



# **Corporate Overview**

April 2019

# **Forward-Looking Statements**

This presentation includes, and our response to various questions may include, forward-looking statements. All statements contained in this presentation other than statements of historical facts, including statements regarding our future results of operations and financial position, our business strategy and plans and our objectives for future operations, are forward-looking statements. The words "anticipate," "believe," "continue," "estimate," "expect," "intend," "may," "will" and similar expressions are intended to identify forward-looking statements. We have based these forward-looking statements largely on our current expectations and projections about future events and financial trends that we believe may affect our financial condition, results of operations, business strategy, short-term and long-term business operations and objectives and financial needs. These forward-looking statements, including statements regarding clinical trials, clinical studies and other clinical work (including the funding therefor, anticipated patient enrollment, safety data, study data, trial outcomes, timing or associated costs), regulatory applications and related timelines, including the filing of an NDA for LIQ861, are subject to a number of risks, uncertainties and assumptions. Moreover, we operate in a very competitive and rapidly changing environment and our industry has inherent risks. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the future events and trends discussed in this presentation may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance, achievements or events and circumstances reflected in the forward-looking statements will occur. We are under no duty to update any of these forward-looking statements after the date of this presentation to conform these statements to actual results or revised expectations, except as required by law. This presentation also contains estimates and other statistical data made by independent parties and by us relating to market size and growth 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. This presentation includes long-term goals that are forward-looking, are subject to significant business, economic, regulatory and competitive uncertainties and contingencies, many of which are beyond the control of us and our management, and are based upon assumptions with respect to future decisions, which are subject to change. Actual results will vary and those variations may be material. Nothing in this presentation should be regarded as a representation by any person that these goals will be achieved and we undertake no duty to update our goals.

#### **Disclaimers**

Liquidia Technologies, Inc. (the "Company) has filed a Registration Statement on Form S-1 (File No. 333-230362) (the "Registration Statement"), as amended, with the Securities and Exchange Commission ("SEC") in connection with the offering to which this presentation relates. Before you invest, you should read the Registration Statement, the preliminary prospectus included within the Registration Statement including the risk factors set forth therein, and other documents the Company has filed with the SEC for more complete information about the Company and this offering. You can obtain these documents for free by visiting EDGAR on the SEC website at www.sec.gov. Alternately, copies of the preliminary prospectus may be obtained by contacting Jefferies LLC, Attention: Equity Syndicate Prospectus Department, 520 Madison Avenue, 2nd Floor, New York, NY 10022, by telephone at (877) 547-6340, or by email at prospectus\_department@Jefferies.com; Cowen and Company, LLC, c/o Broadridge Financial Solutions, Attention: Prospectus Department, 1155 Long Island Avenue, Edgewood, NY 11717 or by telephone at 631-274-2806.

Unless otherwise indicated, information contained in this presentation concerning our industry and the markets in which we operate is based on reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources as well as our own internal estimates and research. Decision Resources Group, the primary source for the market data included in this presentation, was commissioned by us to compile this information. Although we believe the data from these third-party sources is reliable, we have not independently verified any third-party information. In addition, projections, assumptions and estimates of the future performance of the industry in which we operate and our future performance are necessarily subject to uncertainty and risk due to a variety of factors. Such factors could cause results to differ materially from those expressed in the estimates made by the independent parties and by us.



# Novel products via precise control of drug particles

Late-stage clinical biopharmaceutical company focused on transforming the lives of patients

### Proprietary Benefits

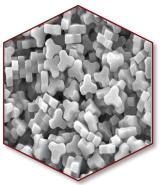
- Not limited by therapeutic area, molecule or route of delivery
- Fully scaled platform offers multiple product advantages



- LIQ861: inhaled dry powder targeting segment of PAH market (\$3.7B U.S.)
- Met primary endpoint in Phase 3 and will submit NDA late-2019



- LIQ865: to manage local, post-operative pain for 3-5 days (Phase 1)
- Poised to expand PRINT Technology advantages into future products



LIQ861



# Seasoned team with relevant commercial and disease area expertise













Neal Fowler

Tim Albury

Robert Lippe

Robert Roscigno, PhD

Ben Maynor, PhD

Jeri Thomas

Chief Executive Officer

Interim Chief Financial Officer

Chief Operations
Officer

Senior VP, Product Dev. Senior VP, R&D

Senior VP, Commercial

Management Employment History Highlights













of Johnson-Johnson



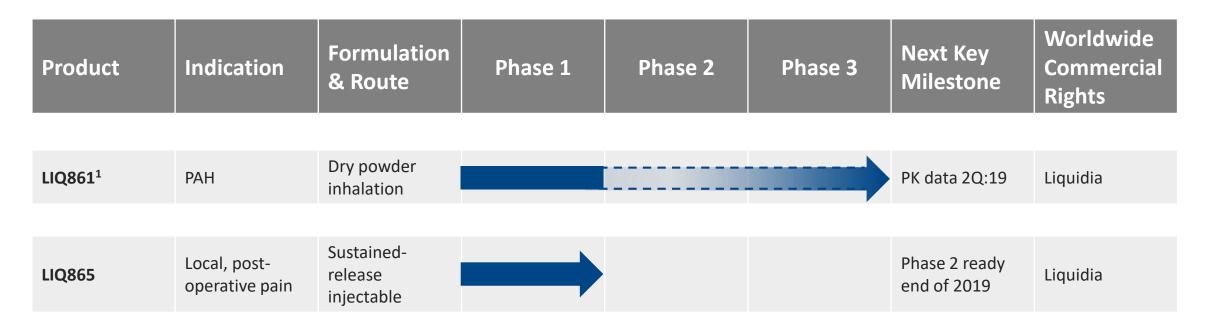








### **Pipeline**



1. After consultation with the FDA, we advanced from a Phase 1 trial directly to a pivotal Phase 3 trial and will seek approval under the 505(b)(2) pathway.

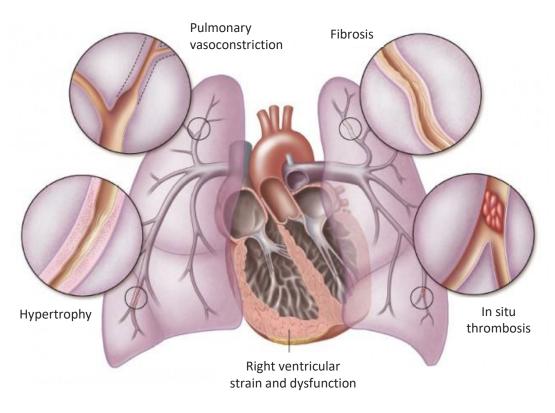


# LIQ861 for PAH

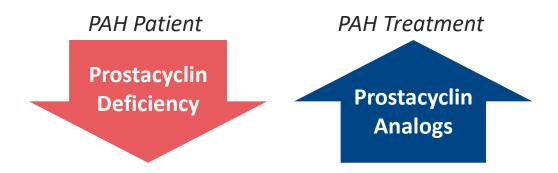
PRINT® treprostinil, dry powder inhalation

### PAH is a rare, progressive disease that results in right heart failure

Multiple pathways are involved in pathogenesis



- Prostacyclin is essential to normal lung function
  - Continually released by lungs to bind local receptors
  - Vasodilates the pulmonary arteries
  - Relaxes smooth muscle
  - Inhibits platelet aggregation



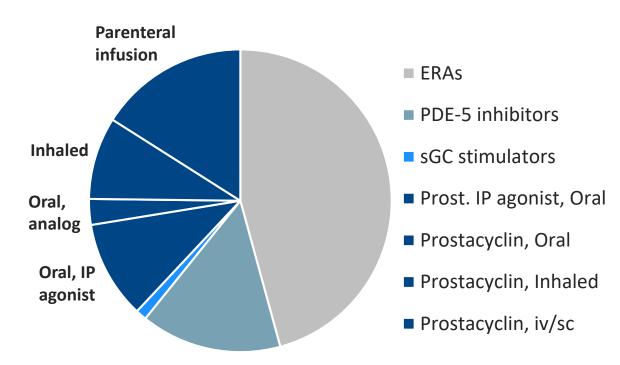
Abnormal changes in arteries of the lungs increase pressure in pulmonary arteries that leads to remodeling of the right ventricle

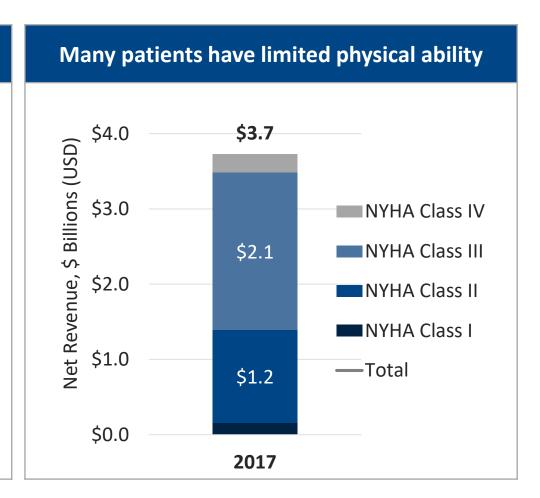
Goal of **prostacyclin therapy** is to **maximize a patient's exposure** to the highest tolerable level of drug



# U.S. market is reliant on prostacyclin products with ~\$1.4B in 2017

# Prostacyclin products are significant share of PAH market







Despite the success of prostacyclin products, the therapy has not been fully optimized



### Maximizing prostacyclin to directly deliver to the lungs is key

Local delivery generates fewer off-tissue effects



#### **Current prostacyclin products have clear tradeoffs**

Oral = Convenient, but with systemic toxicities and minimal symptom relief

- Increases side effects in GI, Nervous and Vascular systems
- Requires up-titration that can be challenging given side effects

**Nebulized** = Targeted, <u>but</u> *provides limited dose range* 

- Limits max dose due to throat irritation, adverse events
- Requires water, power, supplies, cleaning and time to dose

**Infusion** = Effective, but systemic toxicities & site pain, limits on lifestyle

- Delivers continuously via i.v. or s.c. line, 24 hours a day
- Poses potential for infection risk



# Choice of inhaled options is driven by convenience

Tyvaso® share was over 80% of the U.S. inhaled patient population in 2017





- 4x daily, titrated to target of 54 mcg/dose (9 breaths), the maximum recommended dose in label
- Most common AEs cough, headache, nausea, dizziness, flushing, throat irritation, pharyngolaryngeal pain, diarrhea
- Wash daily in warm soapy water (mouthpiece assembly and filter shells)
- Proprietary nebulizer + 13 additional accessories listed in patient starter kit



- 4-10 mins, 6-9x daily, titrated to target of 5 mcg/dose
- Most common AEs flushing, cough, headache, trismus, insomnia, nausea, hypotension, vomiting, alkaline phosphatase increased, flu syndrome, back pain, tongue pain, palpitations, syncope, GGT increased, muscle cramps, hemoptysis, pneumonia
- Wash after each use in warm soapy water & boil weekly
- Proprietary nebulizer + 10 additional spare parts listed in patient user guide



### **LIQ861** combines Effective + Targeted + Convenient into one product

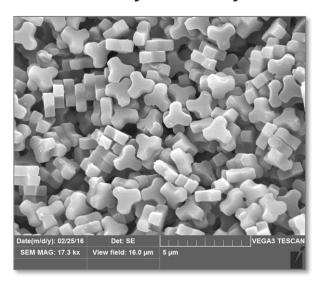
#### **Treprostinil = Proven efficacy**

Trusted prostacyclin-analog

**Proven compound** with FDA approvals for i.v., s.c., inhaled and oral routes

#### PRINT® = Deep-lung delivery

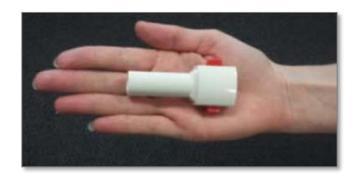
Precise Uniform Trefoil-like



Delivers higher dose levels than approved inhaled formulations

#### **Device = Simple, Disposable**

Disposable & long track record



RS00 Model 8 (DMF # 18418)

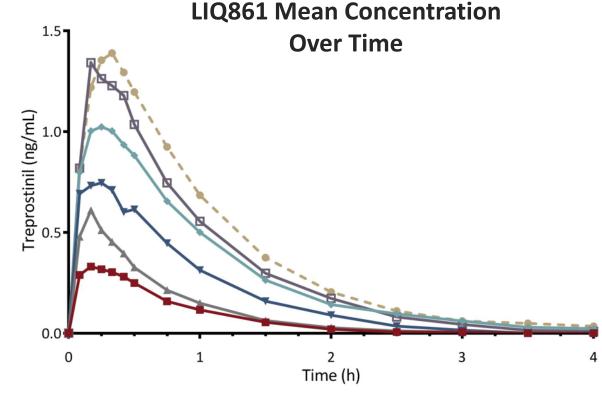
**Compact, easy inhaler** with established commercial track record



# LIQ861 well-tolerated in Ph1 with no reported SAEs, no MTD reached

Results supported moving directly to pivotal study

- n=57 healthy volunteers
- Single, ascending dose
- Dose proportional response
- No dose-limiting toxicities
- TEAEs related to treatment were mild
- No SAFs
- No MTD was reached



Approx. Capsule (TRE fill wt.)	🛨 25 μg	🛨 50 μg	<b>→</b> 75 μg	<table-cell-rows> 100 µg</table-cell-rows>	🖶 125 μg	🕶 150 μg
Approx. Emitted Dose (mcg)	20	40	60	80	100	120
Breaths	1-2	1-2	1-2	1-2	2-4	2-4



# After consulting FDA, initiated Phase 3 INSPIRE pursuant to 505(b)(2)

<u>In</u>vestigation of the <u>Safety and Pharmacology of Dry Powder Inhalation of Treprostinil</u>

Design	Open-label, U.S. multicenter		
Population	At least 100 WHO Group I (PAH) patients; NYHA Class II, III and IV		
Criteria	<ul> <li>On stable dose of Tyvaso® for ≥3 months (or) taking ≤2 approved non-PGI oral PAH therapies</li> </ul>		
Primary endpoint	Incidence of TEAEs and SAEs at 2 months		
Exploratory endpoints	6 minute walk distance (6MWD)		
	Sustained treatment transition (Tyvaso® transitions)		
	NYHA functional class improvement		
	<ul> <li>Quality of life using Minnesota Living with Heart Failure Questionnaire (MLHFQ)</li> </ul>		
PK Sub-Study <sup>1</sup>	Transitions from Tyvaso® in a one-directional crossover to compare bioavailability and PK		
Data collection	Baseline, Week 2, Month 1, Month 2 Visits, with bimonthly follow up for up to 30 months		



We intend to treat patients and collect data until U.S. launch



# **Enrollment suggests LIQ861 is attractive across disease severity**

Faster than expected enrollment driven primarily by interest from Functional Class II add-on patients

		No. Subjects (% of Study) at Month 2 timepoint*			
		LIQ861 Add-Ons (N=65)	Tyvaso <sup>®</sup> Transitions (N=44)	Overall (N=109)	
NYHA Functional Class at Screening	Class II	36 (55%)	36 (82%)	72 (66%)	
	Class III	29 (45%)	8 (18%)	37 (34%)	
Sustained Therapy at Month 2 <sup>^</sup>		59 (91%)	42 (95%)	101 (93%)	



Suggests that LIQ861 may have utility as a first-line prostacyclin



# LIQ861 met primary endpoint in pivotal Phase 3 INSPIRE study

TEAEs observed are consistent with inhaled prostacyclins

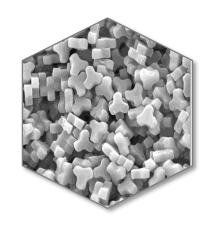
- No SAEs related to LIQ861
- TEAEs in ≥ 4% patients all mild to moderate
- Have not yet reached an MTD
  - At Month 2, dosed up to 150mcg capsule strength<sup>^</sup>
- 93% of patients completed 2-months\*
- Most TEAEs observed during first 2-weeks
- Most TEAEs in Add-On patients at 25mcg
- Positive trends in exploratory endpoints from initial data at Month 2

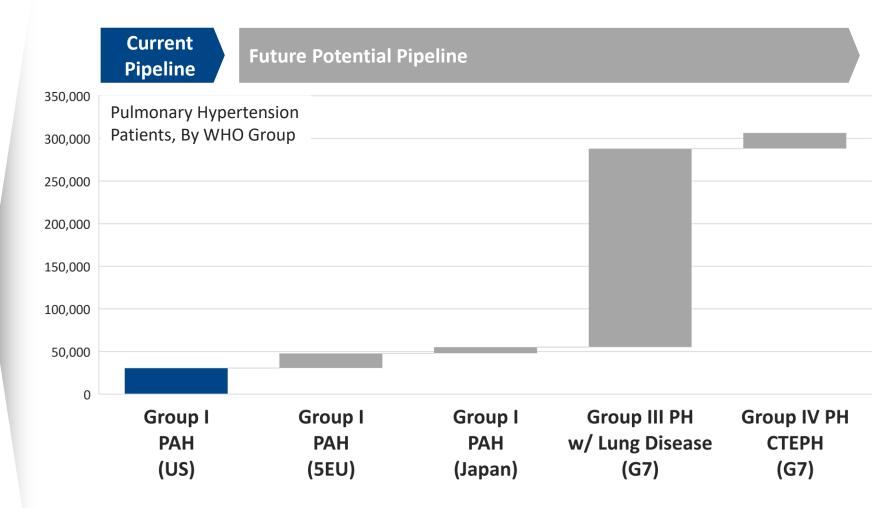
TEAEs at Month 2*	LIQ861 (tresprostinil)			
in ≥ 4% of Patients Receiving LIQ861	Add-ons (n=65)	Transitions (n=44)	All Treated (n=109)	
Cough	46.2%	13.6%	33.0%	
Headache	16.9%	20.5%	18.3%	
Throat irritation	16.9%	9.1%	13.8%	
Dizziness	10.8%	9.1%	10.1%	
Diarrhea	10.8%	4.5%	8.3%	
Oropharyngeal pain	7.7%	2.3%	5.5%	
Nausea	6.2%	4.5%	5.5%	
Dyspnea	4.6%	6.8%	5.5%	
Flushing	7.7%	2.3%	5.5%	
Chest discomfort	6.2%	2.3%	4.6%	



# LIQ861 = Pipeline in a PRINT® particle

Potential addressable PH patient populations over time







# LIQ865 for Local Post-Operative Pain

PRINT® bupivacaine, sustained-release injectable

# Significant unmet medical need for extended, non-opioid pain relief

- Approximately 50%+ of patients report inadequate local post-operative pain relief
- Reducing opioids is a priority for hospitals, payors and FDA
- Improved pain relief and reducing opioid use can drive key metrics, such as faster recovery and time to discharge
- Representing a \$761.1M market, local anesthetics have a known efficacy profile but are limited to 8 hours
- EXPAREL® demonstrates demand for longer acting relief, but too short
  - Physicians are seeking 3 to 5 days of pain relief, according to our market research
  - EXPAREL reportedly offers 24-36 hours in practice





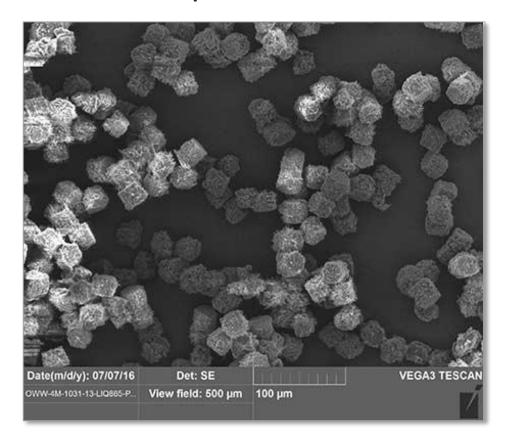




# LIQ865 offers the potential for an optimal product profile

- Target 3 to 5 days duration of action
  - Supported by PK & PD data from Ph 1 studies
- Simple, uniform particles of a single active
  - Easy reconstitution from a powder
- Flexible application at the surgical site
  - Adjustable concentration range to deliver the dose
  - Enables instillation or injection around incision
- Limited potential for dose dumping
  - Compatible with co-administration of instant-release local anesthetics

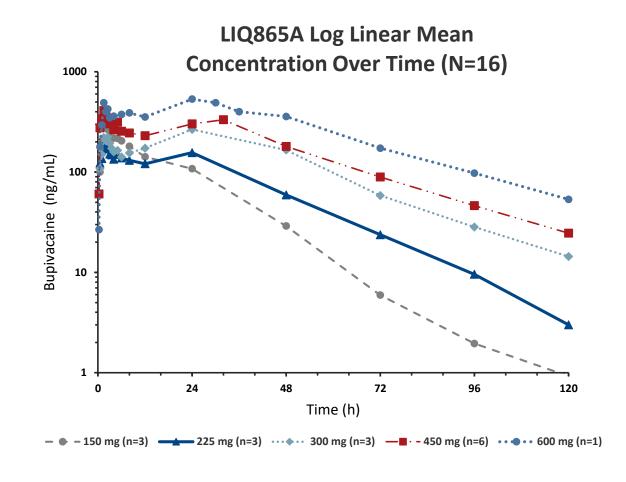
LIQ865: Bupivacaine + PLGA blend





## LIQ865 was well-tolerated at all doses with dose proportional PK in Ph1

- Ph1a, healthy volunteers in Denmark
- Single, ascending dose
- No dose-limiting toxicities
- All adverse events were mild to moderate
- C<sub>max</sub> well below reported thresholds for neurotoxicity and cardiotoxicity
- QST demonstrated pharmacodynamic effect for up to 5 days





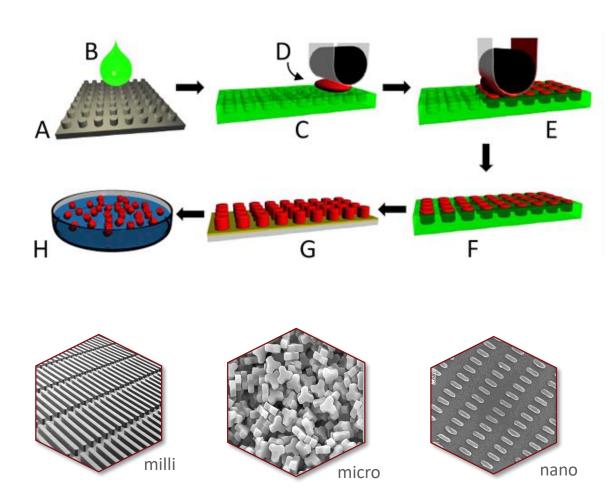
Expect to initiate Ph2-enabling tox studies in March 2019 with Ph2 trials planned for 2020





## Discrete particles through a molding process

Overview of PRINT® Technology



- **Step A:** Etch master template with 3D geometric structures of the desired particle size and shape
- Step B: Apply our proprietary polymeric mold material over master template
- Step C: Cure polymeric material to form PRINT molds with discrete molding cavities that replicate structures of master template
- **Step D:** Design chemical composition of drug particle
- Step E: Apply the drug particle composition to the cavities in the mold to fill the cavities
- **Step F:** Form the drug particles in cavities of the mold
- Step G: Remove drug particles from mold cavities on a harvesting film
- Step H: Remove particles from harvesting film



# PRINT® production technology is highly capable and widely applicable

**Preclinical and R&D** *Highly versatile, flexible* 



Lab Line 2 (2008)

- Highly agile platform enabling process experimentation
- Ideal for early stage process development

cGMP Process Development
Optimization, scale-up



Lab Line 3 (non-cGMP 2015; cGMP 2017)

- Capable of larger batches with increased process control
- We believe Lab Line 3 is fully cGMP compliant to support product launch

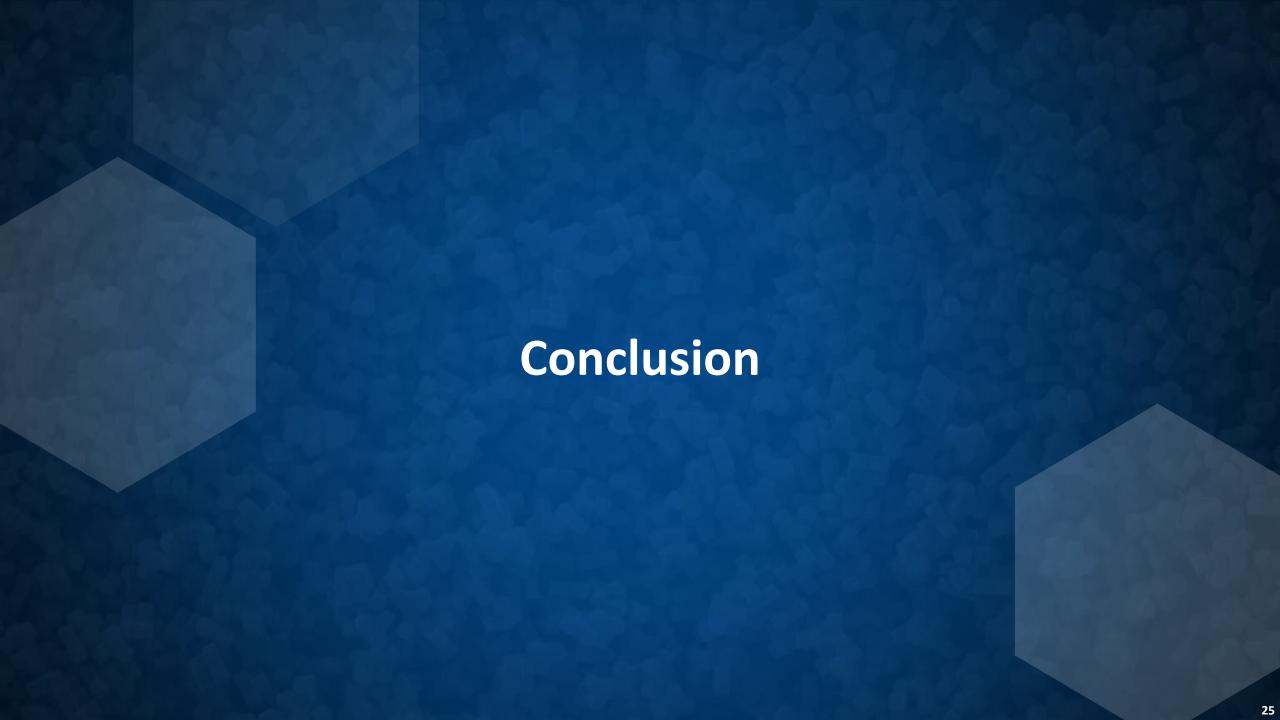
cGMP Production
Repeatable and deployable



#### Commercial Line 1 (expected 2019)

- Optimized drug substance production process
- Designed for continued market supply and scale





# **Anticipated Upcoming Milestones**

Milestone	Anticipated Timing	
Report LIQ861 Ph 3 two-week safety data from INSPIRE trial	1Q:2019	<b>√</b>
Report LIQ861 Ph 3 primary endpoint from INSPIRE trial	1Q:2019	<b>√</b>
Initiate LIQ865 Ph 2-enabling tox studies	March 2019	$\checkmark$
Report LIQ861 PK results	2Q:2019	
NDA submission to the FDA for LIQ861	Late 2019	







**Thank You**