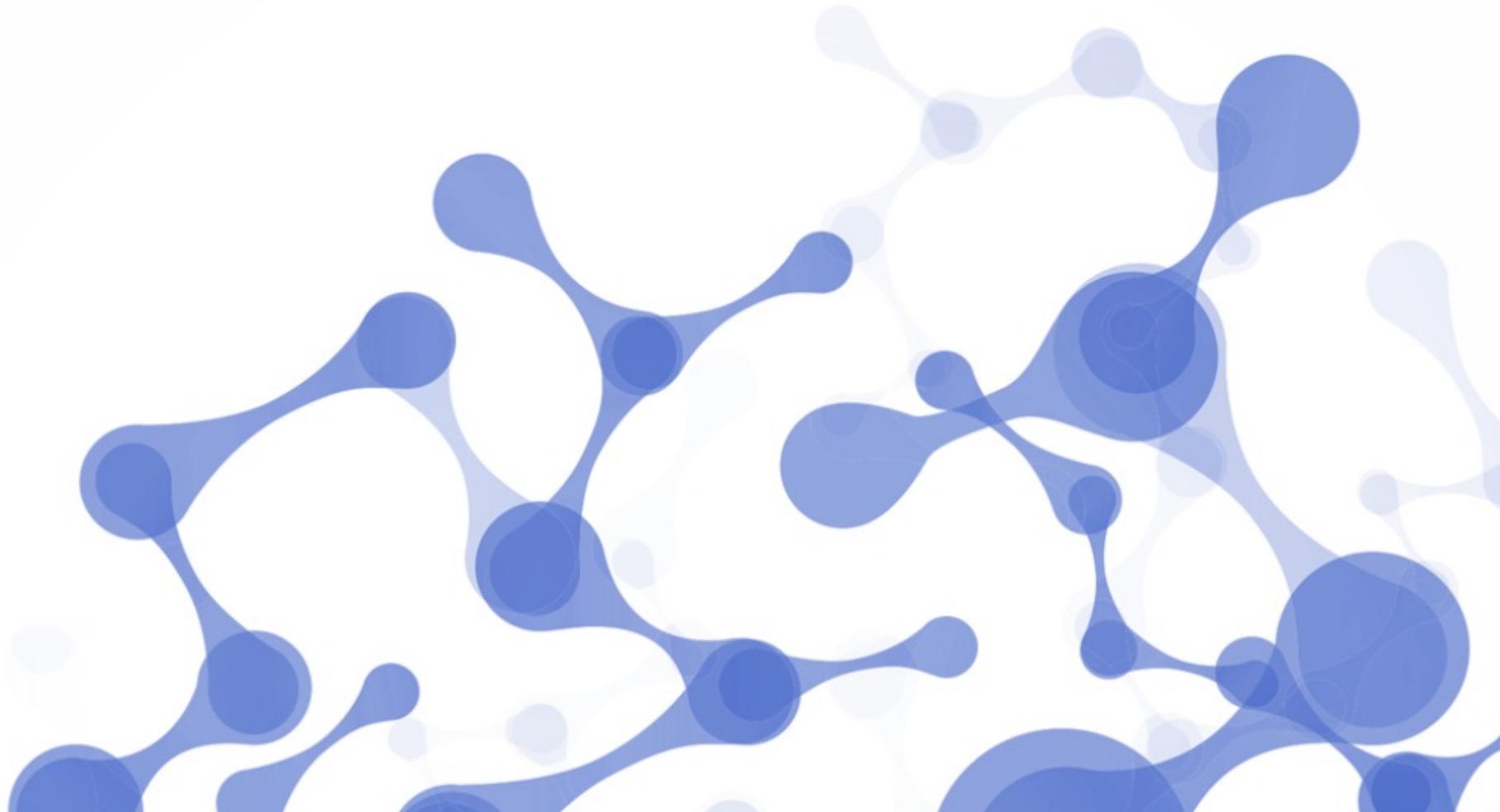




KemPharm^{INC}

Q3 2022 Results Call

November 9, 2022



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While we may elect to update such forward-looking statements at some point in the future, except as required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Although we believe the expectations reflected in such forward-looking statements are reasonable, we can give no assurance that such expectations will prove to be correct. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to this presentation.

This presentation also may contain estimates and other statistical data made by independent parties and by us relating to market size and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk.



Q3 2022 Results Call Agenda

1) Introduction

Travis Mickle, Ph.D.

President and Chief Executive Officer

2) Product Development Updates

Travis Mickle

3) Financial Update

R. LaDuane Clifton, CPA

Chief Financial Officer, Secretary & Treasurer

4) Question and Answer



KEMPHARM VALUE PROPOSITION

Innovative biotech company with a proven regulatory track record targeting rare CNS, neurodegenerative and lysosomal storage diseases

Revenue-generating assets with significant commercial potential in areas of high unmet need

Strong balance sheet which is expected to fund operations and U.S. commercial build into 2026

KemPharm: Q3 2022 and Recent Highlights

- ✓ Completion of the 4-year safety trial
- ✓ Ongoing collaborative dialogue and periodic meetings with the FDA
- ✓ Working to amass and characterize the new data generated since the CRL
- ✓ NDA refiling targeted as early as Q3 2023

Arimoclomol

**KP1077
Development
Program**

- ✓ Phase 1 cardiovascular trial data confirmed initial dosing strengths for Phase 2 trial in IH
- ✓ Data suggest SDX can be safely dosed at levels higher than current MPH products
- ✓ Phase 2 trial initiation in IH expected prior to year-end

- ✓ Potential to realize sales milestone and royalty revenue from AZSTARYS®
- ✓ Potential revenue could provide further capital flexibility and extend operating cash runway

AZSTARYS®

**Strong
Balance Sheet
to Support
Value Creation**

- ✓ Net revenue of \$2.9M includes revenue from arimoclomol EAP program in France
- ✓ Cash, cash equivalents and investments of \$107.4M as of Sept. 30, 2022
- ✓ Available capital expected to extend cash runway into 2026

Product Development Updates



Arimoclomol - Expanding Pipeline Targeting Rare Diseases

Aligns with strategy to build value through the development and commercialization of novel treatments for rare diseases

Niemann-Pick disease type C

- ✓ Ultra-rare progressive, disabling and fatal lysosomal storage disorder
- ✓ No approved treatments exist in the U.S. for NPC

Favorable Acquisition Terms

- ✓ “Capital efficient” financial structure with potential for positive cash flow and no shareholder dilution



High Upside Opportunity

- ✓ NDA-stage investigational drug candidate
- ✓ KemPharm has expertise in NDA resubmissions following CRLs

Early Access Programs

- ✓ Available to NPC patients in the U.S., France, Germany and other European countries
- ✓ French EAP expected to generate annual gross revenue of ~\$12M

Path to Resubmission and Approval Progressing

KemPharm has significant experience with challenging regulatory situations, including two FDA product approvals that followed initial CRLs

Recent Activities

- Continuing to have an ongoing collaborative dialogue and periodic meetings with the FDA
 - ✓ Intended to ensure an optimal NDA data package that addresses all issues in the CRL
- Advancing activities to bolster arimoclomol NDA with confirmatory evidence for resubmission to the FDA
 - ✓ Working to analyze and process the new data generated since the CRL
 - ✓ This includes data obtained from a 4-year arimoclomol safety study and safety data from other clinical trials with arimoclomol

Regulatory Outlook

- Throughout this process, no new issues or concerns have been raised by the FDA
 - No new efficacy trial has been proposed by FDA
- We believe there is a viable pathway to enable a successful NDA resubmission and subsequent approval for arimoclomol in NPC
 - ✓ Path may include, if necessary, additional non-clinical or clinical studies, a Federal Dispute Resolution Request (FDRR) and/or an advisory committee (ad com) requested by either FDA or KemPharm

KemPharm expects to resubmit the NDA for arimoclomol in NPC as early as Q3 2023



KP1077 – Product Candidate Overview

KemPharm is advancing KP1077 as a potential therapeutic treatment for Idiopathic Hypersomnia (IH)

Serdexmethylphenidate

- ✓ 100% SDX with multiple dosing options
- ✓ SDX has already been designated C-IV by DEA

Regulatory & IP Advantages

- ✓ Eligible for Fast-Track, Orphan Drug and Breakthrough Therapy designation
- ✓ Solid IP through 2037 and potentially beyond



Dosing Addresses Symptoms

- ✓ Dosed either 1x daily at bedtime or 2x daily at bedtime and at waking
- ✓ Potential to address primary IH symptoms: sleep inertia and brain fog

Improved Safety & Tolerability

- ✓ Greater tolerability and lower cardiovascular effects could allow for higher, more effective dosing (i.e. greater efficacy)
- ✓ No DDI potential with hormonal contraceptives; antidepressants

KP1077 – Multiple Clinical Programs Targeting Rare Sleep Indications

KP1077 Represents a Potential “Portfolio in a Pill” Opportunity

Idiopathic Hypersomnia

- Lead KP1077 indication
- Investigational New Drug (IND) application cleared by FDA
- Initiation of Phase 2 clinical trial anticipated prior to year-end 2022
- Interim data from Phase 2 clinical trial expected by mid-year 2023
- Top-line data available by EOY 2023

Narcolepsy

- Second KP1077 indication would allow KemPharm to address two rare sleep indications that are underserved by currently available medications
- Initiate narcolepsy Phase 3 Trial post IH Phase 2 results
 - Leverage key data points from IH program to expedite narcolepsy development



Financial Update and Upcoming Milestones



Financial Position is a Source of Strength

Q3 2022 Income Statement Details:

- Net revenue of \$2.9M, primarily from the arimoclomol EAP program in France
- Q3 2022 net loss attributable to common stockholders of (\$6.6M), or (\$0.19) per basic and diluted share, driven primarily by R&D expense of \$5.4M, and general and administrative expense of \$4.0M
 - Partially offset by net revenues of \$2.9 million

Balance Sheet Details as of Sept. 30, 2022:

- Cash, cash equivalents and investments were \$107.4M, a decrease of \$7.1M compared to Q2 2022
 - Driven in part by increased third-party research and development costs related to the KP1077 clinical trial program, the arimoclomol program, other expenses, as well as investment of working capital related to the collection of accounts receivable due from French EAP reimbursements
- Available cash, cash equivalents and investments expected to extend cash runway into 2026



KemPharm: Multiple Growth Catalysts

- ✓ Potential to re-file NDA as early as Q3 2023
- ✓ Anticipated ongoing quarterly revenue from EAP program in France

Arimoclomol

KP1077

- ✓ Phase 2 trial initiation in IH by the end of 2022
- ✓ Interim Phase 2 IH data expected by mid-2023
- ✓ Phase 3 trial in narcolepsy to initiate following IH Phase 2 trial results

- ✓ Potential to realize sales milestone and royalty revenue from AZSTARYS, providing further capital flexibility and extending operating cash runway

AZSTARYS®

**Strong
Balance Sheet
to Support
Value Creation**

- ✓ Solid balance sheet supports development efforts and other pipeline expansion activities
- ✓ Available capital extends cash runway into 2026





KemPharm^{INC}

**Addressing Rare CNS, Neurodegenerative
and Lysosomal Storage Diseases**

For additional information please
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