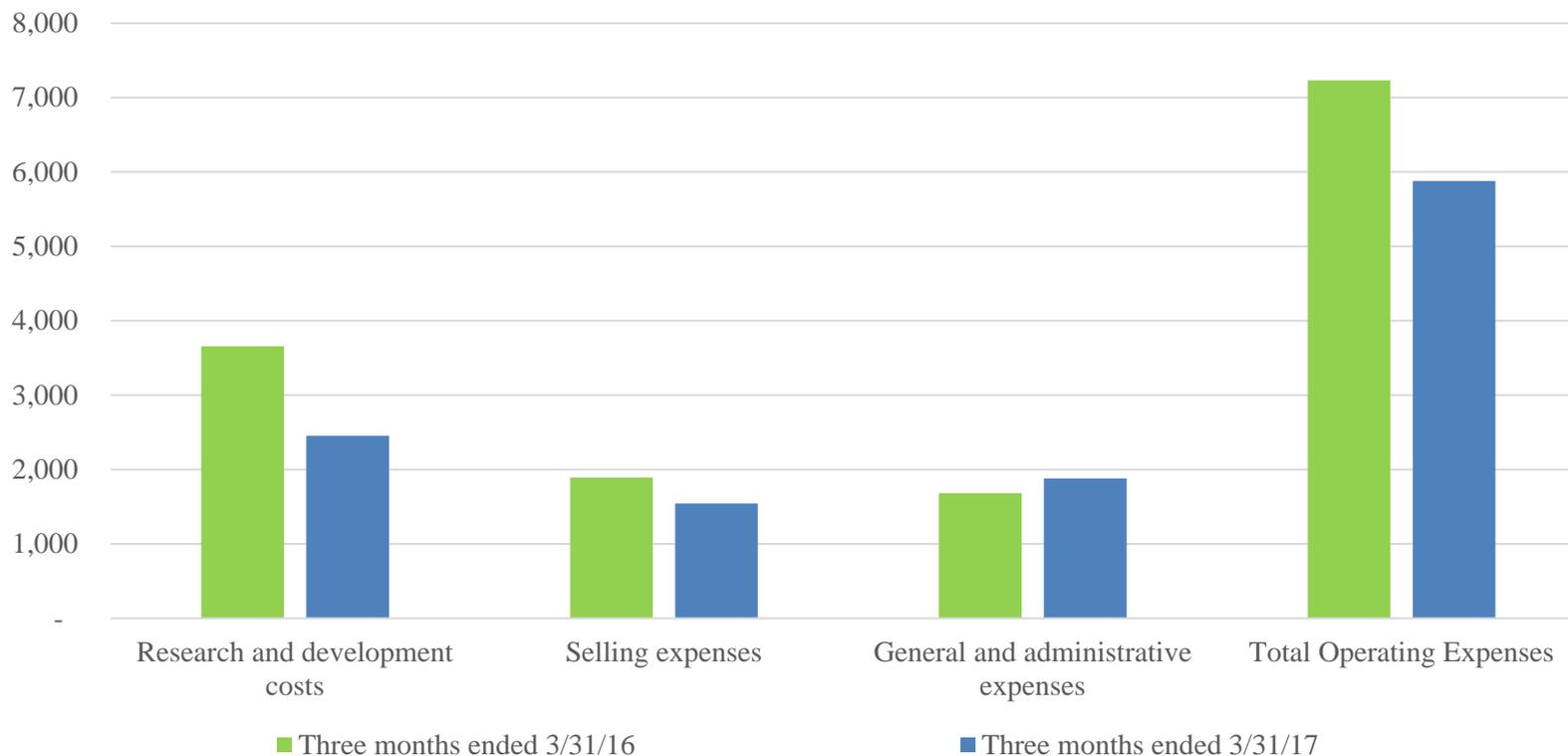
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## Financial Review: First Quarter 2017

# Operating expenses snapshot

(amounts in thousands of US dollars)

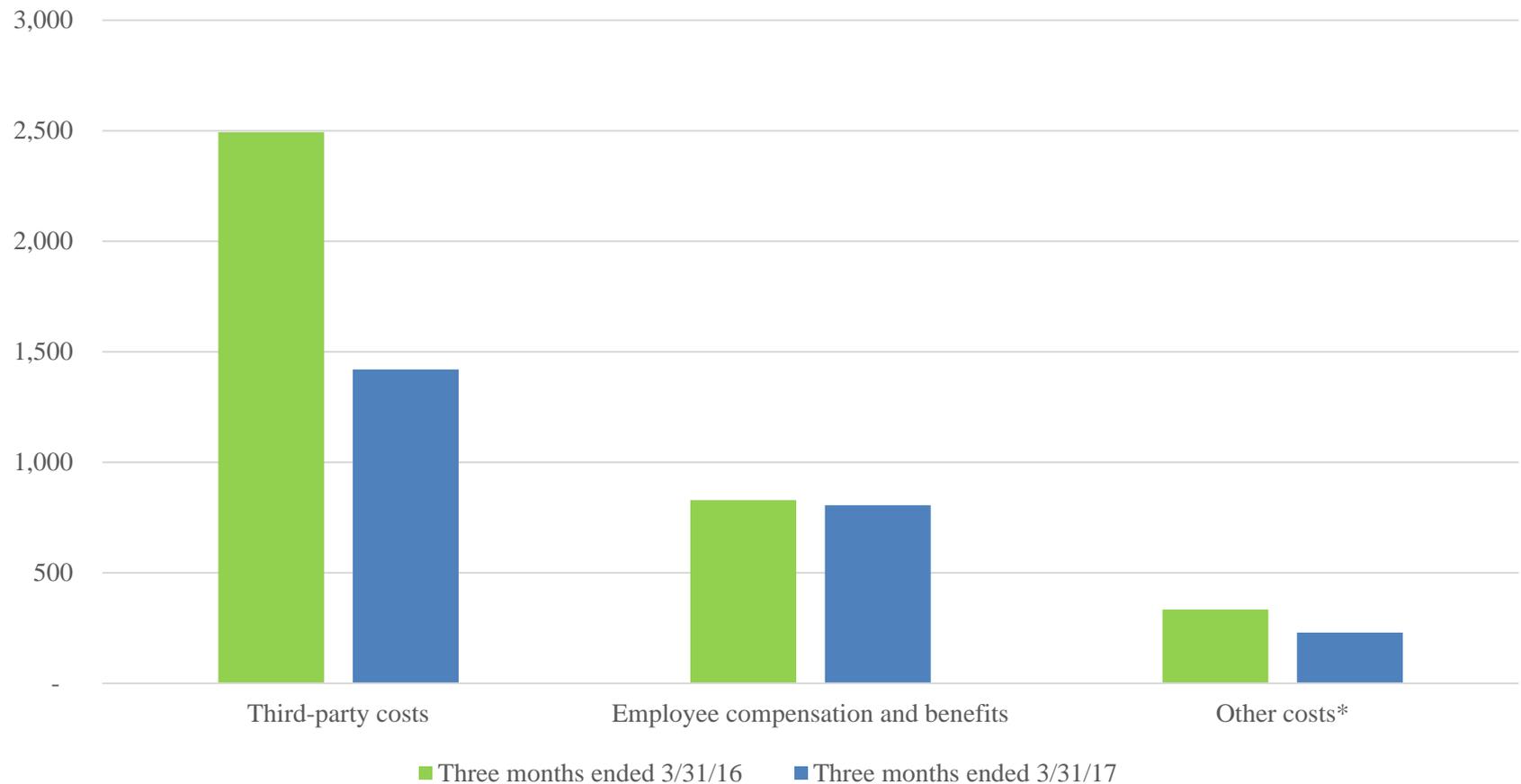
Comparative QTD & YTD



# R&D cost snapshot

## Comparative QTD & YTD

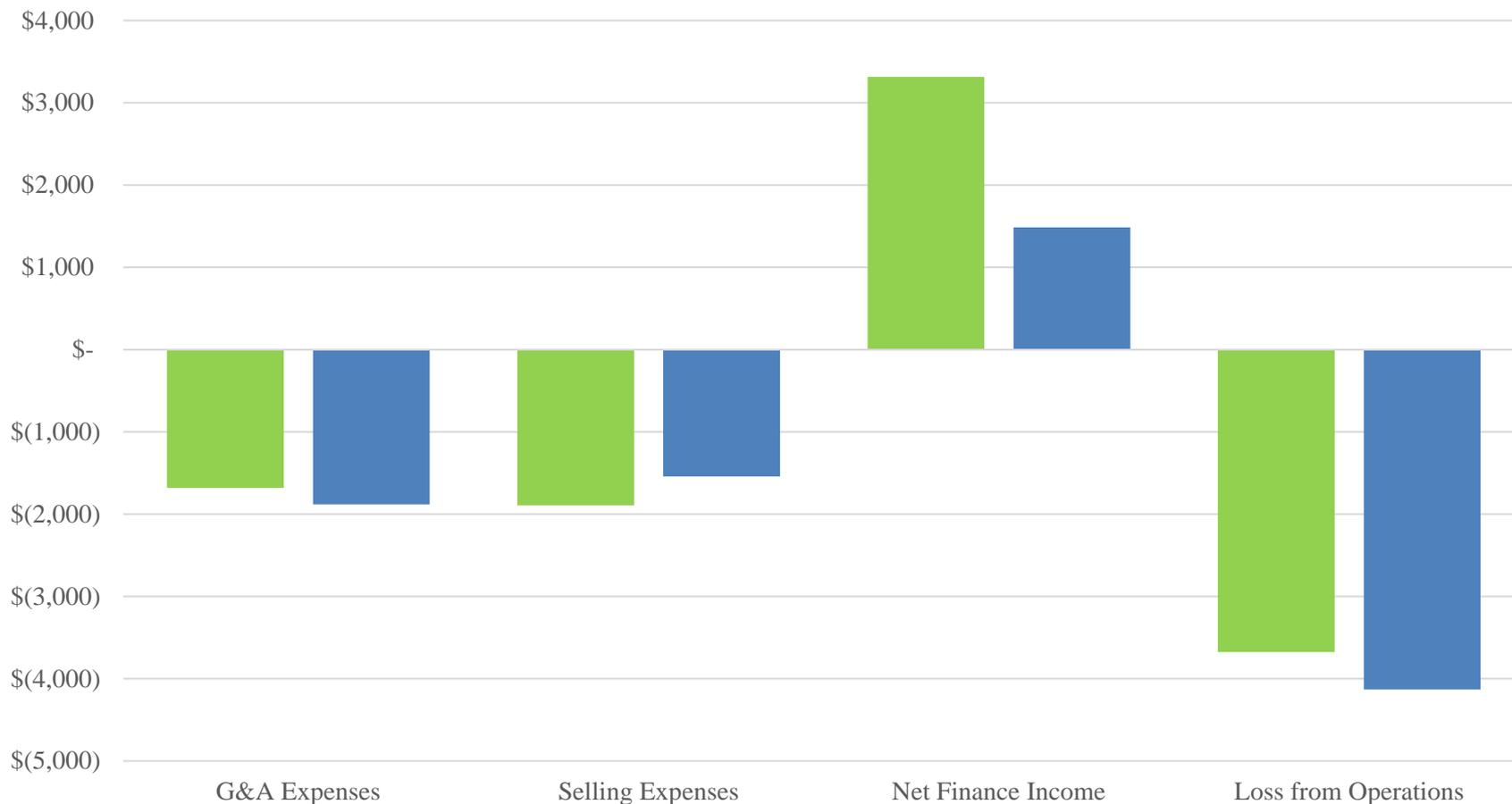
(amounts in thousands of US dollars)



# Abstract of the Condensed interim consolidated statements of comprehensive loss

## Comparative QTD & YTD

(amounts in thousands of US dollars)

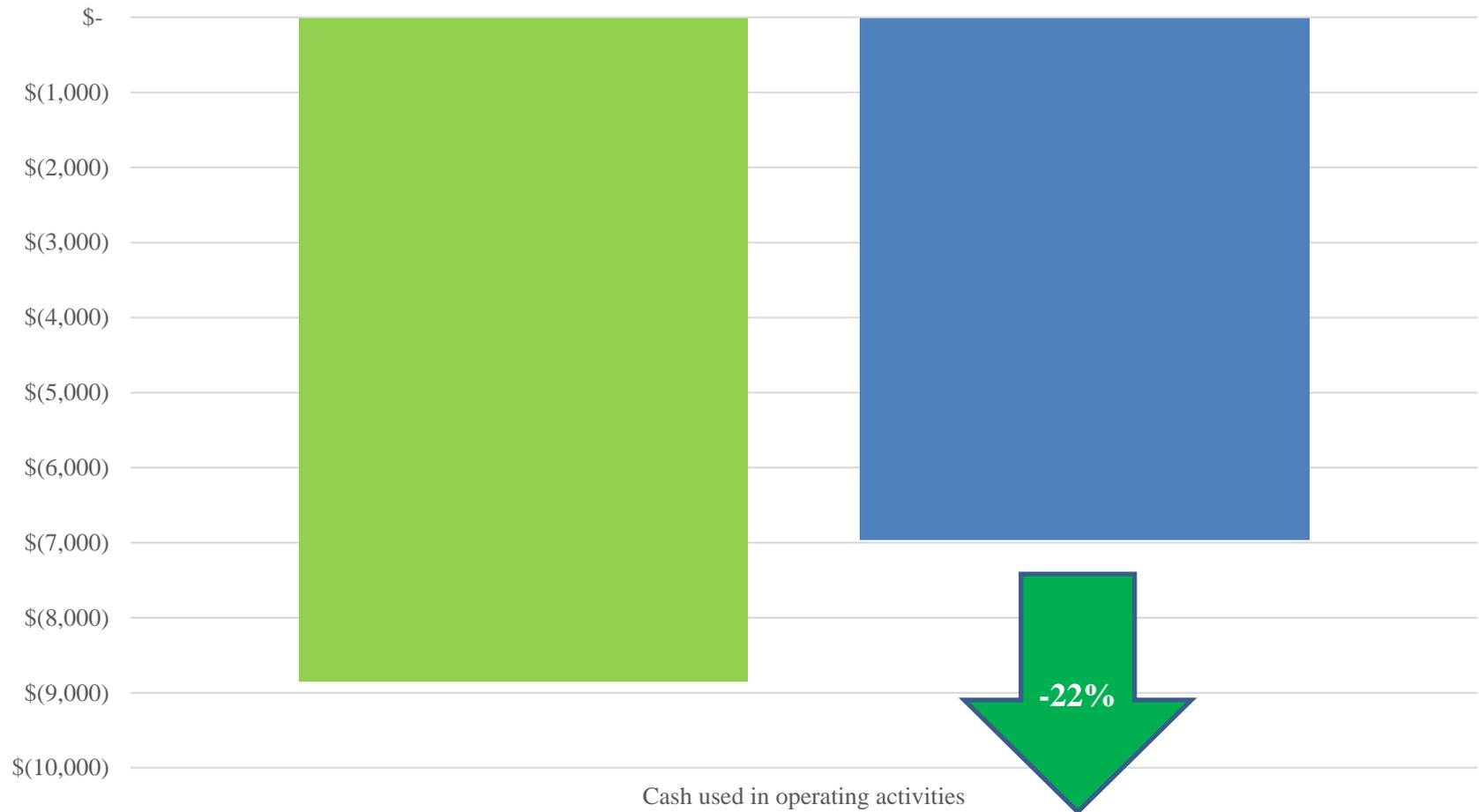


# Cash Flows used in Operations

Comparative QTD & YTD

**Average Monthly Burn Rate Significantly Reduced:  
\$2.9 million in Q1 2016 vs \$2.3 million in Q1 2017**

(amounts in thousands of US dollars)



# Q & A



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