Targeted Therapeutics for Inflammatory Disease

June 2017
Forward Looking Statements/ Safe Harbor

This presentation and the accompanying oral commentary contain “forward-looking” statements that involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this presentation and the accompanying oral commentary, including statements regarding our future financial condition, business strategy and plans and objectives of management for future operations, are forward looking statements. In some cases, you can identify forward-looking statements by terminology such as “believe,” “will,” “may,” “estimate,” “continue,” “anticipate,” “intend,” “should,” “plan,” “might,” “approximately,” “expect,” “predict,” “could,” “potentially” or the negative of these terms or other similar expressions. Forward looking statements appear in a number of places throughout this presentation and the accompanying oral commentary and include statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things, our ongoing and planned preclinical development and clinical trials, the timing of and our ability to make regulatory filings and obtain and maintain regulatory approvals for AQX-1125 and our future product candidates, our intellectual property position, the degree of clinical utility of AQX-1125 and our future product candidates, particularly in specific patient populations, our ability to develop commercial functions, expectations regarding clinical trial data, our results of operations, cash needs, financial condition, liquidity, prospects, growth and strategies, the industry in which we operate and the trends that may affect the industry or us.

Forward-looking statements involve known and unknown risks, uncertainties, assumptions and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. In evaluating these statements, you should specifically consider various factors, including the risks outlined under the caption “Risk Factors” set forth in our Year End Report on Form 10-Q for the quarter-end ended March 31, 2017, which we filed with the Securities and Exchange Commission (“SEC”) on May 9, 2017 and other reports and filings we will make with the SEC from time to time. Forward-looking statements represent our management’s beliefs and assumptions only as of the date of this presentation. Except as required by law, we assume no obligation to update these forward-looking statements publicly, or to update the reasons why actual results could differ materially from those anticipated in the forward-looking statements, even if new information becomes available in the future.
Aquinox Pharmaceuticals, Inc.

- Founded 2006; Founder/CEO David Main
- NASDAQ 2014: AQXP
- Aquinox is discovering and developing novel drug candidates to treat inflammation and immuno-oncology: Primary focus is anti-inflammatory product candidates targeting SHIP1
- As of 2017:
  - 50 employees and growing
  - 2 locations: Vancouver, BC and San Bruno, CA
Experienced Executive Team
Corporate Highlights

- Well capitalized into 2019
- 1st in class drug targeting novel enzyme (SHIP1) with broad anti-inflammatory potential
- Lead Disease Indication:

**AQX-1125 for Interstitial Cystitis / Bladder Pain Syndrome (IC/BPS)**

- Positive Phase 2 results in IC/BPS, Published in Journal of Urology 2016
- Actively Enrolling Patients in Phase 3. Top-line data expected 2018
- Competitive advantage in IC/BPS – a large, underserved market suitable for independent commercialization in the US. Partner RoW
AQX-1125 Overview
AQX-1125 - A Novel First in Class Anti-Inflammatory Therapy

- First in-class SHIP1 activator with broad anti-inflammatory potential

- Favourable ADME
  - Once-daily oral administration
  - High oral bioavailability; minimal metabolism
    - Predominantly eliminated through renal clearance as AQX-1125
  - $T_{1/2} = 21$ hours, $T_{\text{max}} = 1.25$ hours
  - Dose proportional PK, No food effect

- Well tolerated in 7 completed clinical trials
  - Over 385 subjects dosed to date
Activation of SHIP1 by AQX-1125 Puts the Brakes on Inflammation

**SHIP1 Activation:**
- Degrading PIP\(_3\) and increasing PI-3,4-P\(_2\) results in:
  - Decreased cell activation and lowered inflammatory mediators
  - Inhibited cell migration
  - Decreased cell degranulation
- AQX-1125 redirects signalling, does not block it
- Restricted expression of SHIP1 limits off-tissue effects; drug binding site imparts target selectivity and limits off target toxicity

IC/BPS Disease State Overview
IC/BPS Takes a Physical & Mental Toll on Patients

IC/BPS is a life-altering, debilitating condition- characterized by bladder pain and urinary symptoms (Prevalence ~5.5M)\textsuperscript{1-3}

One of the most challenging conditions known to the Urology community (no new oral treatment approved in >20 years)

Fear and anxiety from the unrelenting and unpredictable nature of the disease – “a life sentence…it’s always lurking”\textsuperscript{4}

Etiology is Unknown, Pain is the Hallmark of IC/BPS

IC/BPS is Defined as a Pain Syndrome with a Collection of Symptoms, the Most Important of Which is Pain Perceived to Be in the Bladder

Bladder Pain

Frequency, Nocturia

Urgency

Urinary Incontinence

IC/BPS

OAB

Normal: 6 voids/day
IC/BPS: 17-25 voids during the daytime

Tissue/Organ: AQX-1125 in Inflammation and Pain

(1) Tissue damage triggers damage response, further damage associated with more inflammation triggers damage response.

(2) Vascular leak, activation of resident immune cells, mediator release/production, mast cell degranulation.

(3) Immune cell recruitment.

(4) PAIN.

Leadership 201: Phase 2 Study with AQX-1125 in IC/BPS
LEADERSHIP 201 Phase 2 Trial: July 2013- July 2015

U.S. & Canada; 69 female subjects; on top of background therapy

Screening

Randomization (1:1)

Treatment Period

Follow-Up Period

Day -21
Visit 1

Baseline
Visit 2

Day 7
Visit 2a

Day 14
Visit 3

Day 28
Visit 4

Day 42
Visit 5

Day 70
Visit 6

AQX-1125 200 mg

Once daily

Placebo

**AQX-1125 Reduced Average Daily Bladder Pain Over 6 Weeks**

<table>
<thead>
<tr>
<th>Time</th>
<th>Placebo</th>
<th>AQX-1125 200 mg</th>
<th>p-value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Baseline</td>
<td>-1.0</td>
<td>-1.5</td>
<td>0.521</td>
</tr>
<tr>
<td>2 Weeks</td>
<td>-1.6</td>
<td>-2.1</td>
<td>0.084</td>
</tr>
<tr>
<td>4 Weeks</td>
<td>1.3</td>
<td>-2.4</td>
<td>0.061</td>
</tr>
<tr>
<td>6 Weeks</td>
<td>-1.4</td>
<td></td>
<td></td>
</tr>
<tr>
<td>10 Week Follow-up</td>
<td>-1.5</td>
<td>-1.7</td>
<td></td>
</tr>
</tbody>
</table>

Primary Endpoint - Based on 11-point NRS recorded with an e-diary
AQX-1125 Reduced Maximum Daily Bladder Pain Over 6 Weeks

Based on 11-point NRS recorded with an e-diary
# AQX-1125: Significant Improvement in Secondary Endpoints at Week 6

<table>
<thead>
<tr>
<th>Endpoint</th>
<th>Placebo (N=32)</th>
<th>AQX-1125 200 mg (N=37)</th>
<th>Difference in LS Mean AQX-1125-placebo</th>
<th>p value</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>BLADDER PAIN (11-POINT NRS, CLINIC)</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Average daily pain</td>
<td>-1.1</td>
<td>-2.6</td>
<td>-1.6</td>
<td>0.008</td>
</tr>
<tr>
<td>Maximum daily pain</td>
<td>-1.1</td>
<td>-2.8</td>
<td>-1.6</td>
<td>0.028</td>
</tr>
<tr>
<td><strong>SYMPTOM QUESTIONNAIRES</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>O’Leary-Sant IC Symptom Index (ICSI)</td>
<td>-1.4</td>
<td>-3.8</td>
<td>-2.7</td>
<td>0.005</td>
</tr>
<tr>
<td>O’Leary-Sant IC Problem Index (ICPI)</td>
<td>-1.6</td>
<td>-3.6</td>
<td>-2.5</td>
<td>0.014</td>
</tr>
<tr>
<td>Combined O’Leary-Sant ICSI/ICPI</td>
<td>-3.0</td>
<td>-7.3</td>
<td>-5.1</td>
<td>0.007</td>
</tr>
<tr>
<td>BPIC-SS</td>
<td>-4.0</td>
<td>-8.8</td>
<td>-5.4</td>
<td>0.011</td>
</tr>
<tr>
<td><strong>VOIDING FREQUENCY</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Number of Voids/24h (e-diary)</td>
<td>-0.8</td>
<td>-3.6</td>
<td>-2.8</td>
<td>0.040</td>
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</tbody>
</table>

AQX-1125 Has Similar AE Profile to Placebo

<table>
<thead>
<tr>
<th></th>
<th>LEADERSHIP 201 IC/BPS (6-week dosing)</th>
<th>COMBINED SAFETY DATA Three Phase 2 Trials (LEADERSHIP 201, KINSHIP, FLAGSHIP)</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Placebo N=32</td>
<td>Placebo N=260</td>
</tr>
<tr>
<td></td>
<td>AQX-1125 N=37</td>
<td>AQX-1125 N=263</td>
</tr>
<tr>
<td></td>
<td>n* (%)</td>
<td>n* (%)</td>
</tr>
<tr>
<td>TEAE</td>
<td>25 (78)</td>
<td>143 (55)</td>
</tr>
<tr>
<td>GI Disorders</td>
<td>11 (34)</td>
<td>42 (16)</td>
</tr>
<tr>
<td>Eye Disorders¹</td>
<td>3 (9)</td>
<td>25 (10)</td>
</tr>
<tr>
<td>SAEs</td>
<td>0 (0)</td>
<td>18 (7)</td>
</tr>
<tr>
<td>Deaths</td>
<td>0 (0)</td>
<td>1 (0.4)</td>
</tr>
<tr>
<td>TEAEs Leading to Discontinuation</td>
<td>1 (3)</td>
<td>12 (5)</td>
</tr>
</tbody>
</table>

¹Eye Disorders
- Non-clinical studies showed lens changes in animals, which led us to conduct monitoring in clinical studies
- Monitoring includes slit lamp biomicroscopy, visual acuity, intraocular pressure
- There was no substantial difference in observations at the ophthalmic examination between AQX-1125 and placebo or between start and end of the 6-week treatment period of the Phase 2 study

AQX-1125: Bladder Exposure Through Blood & Urine
Leadership 301: Phase 3 Study with AQX-1125 in IC/BPS
LEADERSHIP 301: Initiated in September 2016

Screening (up to 2 or 6 Weeks)
Treatment Phase (12 Weeks)
Extension Period (52 Weeks)
Follow up period

Randomization (1:1:1)

AQX-1125 200 mg
AQX-1125 100 mg
Placebo

Randomization (1:1)*

AQX-1125 200 mg
AQX-1125 100 mg
AQX-1125 200 mg
AQX-1125 100 mg

Day -42
Day -28/-14
(±3) Visit 1
Day -14 (±3) Visit 1
Day 1 Baseline Visit 2
Day 42 (±3) Visit 3
Day 84 (±3) Visit 4
Day 126 (±7) Visit 5
Day 182 (±7) Visit 6
Day 238 (±7) Visit 7
Day 294 (±7) Visit 8
Day 364 (±7) Visit 9
Day 448 (±7) Visit 10
Day 476 (±7) Visit 11 (Follow-up)
Day 539 (±7) Phone Visit- 11a
Day 630 Visit 12 Ophthalmic Assessment

Visit 1
Visit 1a
subjects requiring a cystoscopy
subjects not requiring a cystoscopy

AQX-1125 200 mg
AQX-1125 100 mg

Safety Follow-Up Visit (4 Weeks post dose)
Follow-Up Telephone Call (3 months post dose)
Ophthalmic Safety Follow-Up Visit (6 months post dose)

Data on File, AQX 2017

Extension period will afford all patients treatment with AQX-1125
IC/BPS Commercial Opportunity
IC/BPS Market Offers an Excellent Growth Opportunity

**Number of Patients**

### Commercial Opportunities Exist for Increasing Diagnosis & Treatment

<table>
<thead>
<tr>
<th></th>
<th>Est. Diagnosed Population&lt;sup&gt;3,4&lt;/sup&gt;</th>
<th>Est. Treated Population&lt;sup&gt;3,4&lt;/sup&gt;</th>
<th>Est. Treatable Population&lt;sup&gt;1,2&lt;/sup&gt;</th>
<th>Est. Prevalence by IC/BPS Symptoms w/Rule-Outs&lt;sup&gt;1,2&lt;/sup&gt;</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>~1M</td>
<td>≤1M</td>
<td>~4.4M</td>
<td>~5.5M</td>
</tr>
</tbody>
</table>

*Est. Treated Population<sup>3,4</sup>*

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*Treatment includes prescription medications, procedures, physical therapy and over the counter (OTC) products.*

Few Clinically Effective and/or FDA Approved Treatment Options

AUA Guidelines

First-Line Treatments
- General Relaxation/Stress Management
- Pain Management
- Patient Education
- Self-care/Behavioral Modification

Second-Line Treatments
- Appropriate manual physical therapy techniques
- Oral: amitriptyline, cimetidine, hydroxyzine, PPS
- Intravesical: DMSO, Heparin, Lidocaine
- Pain Management

Third-Line Treatments
- Cystoscopy under anesthesia w/ hydrodistention
- Pain Management
- Tx of Hunner’s lesions found

Fourth-Line Treatments
- Intradetrusor botulinum toxin A
- Neuromodulation
- Pain Management

Fifth-Line Treatments
- Cyclosporine A
- Pain Management

Sixth-Line Treatments
- Diversion w/ or w/out cystectomy
- Pain Management
- Substitution cystoplasty

Note: For patients with end-stage structurally small bladders, diversion is indicated at any time clinician and physician believe appropriate

Significant Opportunity to Improve Treatment Paradigm

- Only 1 oral product is indicated for IC/BPS, Elmiron®, with many limiting attributes:
  - Limited efficacy in clinical trials and clinical practice
  - TID dosing
  - Unpleasant side effect profile
  - Long time to symptom improvement if patient responds (~3 months)
  - Low adherence/persistence rates¹

- Despite these factors, Elmiron® is the most frequently prescribed treatment for IC/BPS with ~330k total prescriptions & ~$300M U.S. revenues

- Other products used for IC/BPS management are used off-label, based on limited scientific evidence, and offer limited therapeutic utility

AQX-1125, if FDA Approved, May Offer the Ability to Address Unmet Treatment Needs and Become 1st Line Oral Therapy

¹ Hanno PM. Urology. 1997;49(suppl 5A):93-99. * IMS data, FY 2016; IC/BPS only not other uses
Urologists Will Be Key in Driving Diagnosis & Treatment Decisions

- ~60% of patients seeing a HCP for IC/BPS visit a Urologist vs. other specialty*

- Urologists are most frequently reported as the diagnosing specialty† by patients

- Urologists write ~60% of all prescriptions for IC/BPS and generate the highest volume of Elmiron® prescriptions*
  - Patients who visit a Urologist for IC/BPS leave with at least one prescription*

- Aquinox is building relationships within the Urology community

*IMS data 2016
†Market research, data on file
Aquinox is Committed to Raising Awareness for IC/BPS

Building Relationships with Key Advocacy Groups

Healthcare Provider

Patient

AUGS

SUFU

ICNetwork

ICA

Conquering IC. Changing Lives.
Interstitial Cystitis Association
Exploring Additional Opportunities with AQX-1125

Potential Development with AQX-1125

Expanding Urology

Gastrointestinal
Prospective Near-Term Milestones

Near-term data, expanded market opportunities and pipeline advancement

- Abstracts at EAU, AUA, and CUA ✓
- Publication of LEADERSHIP 201 DATA ✓
- Initiation of IC/BPS – 301 Pivotal Trial ✓
- Tablet Manufacturing (Leadership 301) ✓
- Initiate ADME Trial ✓
- Initiate Rat Carcinogenicity Study ✓
- Initiate Mouse Carcinogenicity Study
- Safety & Other Required Phase 1 Trials
- Report ADME results
- LEADERSHIP 301 Top-Line Data (2018)
- Potential New AQX-1125 Indication
Financial & Stock Information

- ~$141.6M cash, cash equivalents and short-term and long-term investment as at March 31, 2017
  - ~$27.6M operating expenses in 2016
  - Raised $75.4M\(^1\) in September, 2016
    - To fund additional clinical development, pre-commercial and market assessment activities, and research, development and manufacturing of product candidates
  - Existing cash to fund first LEADERSHIP 301 trial and supporting activities
  - 23.5M\(^2\) Shares Outstanding (~24.7M fully diluted)

- NASDAQ: AQXP

\(^{1}\)Before underwriting discounts and commissions and offering costs, \(^{2}\)Includes shares issued in September 2016 public offering.
Aquinox Summary

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