KemPharm INC

Q4 and FY 2021 Results Call

March 30, 2022

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KEMPHARM VALUE PROPOSITION

Innovative pharmaceutical company discovering and developing novel treatments for CNS and rare diseases

Two FDA approved and partnered medications, AZSTARYS® and APADAZ®, validate approach and science

Focus on high-value areas with significant unmet needs in CNS/rare disease with potential to internally commercialize

FY 2021 Year in Review: KemPharm's Transformation

Corporate and Regulatory Highlights

Jan 8: Regained listing on The Nasdaq Capital Market

Mar 2: FDA approval of AZSTARYS®

May 7: DEA designates SDX as a CIV controlled substance

Jun 9: Added to Russell 2000 and 3000 indexes

Jul 21: Commercial launch of AZSTARYS

Oct 19: Up-listed to The Nasdaq Global Select Market

Dec 13: Added to Nasdaq Biotech Index

Dec 13: Named 2021 David J Gury Company of the Year by BioFlorida

Dec 20: $50M Share Repurchase Program announced

Financial Highlights

Jan 8: Complete $50M secondary offering

Jan 12: Repaid $30.3M in debt, converted $31.5M into preferred shares, leaving a remainder of $7.6M due Mar 31, 2023

Jan 26: Complete warrant exercise inducement transaction with gross proceeds of approx. $44M

Feb 8: Repaid remainder of debt

Jun 18: Complete second warrant exercise inducement transaction, eliminating substantial portion of outstanding warrant overhang and gross proceeds of approx. $39.1M

Dec 31: End year with $127.8M in cash, cash equivalents and long-term investments, and $0 debt

KemPharm: Q4 2021 and Recent Highlights

- ✓ KP1077: Substantial high-value opportunity with significant unmet need
- ✓ "Higher Dose" SDX data suggest targeted pharmacodynamic effects that could benefit patients with IH and other sleep disorders

Initiation of KP1077 Development Program

Opportunities to Further Expand Pipeline

- ✓ Continuing efforts to build a highly differentiated pipeline of development assets
- ✓ Focused on high-value areas with significant unmet needs in **CNS/rare disease** with potential to internally commercialize

AZSTARYS® Launch Gaining Traction

- ✓ Full national team in place
- ✓ Sales force size doubled in early 2022
- ✓ **Currently 110M+ covered commercial lives**

Strong Balance Sheet to Support Value Creation

- ✓ Cash, cash equivalents and long-term investments of **$127.8M** as of Dec 31, 2021
- ✓ Solid balance sheet supports development efforts and other pipeline expansion activities
- ✓ **Available capital extends cash runway beyond 2025**

Pipeline of Product Candidates with Substantial Milestones in 2022 and Beyond

Indication	Product Candidate	Phase of Development	Anticipated Timing of Next Milestone
Rare Sleep Disorders			
Idiopathic Hypersomnia (IH)	KP1077	Phase 2	Q3 2022
Narcolepsy Type I and II	KP1077	Phase 2	Q4 2022
Sleep Disorders	TBD	In-licensing, acquisition or internal candidate	H2 2022
First-in-Class Therapy			
Stimulant Use Disorder (SUD)	KP879	Phase 2	External Funding and Collaborations
In-licensed or Acquired Product(s)			
CNS or Related	TBD	Phase 2 or later	H2 2022

SDX Product Candidate: KP1077

For the Treatment of Idiopathic Hypersomnia (IH)

Idiopathic Hypersomnia (IH)

- There are 10.3 IH patients per 100,000 people in the US[1]
  - ~37,000 diagnosed IH patients actively seeking treatment[2]
  - Patient population may be much larger (not seeking treatment, undiagnosed, misdiagnosed)
- Symptoms are highly debilitating – **IH can be more debilitating than narcolepsy**
  - Chronic daytime sleepiness
  - Long and unrefreshing naps
  - Extreme difficulty waking (sleep inertia and/or sleep drunkenness)
  - Severe brain fog
  - Some experience excessively long sleep times (~25% of patients “long sleepers”, >10hrs)
- IH patients report memory problems, errors in habitual activities, mind blank and attention problems as part of their disability
  - KOLs identified depression as a common comorbidity encountered with patients
  - Patient survey data indicates that current medication effectiveness was poorly rated at 5.4/10[(3)]

Sources: (1) https://doi.org/10.1093/sleep/zsy061.624
(2) https://www.sleepcountshcp.com/what-is-idiopathic-hypersomnia
(3) https://www.sleepcountshcp.com/idiopathic-hypersomnia-treatment-options

KP1077 Product Candidate Overview

- 100% Serdexmethylphenidate (SDX) product with multiple dosing options depending on patient needs
  - Dosed either QD (1x daily at bedtime) or BID (2x daily at bedtime and upon waking)
- Features and benefits already demonstrated:
  - **SDX has already been designated C-IV by DEA**
  - No DDI potential with hormonal contraceptives and antidepressants
- Potential additional features and benefits to be studied:
  - **Greater tolerability** could allow for higher, more effective dosing (i.e. greater efficacy)
  - Dosing regimen addresses the two primary issues associated with IH
    - Night-time dosing addresses sleep inertia (waking)
    - Morning dosing addresses daytime brain fog; considered most problematic symptom of IH
  - **Lessened effect on heart rate and blood pressure** vs. other MPH products
- Orphan drug designation potential
  - Fast-track eligible
  - Break-through designation eligible
- No generic equivalent and not substitutable; **solid IP through 2037** and potentially beyond

MOA[1] and Trial Data Suggest KP1077 is Well Positioned to Demonstrate Efficacy

- **Ritalin and Ritalin SR (racemic methylphenidate) are indicated to treat narcolepsy[2]**
  - *Methylphenidate-based products have demonstrated some efficacy in treating excessive daytime sleepiness associated with narcolepsy, a similar rare sleep disorder with the primary symptom of Excessive Daytime Sleepiness (EDS)*

- **Phase 1 trial results suggest SDX at higher-doses can produce the desired effects of wakefulness and a feeling of being energized in subjects with a history of high-dose stimulant use**
  - *Additional effects of hypervigilance and insomnia were also indicative of the potential to address excessive sleepiness*
  - *Trial data collected during AZSTARYS development provides further evidence of potential effects and pharmacokinetics*

Drowsiness/Alertness VAS[a]

(bipolar scale: 0 to 100)

Baseline Score Range:	**37 – 50**
Peak Score Range:	**67 - 89**

Energized VAS[b]

(unipolar scale: 0 to 100)

Baseline Score Range:	**15 - 20**
Peak Score Range:	**61 - 82**

Notes:

(a) Drowsiness/Alertness Visual Analogue Scale is an at-the-moment bipolar scale where a score of 50 is neither drowsy or alert, a score of 0 is Strong Drowsiness and a score of 100 is strong Alertness

(b) Energized Visual Analogue Scale (VAS) is an at-the-moment unipolar scale measuring the feeling of excess energy where a score of 0 is "definitely no" energy and 100 is "definitely so"

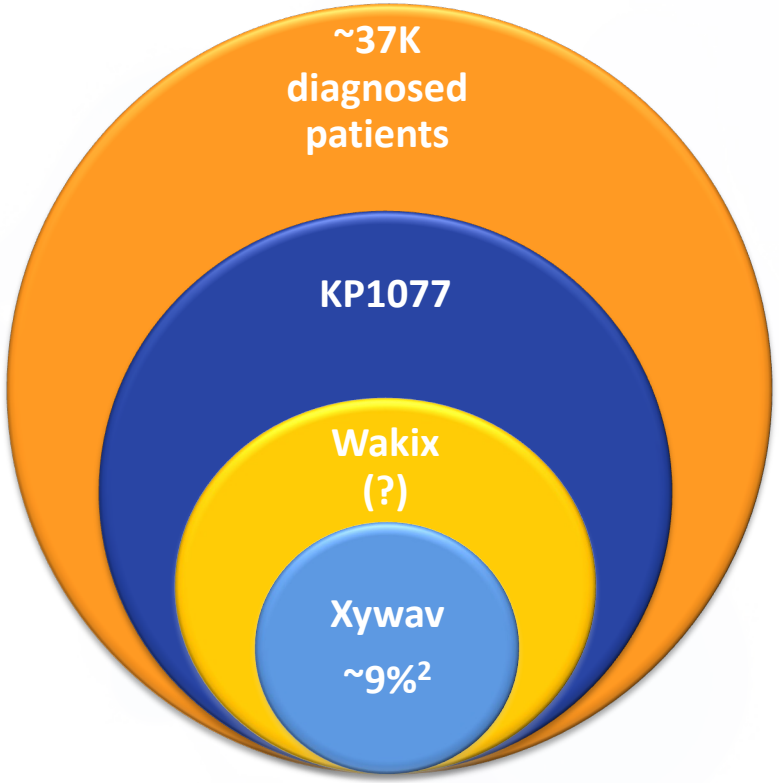
(1) Mechanism of action

(2) Ritalin Package Insert

KP1077 Could Capture a Large Share of the IH Market Based on Potential Clinical Differentiation and Combination Use

- It is estimated that ~37K patients are currently diagnosed with IH and actively seeking treatment[1]
- Xywav® received FDA approval in August 2021 as the first therapy for IH
- According to analysts, Xywav projected sales are ~$300 million for IH by the end of 2025
  - Assuming an average price of ~$94K per patient per year, IH patient share for Xywav by 2025 is expected to be ~3,200 patients (~9% of diagnosed patients)[2]
- Potential factors that may result in higher adoption of KP1077, compared to Xywav (approved) or Wakix® (in development):
  - Different MOA than Xywav suggests possible concomitant use; **75% of patients in Xywav IH trial also took stimulant medication**
  - **Improved efficacy of KP1077** due to increased tolerability vs. other stimulants
  - **KP1077 safety profile** related to C-IV, improved cardiovascular safety and lack of significant DDI potential
  - Xywav barriers to uptake including AEs, REMS and GHB stigma
  - Xywav promotion and disease awareness helps all marketed products
  - Wakix barriers to uptake related to DDI potential and different MOA

Illustrative Market Share based on Potential Differentiation



Sources: (1) https://www.sleepcountshcp.com/what-is-idiopathic-hypersomnia
(2) https://investor.jazzpharma.com/investors/events-presentations

Business Development Focus

- Maximizing Value Potential of SDX
- Pipeline Additions Through In-Licensing

Platform Potential of SDX

- The properties of SDX opens the door to explore indications outside ADHD and IH:
  - The **only C-IV methylphenidate-based product**; all others are C-II
  - Unique PK profile allowing for gradual and continuous release throughout the day
  - Currently, no generic equivalent for SDX and not substitutable
- SDX expected to provide benefit to patients with both Type I and II narcolepsy
  - Initiate clinical trial shortly after IH trial initiation
- Recent trial data suggest SDX has potential as a treatment option for Stimulant Use Disorder (SUD)
  - KP879 Phase 1 clinical trial data was compelling; scientific rationale still exists
  - Development program will be challenging and lengthy
  - Actively seeking partnership with government, academia and/or industry to advance
    - Engaged with top-tier firm to investigate and pursue various government grants/funding

Pipeline Expansion Strategy to Accelerate Value Creation

- Currently reviewing several earlier stage internal programs as potential lead candidates to add to the development pipeline
- External focus is primarily within the broad CNS/rare diseases space, with a particular focus on rare diseases or niche/specialty markets
- Actively seeking assets in Phase 2 stage or later, subject to our evaluation criteria, for in-licensing/acquisition
  - Assets need to fit within the framework of the business and current market dynamics

Due diligence on a host of assets takes time as we seek to:

1) Understand whether a potential molecule shows an efficacy signal for a specific indication
2) Understand the regulatory pathway (hurdles, time, cost, probability of success)
3) Understand commercial potential, competitive landscape, resources
4) Evaluate business structure and other dynamics that impact the transaction
5) Compare opportunities to assess best investment for capital allocation

AZSTARYS®

D-Methylphenidate Prodrug Product for the Treatment of ADHD

AZSTARYS® - U.S. Commercialization Moving Forward as Expected

- **Full national field team staffing in place; national rollout underway**
  - As of April 2022, the AZSTARYS field sales team will have doubled in size to 90 from 45 at year end 2021
    - Added approximately **700 new prescribers during the first 2 months of 2022**
    - Over 60 prescribers have written more than 30 prescriptions
    - Over **2,600 pharmacies** have dispensed AZSTARYS
  - **As of today, over 110 million commercial** lives have access to AZSTARYS
    - AZSTARYS has been added by more than 20 managed care organizations including **2 of the 3 largest PBM's**
- **AZSTARYS Commercial Launch is a Significant Opportunity for KemPharm**
  - License agreement with Commave, an affiliate of GPC, provides significant economic benefits to KemPharm tied to the commercialization of AZSTARYS
  - *Current growth trajectory makes the potential for earning sales milestones in 2022 attainable*

Source: (1) Estimates from Corium, Inc.

Financial Update

Q4 and FY 2021 Results; Financial Position is a Source of Strength

- Q4 2021 revenue of $2.6M and FY 2021 revenue of $28.7M, derived primarily from royalties and consulting service fees
- Q4 2021 net loss ($2.7M), or ($0.08) per share, and FY 2021 net loss attributable to common stockholders of ($62.9M), or ($2.11) per share
- Q4 2021 net operating loss was ($2.8M), and FY 2021 net operating income was $7.7M
- Looking ahead, R&D expense will increase in FY 2022 with the start of the KP1077 development program

- Balance sheet details as of Dec. 31, 2021:
  - Cash, and cash equivalents and long-term investments was $127.8M as of Dec 31, 2021
  - All debt was eliminated during 2021
  - $50M share repurchase program in place through 2023
  - Available cash, cash equivalents and long-term investments extends cash runway beyond 2025

Upcoming Clinical, Reg and BD Milestones Create Potential Near-Term Value

Milestone	Q1 2022	Q2 2022	Q3 2022	Q4 2022	Q1 2023	Q2 2023
KP1077 for IH						
Type B meeting with FDA	✓					
IND filing/may proceed		x				
Phase 1 CV differentiation trial		x	x			
Phase 2 trial			x			x
KP1077 for Narcolepsy						
Type B meeting with FDA			x			
IND filing				x		
Phase 2/3 trial initiation				x		
KP879						
Final trial results	✓					
Additional clinical stage candidate(s)						

*Note: "X" denotes an event, **blue** box denotes activity timeframe*

KemPharm: Development Activity Ramping Up in FY 2022

- ✓ **IND filing as early as Q2 2022**
- ✓ Phase 2 trial initiation in 2H 2022
- ✓ Cardiovascular trial initiation Q2 2022; Data Q3 2022

KP1077 Development Program

Pipeline Expansion Well Underway

- ✓ Continuing efforts to build a highly differentiated pipeline of development assets
- ✓ Focused on high-value areas with significant unmet needs in **CNS/rare disease** with potential to internally commercialize
- ✓ Internal product candidate advancing; potential announcement as early as Q2 2022

- ✓ **Full national team in place**
- ✓ 110M+ covered commercial lives
- ✓ Expanded launch of AZSTARYS supports revenue potential from royalties and milestones in 2022

AZSTARYS® Now Launched Nationally

Strong Balance Sheet to Support Value Creation

- ✓ Cash, cash equivalents and long-term investments of **$127.8M** as of Dec 31, 2021
- ✓ Solid balance sheet supports development efforts and other pipeline expansion activities
- ✓ **Available capital extends cash runway beyond 2025**

KemPharm INC

Leveraging our LAT® Prodrug Technology to Create Long-Term Value

For additional information please contact:
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