



# Aquestive Therapeutics Corporate Presentation

December 2019

Advancing medicines.  
Solving problems.  
Improving lives.



# Forward Looking Statement

Certain statements in this presentation and associated oral statements made by management may constitute "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as "believe," "anticipate," "plan," "expect," "estimate," "intend," "may," "will," or the negative of those terms, and similar expressions, are intended to identify forward-looking statements. These forward-looking statements may include, but are not limited to, statements about our growth and future financial and operating results and financial position, ability to advance Libervant to the market, regulatory approvals and pathways, clinical trial timing and plans, short-term and long-term liquidity and cash requirements, cash funding and cash burn, business strategies, market opportunities, and other statements that are not historical facts.

These forward-looking statements are based on our current expectations and beliefs and are subject to a number of risks and uncertainties that could cause actual results to differ materially from those described in the forward-looking statements. Such risks and uncertainties include, but are not limited to, risks associated with the Company's development work, including any delays or changes to the timing, cost and success of our product development activities and clinical trials; the risks of delays in FDA approval of our drug candidates or failure to receive approval; the risks inherent in commercializing a new product (including technology risks, financial risks, market risks and implementation risks and regulatory limitations); risk of development of our sales and marketing capabilities; risk of legal costs associated with and the outcome of our patent litigation challenging third party at risk generic sale of our proprietary products; risk of sufficient capital and cash resources, including access to available debt and equity financing and revenues from operations, to satisfy all of our short-term and longer term cash requirements and other cash needs, at the times and in the amounts needed; risk of failure to satisfy all financial and other debt covenants and of any default; risk related to government claims against Indivior for which we license, manufacture and sell Suboxone and which accounts for the substantial part of our current operating revenues; risks related to the outsourcing of certain sales, marketing and other operational and staff functions to third parties; risk of the rate and degree of market acceptance of our products and product candidates; the success of any competing products, including generics; risk of the size and growth of our product markets; risk of the effectiveness and safety of our products and product candidates; risk of compliance with all FDA and other governmental and customer requirements for our manufacturing facilities; risks associated with intellectual property rights and infringement claims relating to the Company's products; risk of unexpected patent developments; the impact of existing and future legislation and regulatory provisions on product exclusivity; legislation or regulatory action affecting pharmaceutical product pricing, reimbursement or access; claims and concerns that may arise regarding the safety or efficacy of the Company's products and product candidates; risk of loss of significant customers; risks related to legal proceedings, including patent infringement, investigative and antitrust litigation matters; changes in governmental laws and regulations; risk of product recalls and withdrawals; uncertainties related to general economic, political, business, industry, regulatory and market conditions and other unusual items; and other risks and uncertainties affecting the Company including those described in the "Risk Factors" section and in other sections included in the Company's Annual Report on Form 10-K filed with the SEC on March 14, 2019 and in our quarterly reports on Form 10-Q.

Given these uncertainties, you should not place undue reliance on these forward-looking statements, which speak only as of the date made. All subsequent forward-looking statements attributable to us or any person acting on our behalf are expressly qualified in their entirety by this cautionary statement. The Company assumes no obligation to update forward-looking statements or outlook or guidance after the date of this press release whether as a result of new information, future events or otherwise, except as may be required by applicable law.

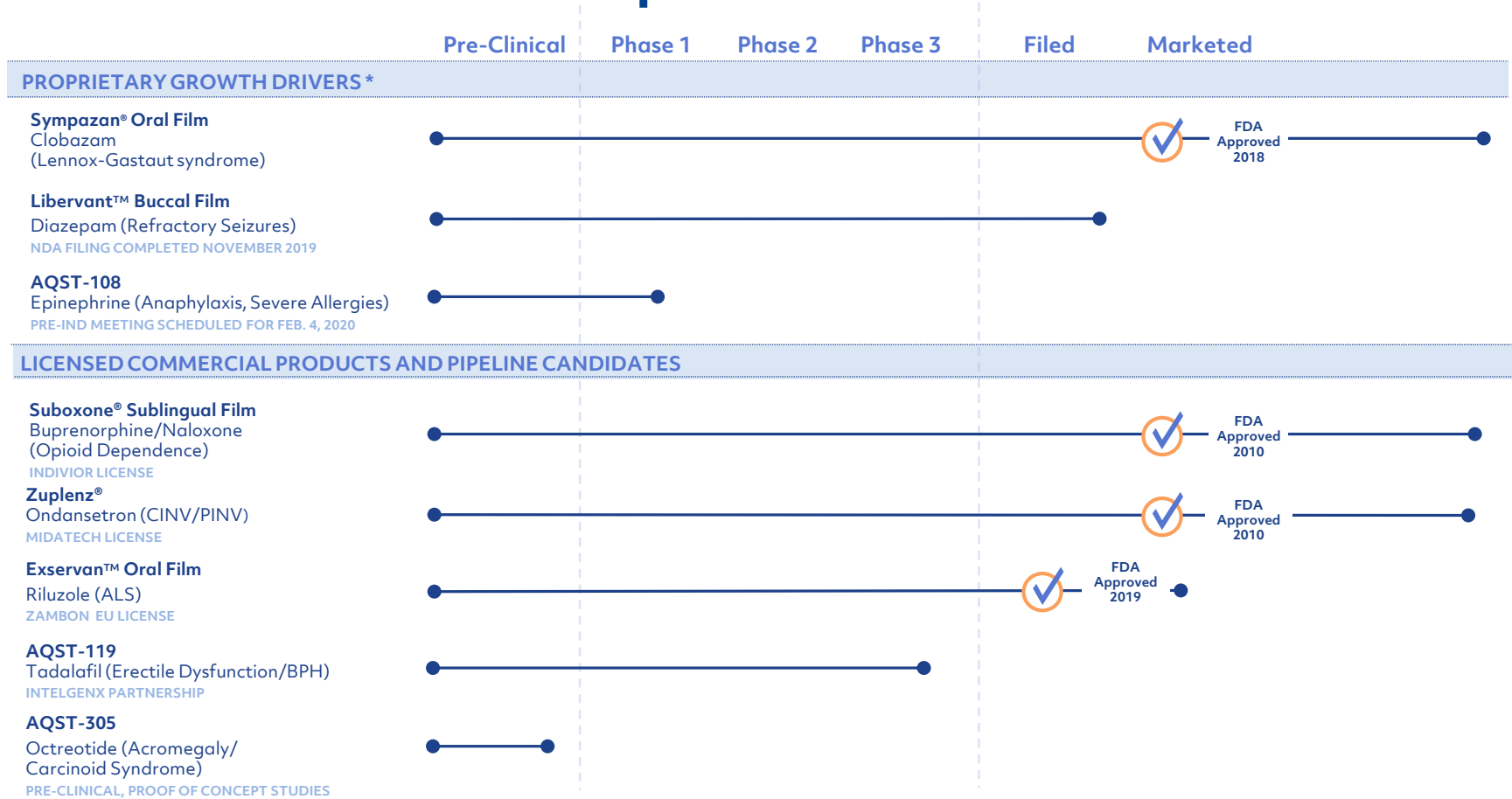
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# Corporate Highlights

- ▶ **Commercial-stage, specialty pharmaceutical company** with comprehensive capabilities to advance medicines from pipeline to market
- ▶ **Advancing a late-stage pipeline that** features promising treatments for patients and caregivers living with complex conditions, including hard to manage epilepsies and anaphylaxis
- ▶ **Launched first proprietary product in epilepsy franchise** and established commercialization infrastructure for engaging high-volume prescribers - 90% overlap with most advanced product candidate
- ▶ **Valuable intellectual property portfolio** with 200+ worldwide patents and >90 additional patents pending that offer protection through 2037
- ▶ **Ongoing collaborations provide continuing revenue** and options for non-dilutive capital



# Robust Portfolio & Pipeline



# PharmFilm Delivers Meaningful Differentiation

Diastat  
rectal gel



Clobazam



Epinephrine  
injection



VS



- ▶ Can deliver rapid onset of action with entry into systemic circulation
- ▶ Ease of administration
- ▶ Demonstrated bioequivalence, safety and tolerability
- ▶ Non-invasive



# Our Medicines

Advancing medicines.  
Solving problems.  
Improving lives.



Caregivers struggle with complex treatment regimens and seek effective, simple solutions to reduce seizures and ease the daily burdens of treatment

## Solving Problems In EPILEPSY

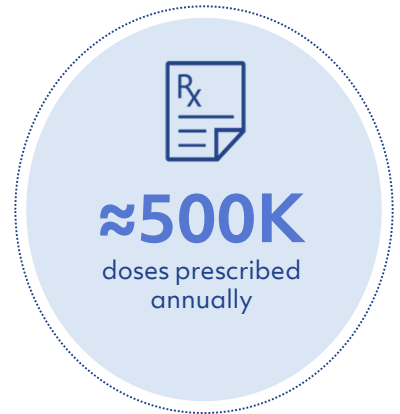
**Lennox-Gastaut Syndrome (LGS)** : A rare, severe, and intractable form of epilepsy involving daily seizures of multiple types that often result in severe cognitive impairment and developmental delays<sup>1</sup>

**50K** Of nearly  
3.4M patients  
with epilepsy,  
have seizures  
related to **LGS**

**30<sup>to</sup> 40%**  
of people with LGS  
have **dysphagia**<sup>2</sup>

### Suboptimal Treatment ONFI® Oral Benzodiazepine

- Previously available only in tablet and oral suspension (brand and generic)
- Crushing, mixing, and measuring these forms of clobazam increases caregiver burden and can lead to sub-optimal dosing<sup>3</sup>



# Sympazan Overview and Launch Update



## Sympazan (clobazam) oral film

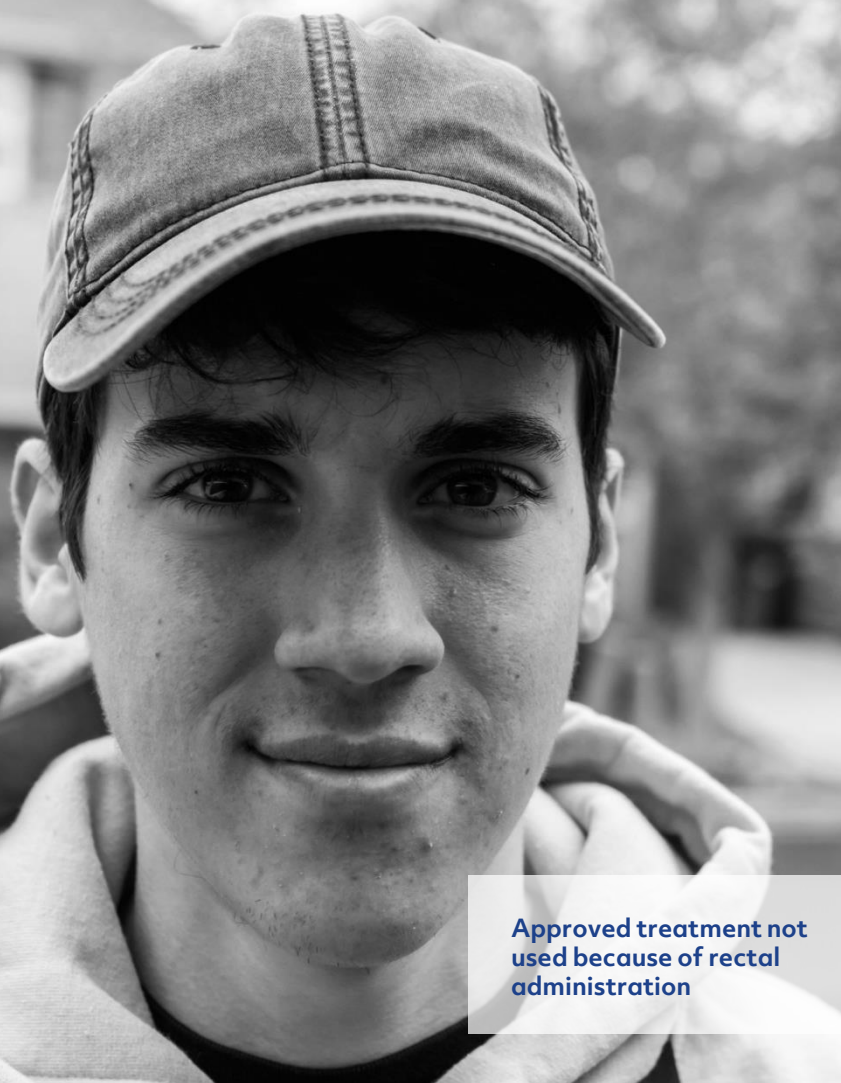
- Approved in November 2018 and launched in December 2018
- First and only film approved for treatment of seizures associated with LGS
- Offered in 5 mg, 10 mg, and 20 mg dosages
- Team of 50+ experienced commercial professionals advancing discussions with payers and healthcare providers
  - 90% overlap with Libervant high-volume prescriber base

## Launch Update

- Shipments from wholesalers to retailers grown by over 50% since end of 2Q19
- Prescriber base increased by over 56% since end of 2Q19
- Tracking in line with expectations to generate ~\$65 million in net revenue at peak\*
- On pace to achieve 70% covered commercial lives by year end 2019







**Approved treatment not  
used because of rectal  
administration**

## Solving Problems In **EPILEPSY**

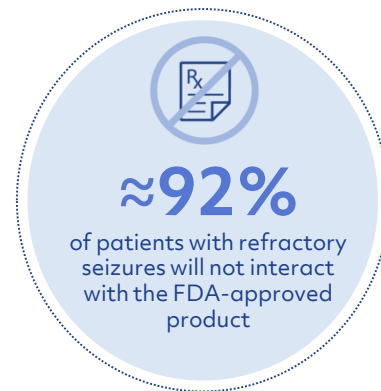
**Refractory seizures:** The failure of 2 or more AED regimens to achieve sustained freedom of seizures resulting in comorbid illnesses, psychological dysfunction, social stigma, and increased risk of mortality<sup>1-3</sup>

**1M** Epilepsy  
patient visits<sup>4</sup>  
to **EMERGENCY  
DEPARTMENTS**  
annually

**1.2M** epilepsy  
patients<sup>5</sup>  
suffer from uncontrolled,  
**refractory  
seizures**

### **Suboptimal Treatment** *Diazepam Rectal Gel*

- 14-step, rectal administration<sup>6</sup>
- Length of time to administer
- Potential for inaccurate dosing



# Libervant™ (diazepam) Buccal Film Overview

## Overview

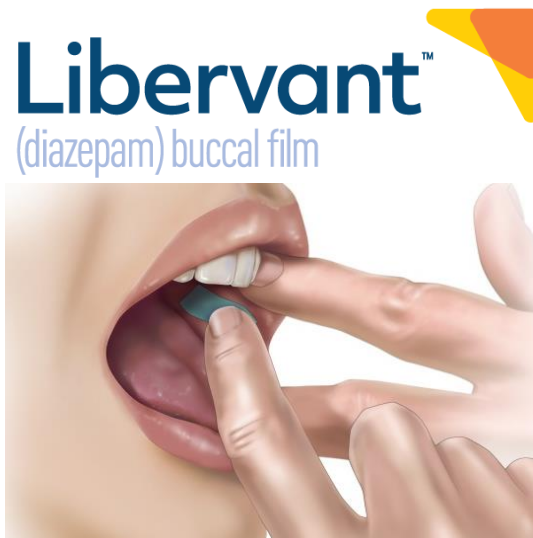
- In development for management of select patients with refractory epilepsy who require treatment to control episodes of increased seizure activity, or “seizure clusters”
- Potential to become the preferred rescue medication by patients and providers looking for clinically differentiated treatment in a preferred dosage form

## Pre-NDA meeting held late December 2018

- Agreed to conduct single dose crossover study of DBF versus Diastat – completed in July 2019
- Provided a clear path to regulatory filing and approval

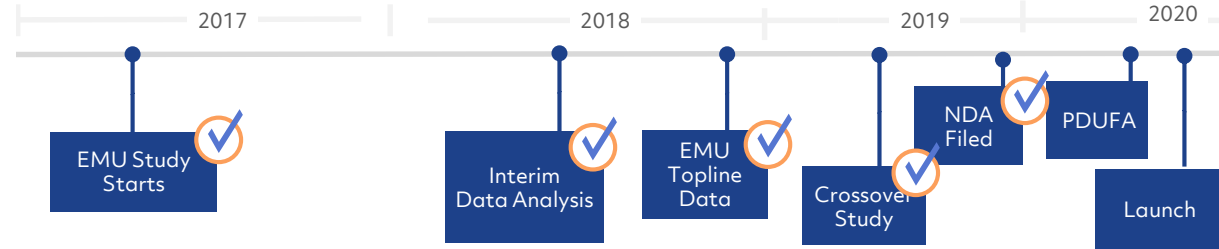
## Rolling NDA Submission completed in late November 2019

- First section submitted in May 2019
- Second section submitted in September 2019
- Third section submitted in November 2019





# Pivotal Trial Design and Expected Timing



Study	Patient Group	Primary Objectives	Secondary Objectives
<b>Dose Proportionality Study (#162013)</b>	Healthy adults	<ul style="list-style-type: none"> <li>Demonstrate dose proportional plasma levels for 5, 10 and 15mg doses</li> </ul>	
<b>Pivotal Bioavailability Study (#162021)</b>	Healthy adults	<ul style="list-style-type: none"> <li>Compare pharmacokinetics and bioavailability in healthy subjects to reference product (diazepam rectal gel)</li> </ul>	
<b>Crossover Study</b>	Adult patients	<ul style="list-style-type: none"> <li>Compare pharmacokinetics after administration of Libervant or Diastat in the same patients under real world conditions (on current, stable AED regimen, outside EMU, in fed state)</li> </ul>	
<b>Adult EMU Study (#160326)</b>	Adult patients with epilepsy (n=30)	<ul style="list-style-type: none"> <li>Compare the pharmacokinetics and bioavailability in subjects with epilepsy in interictal condition, when they are not experiencing seizures, versus the ictal/per-ictal condition, when they are experiencing seizures</li> </ul>	<ul style="list-style-type: none"> <li>Evaluate safety following single-dose administration</li> </ul>
<b>Pediatric EMU Study (#160325)</b>	Pediatric patients with epilepsy (n=16)		<ul style="list-style-type: none"> <li>Evaluate usability in the interictal and ictal/per-ictal conditions</li> </ul>
<b>Safety Study (#42-1703)</b>	Children, adolescents and adults with refractory seizures (n=100)	<ul style="list-style-type: none"> <li>Safety and tolerability of chronic intermittent use of diazepam buccal film assessed by examination for any pathological changes in the oral mucosa and gustatory changes</li> </ul>	<ul style="list-style-type: none"> <li>General safety-tolerability</li> <li>Evaluate usability, including quality of life measures</li> </ul>

Additional clinical trials include a food effect, phase one study, and label comprehension study

# Libervant™ Crossover Study

## Trial Design

- Single dose crossover study
- Compare diazepam plasma concentrations from a dose of Libervant and its reference listed drug, Diastat® Rectal Gel, in the same patient population
- Subjects dosed in accordance with body weight into one of four weight categories
- 28 of 31 patients included in primary analysis (stable on concurrent anti-epileptic medications)

## Observations

- Confirmed dosing algorithm
- Non responders:
  - 3 patients failed to achieve therapeutic concentrations using rectal gel - but did achieve a response from film

## Met Co-Primary Endpoints Based on Preliminary Analysis

- Diazepam exposure following buccal film showed comparable bioavailability to rectal gel as assessed by maximal plasma concentration ( $C_{max}$ )
- Bioavailability of diazepam administered as buccal film, assessed by Area Under the Curve (AUC), was the same or higher than rectal gel
- Time to maximal concentration ( $T_{max}$ ) of diazepam film administration in patients under fed conditions was comparable to the results from previous studies of healthy volunteers who were under fasting conditions



Living with severe allergies, or caring for someone who is, can lead to stress, anxiety, and negatively impact quality of life. Interest in new treatment options is high.

## Solving Problems In **ANAPHYLAXIS**

*A systemic hypersensitivity reaction that can be rapidly and unpredictably fatal (drug, food, insect venom)<sup>1</sup>*

Affects up to  
**2% of U.S.  
population**<sup>2</sup>

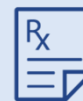
Increases  
**Emergency  
Department**  
and hospital visits

Approximately  
**186<sup>to</sup>225**  
deaths per year<sup>3</sup>

### Suboptimal Treatment

*EpiPen®*

- Difficult administration
- Inaccurate dosing
- Painful intramuscular injections
- Inconvenient portability

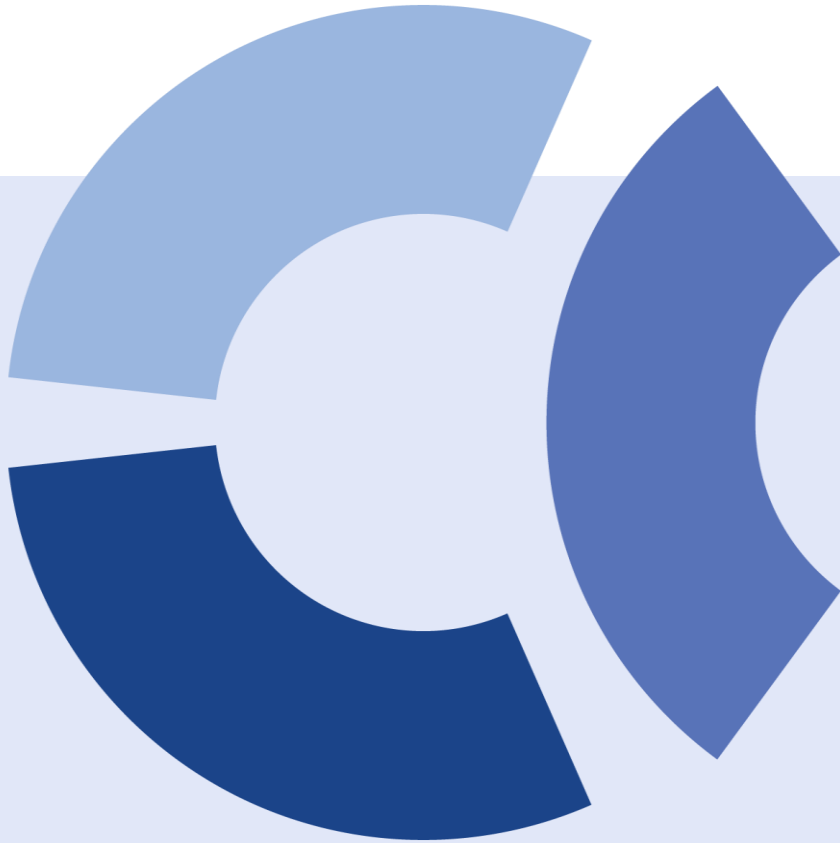


**≈\$1.7B** and  
more than **3.5M** total  
prescriptions

# AQST-108 Proof of Concept Study

## AQST-108 Overview

- Sublingual film formulation of epinephrine for the treatment of anaphylaxis and severe allergic reactions
  - Re-formulated and more advanced prototype developed after first human proof concept trials
- Phase 1 dose escalation proof-of-concept study in healthy subjects demonstrated ability to deliver systemic epinephrine using proprietary PharmFilm formulation
  - Submitted pre-IND meeting request to FDA in early November
  - Pre-IND meeting scheduled for February 4, 2020



# **Financials, Team & Upcoming Milestones**

Advancing medicines.  
Solving problems.  
Improving lives.

# Financial Summary

## Third Quarter 2019 Results

- Total revenues of \$12.4 million
  - Manufacturing revenue of \$9.2 million with 72 million shipped product doses
- Adjusted non-GAAP gross margin of 68%
- Adjusted EBITDA loss of \$8.4 million
- Net loss of \$18.4 million, or \$0.74 loss per share
- Cash and cash equivalent of \$20.9 million at 9/30/19

## Full Year 2019 Guidance\*

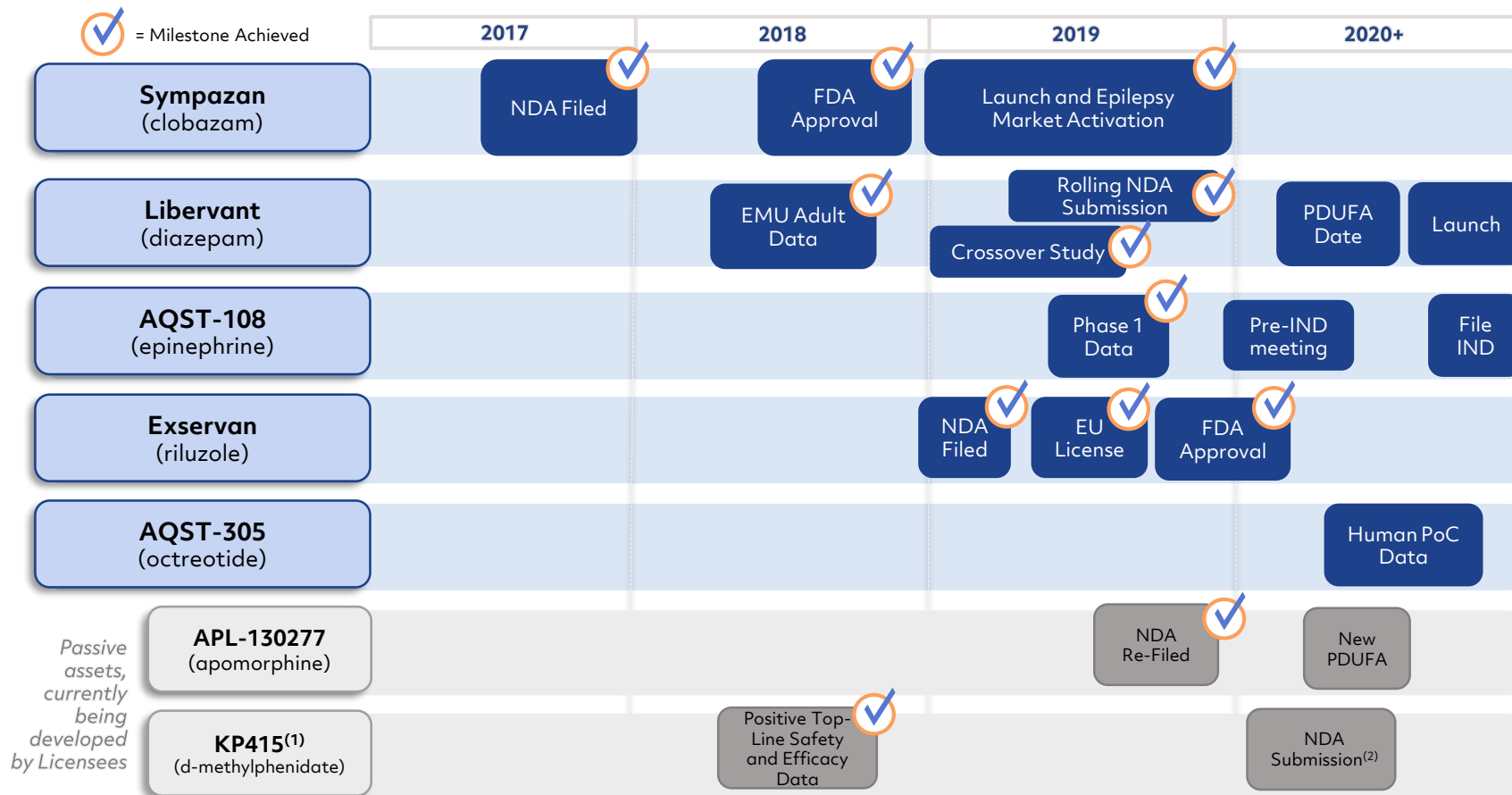
- Total revenues of \$45 to \$47 million
- Non-GAAP gross margins of 67% to 69%
- Non-GAAP EBITDA loss of \$49 to \$50 million, excluding non-cash stock compensation expenses
- Cash burn rate of \$60 to \$65 million

**Completed debt refinancing in July 2019, adding \$15 million new capital and opportunity for additional \$30 million**

**\$150 million universal shelf effective in September 2019, including \$25 million ATM facility**



# Multiple Upcoming Near-Term Catalysts



## CORPORATE INFORMATION, PharmFilm® technology, SYMPAZAN®, LIBERVANT™ AND EPINEPHRINE DATA

- Data on file

### LENNOX-GASTAUT SYNDROME (LGS) ( SLIDE 7)

1. LGS Foundation. About Lennox-Gastaut Syndrome. Available at: <http://www.lgsfoundation.org/aboutlgs>. Accessed October 11, 2018.
2. Ogawa K, Kanemoto K, Ishii Y, Koyama M, Sirasaka Y, Kawasaki J, Yamasaki S. Long-term follow-up study of Lennox-Gastaut syndrome in patients with severe motor and intellectual disabilities: with special reference to the problem of dysphagia. *Seizure*. 2001; 10:197-202
3. Scarpa M, Stegemann S, Hsiao WK, et al. Oral dispersible films: Towards drug delivery in special populations. *Int J Pharm*. 2017;523(1):327-335.
4. Heller AH, Wargacki S, Jung C, Wyatt DJ, Schobel AM. Pharmacokinetics of clobazam oral soluble film. Poster presented at the American Society for Experimental Neurotherapeutics (ASENT) 20th Annual Meeting; March 7-10, 2018; Rockville, MD.
5. Integrated Clinical and Statistical Report. A pivotal, open-label, randomized, single-dose, four-period, four-arm, crossover, comparative bioavailability study of clobazam 20 mg and 10 mg oral films and ONFI® 20 mg and 10 mg tablets in healthy male and female volunteers under fasting conditions. August 2017
6. ONFI [package insert]. Deerfield, IL: Lundbeck; 2011

### REFRACTORY SEIZURES ( SLIDE 9)

1. Marawar R, Basha M, Mahulikar A, Desai A, Suchdev K, Shah A. Updates in Refractory Status Epilepticus. *Crit Care Res Pract*. 2018;2018:9768949. Published 2018 May 8. doi:10.1155/2018/9768949
2. Sperling MR. Sudden Unexplained Death in Epilepsy. *Epilepsy Curr*. 2001;1(1):21-23.
3. Claassen J., Goldstein J. N. Emergency neurological life support: status epilepticus. 2017;27(1):152-158. doi: 10.1007/s12028-017-0460-1
4. Seizure visits to ED: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC2657249/>
5. Laxer, K. et al, The consequences of Refractory Epilepsy and its treatment; *Epilepsy & Behavior*; Vol 37, Aug 2014, Pgs 59-70; <https://doi.org/10.1016/j.yebeh.2014.05.031>
6. Diastat administration and disposal instructions [www.diastat.com](http://www.diastat.com)

### ANAPHYLAXIS ( SLIDE 13)

1. Fischer D, Vander Leek TK, Ellis AK, Kim H. Anaphylaxis. *Allergy Asthma Clin Immunol*. 2018;14(Suppl 2):54. Published 2018 Sep 12. doi:10.1186/s13223-018-0283-4
2. WoNUMBER 2
3. od, R., Camargo, et al Anaphylaxis in America: The prevalence and characteristics of anaphylaxis in the United States. *J ALLERGY CLIN IMMUNOL VOLUME 133*, Ma L, Danoff TM, Borish L. Case fatality and population mortality associated with anaphylaxis in the United States. *J Allergy Clin Immunol*. 2013;133(4):1075-83. doi: 10.1016/j.jaci.2013.10.029