



Corporate Overview

March 2020

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 and the defense and approval of the NDA, if filed, 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.

Novel products via precise control of drug particles

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

Supporting NDA

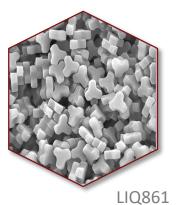
- LIQ861: inhaled dry powder targeting segment of PAH market in U.S.
- Submitted to FDA in January under 505(b)(2) regulatory pathway
- Retain worldwide commercial rights

Pipeline Growth

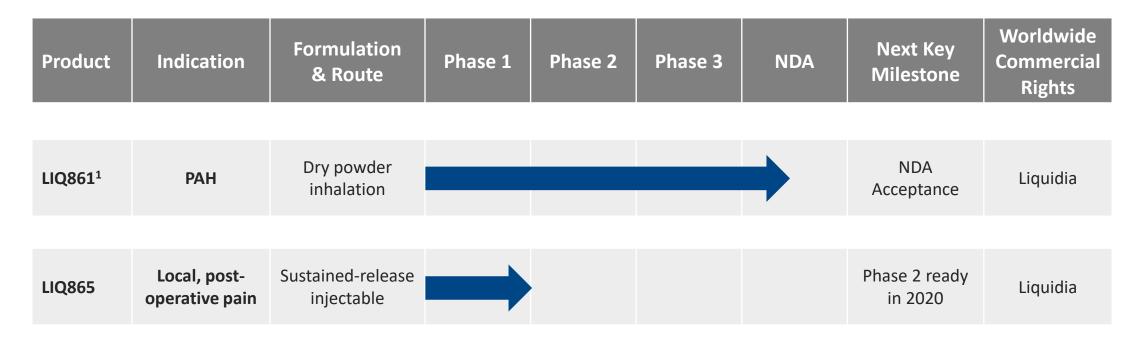
- LIQ865: local, post-operative pain relief for 3-5 days; completed Phase 1
- Poised to expand PRINT Technology advantages into future products



- Broadly applicable across therapeutic areas, modalities and routes of delivery
- Fully scaled PRINT® platform offers multiple product advantages



Pipeline



¹ After consultation with 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 can conveniently maximize treprostinil delivery directly to lungs

Fewer systemic toxicities than oral or parenteral administration

Market

- ~30,000 WHO Group I (PAH) patients diagnosed and treated
- ~\$1.4B+ of drugs in the prostacyclin deficient pathway, mostly in delivery of treprostinil
- Nebulized treprostinil generated \$415.6M in net sales for 2019

LIQ861 Profile

- First DPI treprostinil designed to enhance deep-lung delivery using convenient device
- Potential to optimize treprostinil therapy by dosing to patient benefit versus tolerability
- Potential to delay transition to more invasive therapies based on higher tolerated dose

Business Case

- Attractive costs of production with fully scaled PRINT® manufacturing
- Efficient commercial effort addressable with targeted sales force in rare disease
- Protected sales with IP position into 2030's
- Upside potential by expanding use outside of WHO Group I (PAH)



LIQ861 for PAH

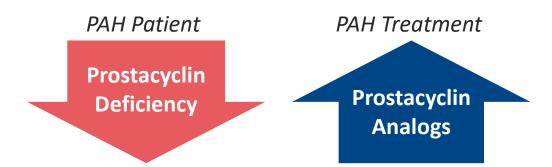
PRINT® treprostinil, dry powder inhalation

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

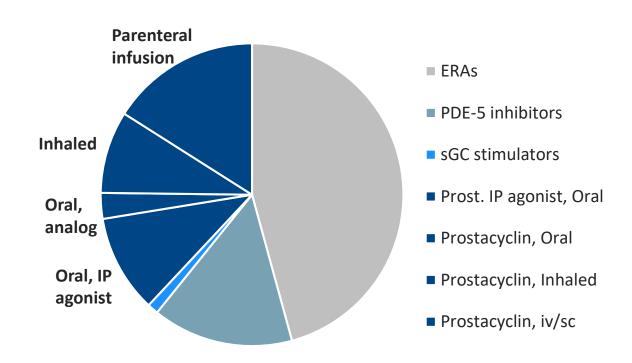
Abnormal changes in arteries of the lungs increase pressure in pulmonary arteries

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



\$1.4B of \$3.7B market in prostacyclin pathway in 2017





Goal of prostacyclin therapy is to maximize exposure to highest tolerable level

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, but 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

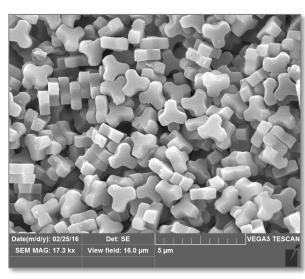
Treprostinil = Proven efficacy

Trusted prostacyclin-analog

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

PRINT® = Deep-lung delivery

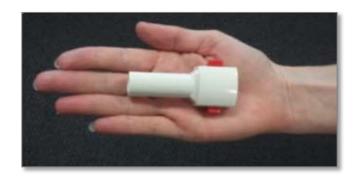
Precise, Uniform, Trefoil



Delivers higher dose levels than approved inhaled formulations

Device = Simple, Disposable

Disposable & long track record



RS00 Model 8 (DMF # 18418)

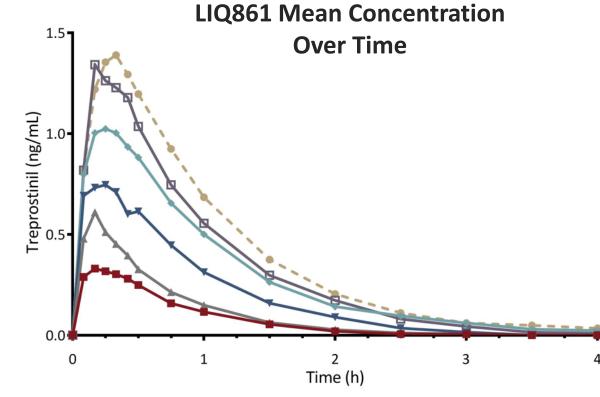
Compact, easy inhaler with established commercial track record



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 SAEs
- No MTD was reached



Approx. Capsule (TRE fill wt.)	🛨 25 μg	🛨 50 μg	- ₹ 75 μg	🕶 100 μg	🖶 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	 On stable dose of Tyvaso® for ≥3 months (or) taking ≤2 approved non-PGI oral PAH therapies 				
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				
	 Quality of life using Minnesota Living with Heart Failure Questionnaire (MLHFQ) 				
PK Study	Establish comparative bioavailability to Tyvaso, the reference listed drug				
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



LIQ861 met primary endpoint in pivotal Phase 3 INSPIRE study

TEAEs observed consistent with inhaled prostacyclins

- Final enrollment (n=121) through Month 2
 - 55 patients transitioned from Tyvaso to LIQ861 with 96% on drug at Month 2 (Transitions)
 - 66 patients prostacyclin-naïve adding LIQ861 with 91% on drug at Month 2 (Add-Ons)
- Safety data consistent with preliminary data presented in 2Q19 (n=109)[^]
- TEAEs in > 4% patients all mild to moderate and no SAEs related to LIQ861
 - No new TEAEs observed: cough, headache, throat irritation, dizziness, diarrhea, oropharyngeal pain, nausea, dyspnea, flushing, chest discomfort
 - Most TEAEs observed during first 2-weeks
- Have not yet reached an MTD, dosing up to 200 mcg capsule-strength



Positive data on comparable PK and exploratory endpoints at Month 2

- Confirmed comparative bioavailability of 75 mcg capsule-strength '861 to 54 mcg (9 breaths) Tyvaso
- Observed high rate of sustained treatment throughout the INSPIRE study
- Effectively titrated >80% Add-On patients from 25 mcg to 75 mcg capsule-strength or higher
- Maintained or improved NYHA Functional Class in more than 90% of all patients
- Improved 6MWD and quality of life, as measured by MLHFQ, in both patient groups
- More than 80% of INSPIRE patients remained on LIQ861 at Month 4
- No significant changes in safety or tolerability at Month 4 compared to Month 2
 - Expect to publish and present clinical data from INSPIRE and PK studies during the course of 2020



LIQ865 for Local Post-Operative Pain

PRINT® bupivacaine, sustained-release injectable

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

- ~50%+ of patients report inadequate local post-operative pain relief
- Reducing opioids is a priority for hospitals, payors and FDA
- Improved pain relief and reduced opioid use can drive key metrics, such as faster recovery and time to discharge
- Local anesthetics have known efficacy profile but are limited to 8 hours
- EXPAREL® demonstrates demand for longer acting relief
 - Provides 24-36 hours as reported in practice
 - Generated \$331M in 2018 net sales and reportedly used in ~5M patients to date
- Physicians desired 3 to 5 days of pain relief in market research





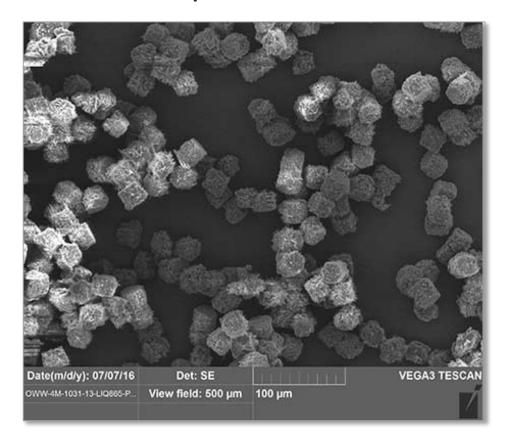




LIQ865 offers the potential for an optimal product profile

- 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

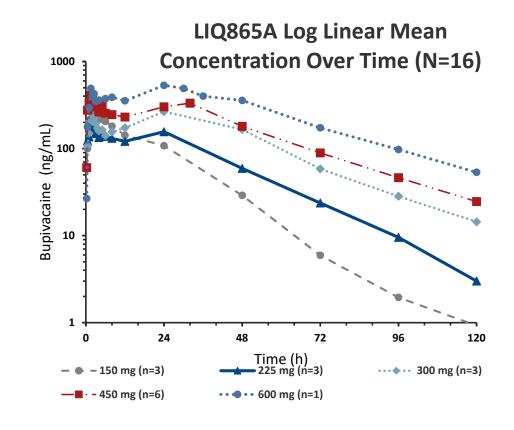
LIQ865: Bupivacaine + PLGA blend





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_{max} well below reported thresholds for neurotoxicity and cardiotoxicity



Preliminary QST results indicate a duration of hypoesthesia and hypoalgesia up to 3-5 days, depending upon stimulation modality, particularly at doses of 300 mg and higher



Preliminary data from nonclinical studies confirm safety in surgical models

Conducted during 2019

To assess incision tensile
strength & peripheral nerve
effects

Results acceptable and not statistically different from controls

To assess bone healing

- Observed dose-dependent delayed healing at the two doses studied
- No adverse effects noted on surrounding soft tissues
- Planning study at lower doses of '865 to determine NOAEL

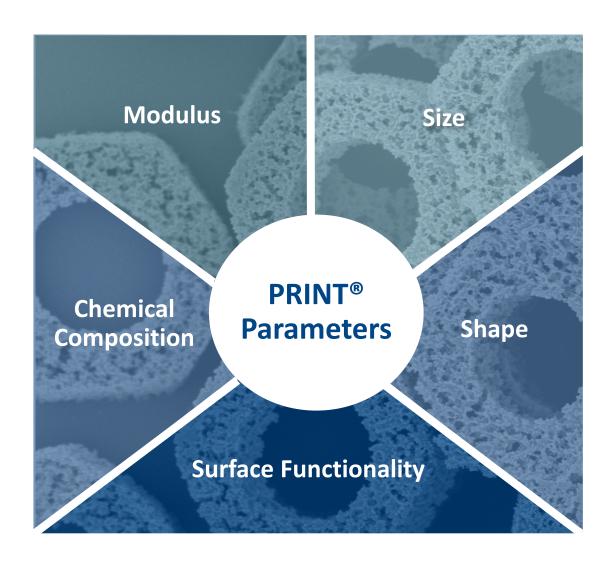
To assess soft tissue healing

 Results acceptable and comparable to vehicle-treated, salinetreated, and Marcaine-treated sites





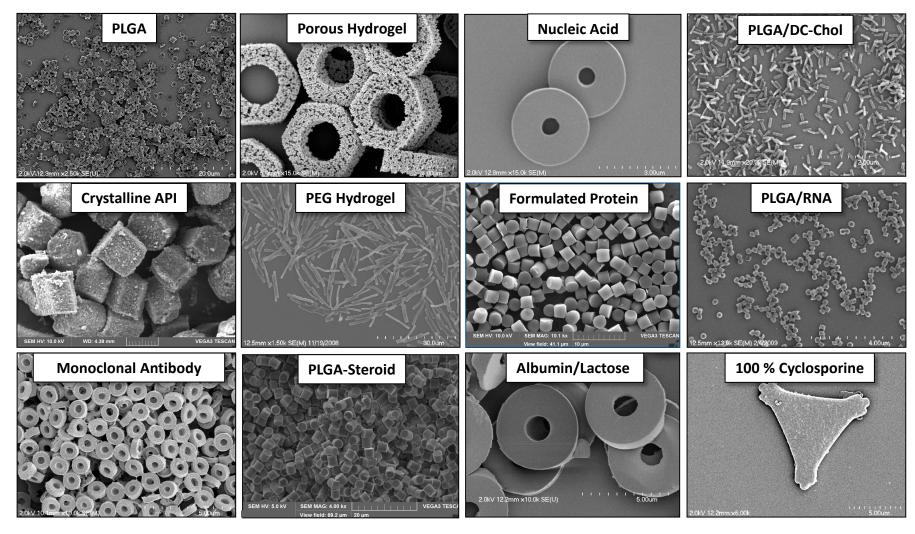
Independent and precise design of each particle feature





Compatible with nearly any material, payload and route of delivery

Examples, not exhaustive





PRINT® production has been scaled for clinical and commercial demands

Preclinical and R&D *Highly versatile, flexible*



Lab Line 2

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

cGMP Process Development
Optimization, scale-up



Lab Line 3

- 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

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





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Thank You