



## Aquestive Therapeutics Corporate Presentation

May 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." Words such as "anticipate," "believe," "could," "estimate," "expects," "intend," "may," "plan," "potential," "project," "will," "would," or the negative of those terms, and similar expressions, are intended to identify forward-looking statements. Such statements include, but are not limited to, statements about regulatory approvals and pathways, clinical trial timing and plans, the achievement of clinical and commercial milestones, future financial and operating results, business strategies, market opportunities, financing, 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); development of our sales and marketing capabilities; issues related to the outsourcing of certain operational and staff functions to third parties; the rate and degree of market acceptance of our products and product candidates; the success of any competing products, including generics; the size and growth of our product markets; the effectiveness and safety of our product candidates; risks associated with intellectual property rights and infringement, including the outcome of any patent infringement litigation relating to the Company's products; 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; risks related to legal proceedings, including ongoing patent infringement, investigative and antitrust litigation matters; changes in governmental laws and regulations; the impact 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 included in the Company's Annual Report on Form 10-K filed with the SEC on March 14, 2019.

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 our forward-looking statements after the date of this press release whether as a result of new information, future events or otherwise, except as may be required under applicable law.

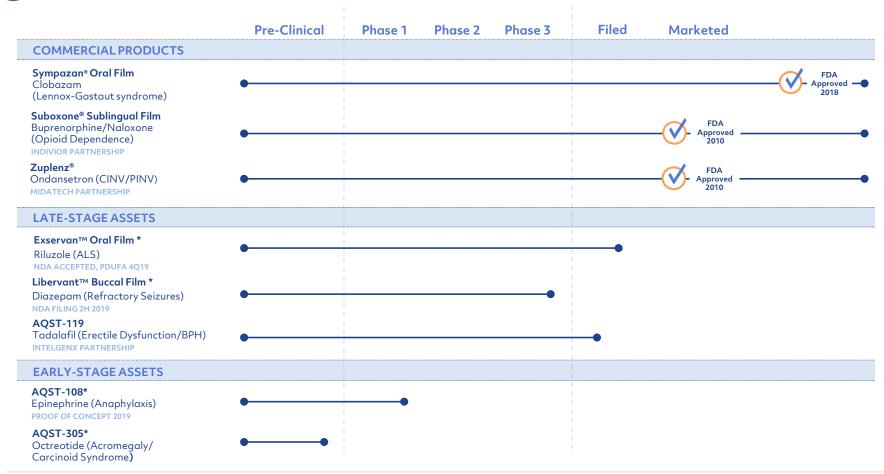


## **Corporate Highlights**

- ► Commercial-stage, specialty pharmaceutical company with comprehensive capabilities to advance medicines from pipeline to market
- Launched first proprietary product in epilepsy franchise and established commercialization infrastructure with 90% overlap in high-volume prescribers with most advanced product candidate
- Advancing a late-stage pipeline that features promising treatments for patients and caregivers living with complex conditions, including hard to manage epilepsies and anaphylaxis
- ▶ Ongoing collaborations provide continuing revenue and options for non-dilutive capital
- World-class manufacturing capabilities have been leveraged to produce billions of film medications to meet the needs of patients worldwide
- ▶ Valuable intellectual property portfolio with 200+ worldwide patents and >90 additional patents pending that offer protection through 2037
- Experienced management team and board with a proven track record of success



## Robust Portfolio







## PharmFilm Delivers Meaningful Differentiation

Diastat rectal gel



Clobazam



Epinephrine injection

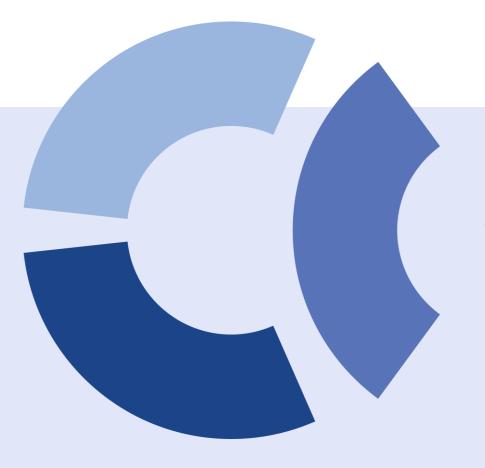




- Rapid onset of action with entry into systemic circulation
- Ease of administration
- Demonstrated bioequivalence, safety and tolerability
- Non-invasive







## **Our Medicines**

Advancing medicines. Solving problems. Improving lives.



# 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

**30** to **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>







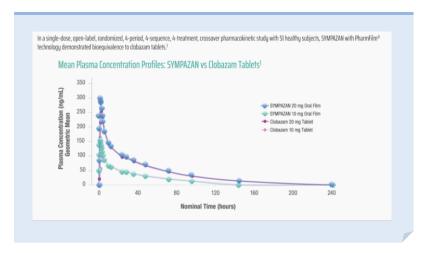
## Sympazan (clobazam) Oral Film



### **RESULTS**

- Proven bioequivalent to clobazam<sup>(1)</sup>
- Reduce weekly drop seizures from 41%-68%<sup>(1)</sup>

In a single-dose, open-label, randomized, 4-period, 4-sequence, 4-treatment, crossover pharmacokinetic study with 51 healthy subjects, SYMPAZAN with PharmFilm® technology demonstrated bioequivalence to clobazam tablets(1)





- Only film formulation approved as adjunctive treatment for seizures associated with LGS
- · May ease caregiver burden with simple administration and accurate dosing
- Launched in December 2018; Team of 50+ experienced commercial professionals is advancing discussions with payers and health care providers
  - o 90% overlap with Libervant in high-volume prescriber base





## Sympazan Launch – Early Progress



### **2018 Key Accomplishments**

- Developed commercial function of 50+ professionals, including hiring and training 30+ sales team
- Active engagement in epilepsy market throughout 2018 leading up to the launch with Medical Affairs, Conference Presentations, Sympoziums and KOL interactions
- Launched Sympazan in December 2018

### Since Launch

- >90% of target HCPs engaged an average of 2.5x
- o Over 85 peer programs executed with an average of 9 attendees per program
- Shipped 700 units with single digit returns from pharmacy

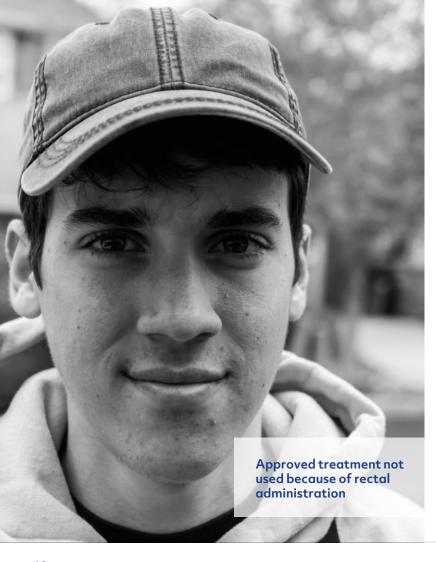
### Payer coverage

- Top 50 commercial payers and top 20 Medicaid states have been engaged in discussions
- Over 40% of target commercial lives are now covered while payer negotiations are underway
  - On pace to reach exit target of 70%
- Covered in top five Medicaid states for clobazam prescriptions
- Covered by leading national pharmacy benefit manager

### **Early Launch Performance**

- Very early TRx and pharmacy pull-through tracking to launch expectations
- Performance is benchmarking to successful analog neurology launches (Banzel (rufinamide) oral suspension and Fycompa (perampanel) oral suspension)





## Solving Problems In EPILEPSY

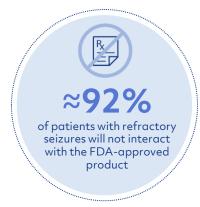
**Refractory seizures**: The failure of 2 or more AED regimens to achieve sustained freedom of seizures resulting in comorbid illnesses, psychological dysfunction, social stigma, and increased risk of mortality<sup>1-3</sup>

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

1.2 M epilepsy patients 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







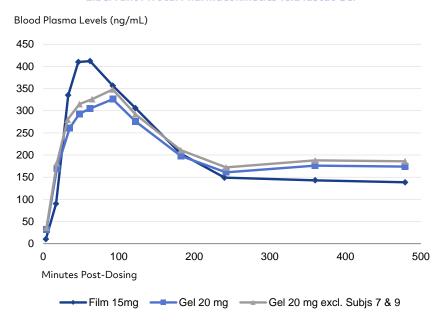
## Advancing Treatment Paradigm for Refractory Seizures



### **RESULTS**

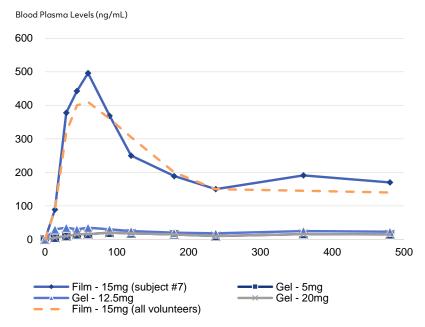
Dissolves Quickly And Demonstrates Consistent, Rapid Achievement Of Therapeutic Blood Levels

### Libervant Pivotal Pharmacokinetics vs. Diastat Gel(1)



- Libervant produces peak diazepam plasma levels similar to diazepam rectal gel using lower doses of active ingredient
- Less inter-subject variability compared to all doses of Diastat

### Libervant: Pivotal Pharmacokinetics vs. Diastat Gel (1) – Individual Subject #7



- Current standard of care (Diastat rectal gel) exhibits a population subset that does not obtain expected plasma concentrations of diazepam
- Libervant dosed under fasted conditions showed consistent plasma concentrations; even in subset of Diastat 'non-responders'



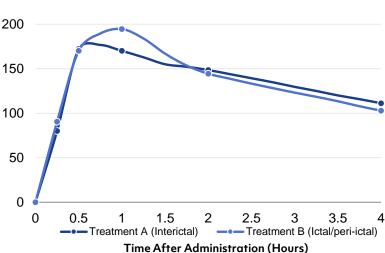


### Studies Demonstrate a Favorable Product Profile



Comparison of Subjects with Epilepsy Treated with DBSF 12.5mg in Interictal State vs. Ictal/Per-Ictal State (1)





 Based on comparison of 21 subjects with valid pharmacokinetic profiles for both Treatment A and Treatment B Frequency of Patients Experiencing Treatment-Emergent Adverse Events Considered Possibly or Probably Related to Study Drug and Number of Events – Safety Population

ADVERSE EVENTS N (%)	PERIOD A (N=33)	PERIOD B (N=33)	OVERALL (N=35)
Somnolence	2 (6.1%)	0	2 (5.7%)
Dizziness	1 (3.0%)	0	1 (2.9%)
Hypoaesthesia	1 (3.0%)	0	1 (2.9%)
Nausa	0	1 (3.0%)	1 (2.9%)
Paraethesia oral	1 (3.0%)	1 (3.0%)	1 (2.9%)

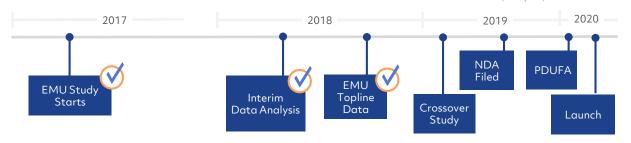
- Majority of treatment-emergent adverse events were classified as unrelated to study drug
- Most common adverse events that were classified as probably related to study drug were somnolence and dizziness and were relatively infrequent (overall frequency of 5.7% of subjects and 2.9% of subjects, respectively)





## Pivotal Trial Design and Expected Timing





Study	Patient Group	Primary Objectives	Secondary Objectives	
Dose Proportionality Study (#162013)	Healthy adults	Demonstrate dose proportional plasma levels for 5, 10 and 15mg doses		
Pivotal Bioavailability Study (#162021)	Healthy adults	Compare pharmacokinetics and bioavailability in healthy subjects to reference product (diazepam rectal gel)		
Crossover Study	Adult patients	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)		
Adult EMU Study (#160326)	Adult patients with epilepsy (n=30)	Compare the pharmacokinetics and bioavailability in subjects with epilepsy in interictal condition, when they	Evaluate safety following single-dose administration	
Pediatric EMU Study (#160325)	Pediatric patients with epilepsy (n=16)	are not experiencing seizures, versus the ictal/peri- ictal condition, when they are experiencing seizures	Evaluate usability in the interictal and ictal/per-ictal conditions	
Safety Study (#42-1703)	Children, adolescents and adults with refractory seizures (n=100)	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	<ul> <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





### **Solving Problems In**

## **ANAPHYLAXIS**

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

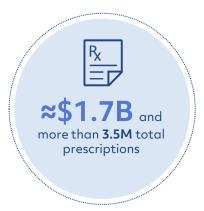
5% of U.S. population<sup>2</sup>

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





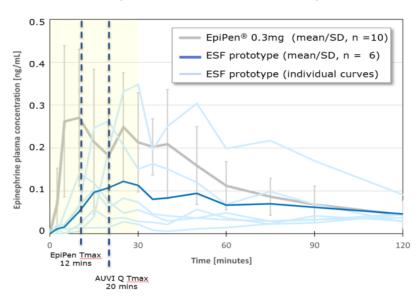


## AQST-108 (epinephrine): Proof of Concept Study

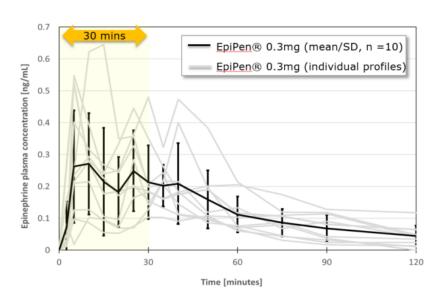
### **RESULTS**

- Successfully demonstrated ability to achieve significant oral absorption using PharmFilm® technology
- Highlighted the variability inherent in EpiPen administration

### Mean profiles for Epinephrine Sublingual Film (ESF) compared to EpiPen® in Human Crossover Study

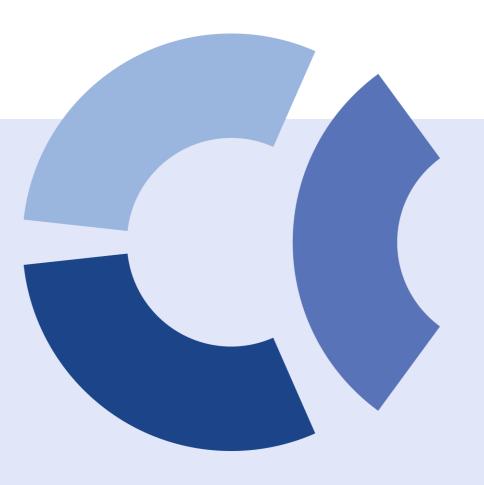


### Mean profiles for EpiPen® in Human Crossover Study









# Financials, Team & Upcoming Milestones

Advancing medicines.
Solving problems.
Improving lives.



### First Quarter 2019 Results

- Total revenues of \$12.6 million
  - Manufacturing revenue of \$6.7 with 57 million shipped product doses
- Adjusted non-GAAP gross margin of 77%
- Total costs and expenses of \$25.7 million
- Adjusted EBITDA loss of \$10.8 million
- Net loss of \$14.7 million, or \$0.59 loss per share
- Cash and cash equivalent of \$39.9 million at 3/31/19

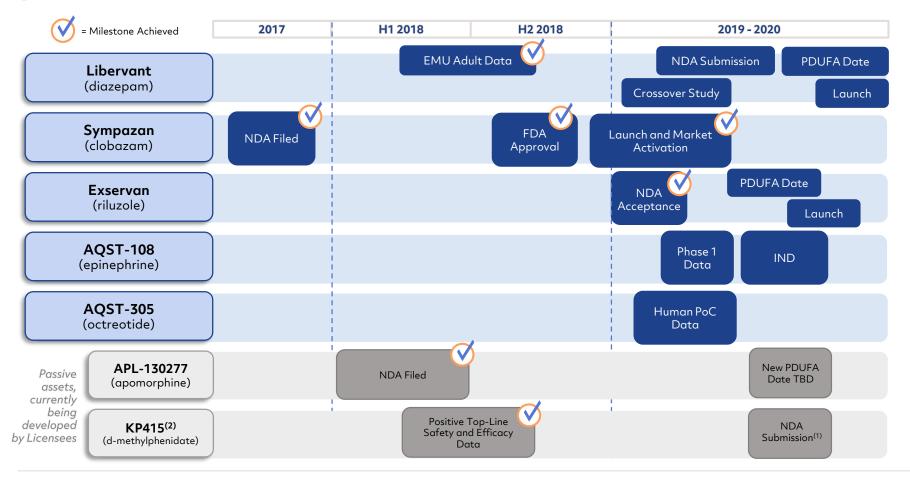
### Full Year 2019 Guidance

- Total revenues of \$33 million to \$45 million
  - Suboxone and Sandoz Authorized Generic manufacturing revenue of \$23 million to \$30 million based on volume guidance range of 190 to 240 million strips
- Non-GAAP gross margins of 70% to 72%
- Non-GAAP EBITDA loss of \$40 million to \$45 million, excluding non-cash stock compensation expenses
- Cash burn of ~\$45-\$50 million after considering interest, capital spending and working capital effects, but prior to any non-dilutive capital transactions





## Multiple Upcoming Near-Term Catalysts







## Management Team & Board of Directors



**Team of innovators** with a record of growing billiondollar businesses



Strong expertise in drug development, intellectual property and capital markets



CNS Commercial team with experience on 50+ branded and generic launches



**Board of Directors** includes experienced pharmaceutical industry leaders

### **Leadership Team**

### **Keith Kendall**

President, Chief Executive Officer: **Board Director** 

### **Dan Barber**

SVP, Chief Operating Officer

### **Peter Boyd**

SVP, Business Process & IT

### Lori Braender

SVP, General Counsel and Chief Compliance Officer

### Ken Marshall

SVP. Commercial Leader

### John Maxwell

SVP, Chief Financial Officer

### Mark Schobel

Chief Innovation and Technology Officer

### Gary Slatko, M.D.

SVP, Chief Medical Officer

### Theresa Wood

SVP, Human Resources and Organizational Development

### **Board of Directors**

### **Sandy Costa**

Chairman, Board of Directors; Former President & COO, Quintiles

### **Doug Bratton**

Founding Partner and Chief Investment Officer, Crestline Investors/Ed Bass Group

### Greg Brown, M.D.

Founding Managing Director, Healthcare Royalty Partners

### John Cochran

Partner and COO, Crestline Investors/Ed Bass Group

### **Nancy Lurker**

CEO of EyePoint Pharmaceuticals, former CEO of PDI Corporation

### James S. Scibetta

CEO of Maverick Therapeutics, former President of Pacina Pharmaceuticals





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

Data on file

#### LGS / SYMPAZAN™ (SLIDES 7-9)

- 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 10)**

- 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. Chen et al, Prevalence and risk factors of seizure clusters in adult patients with epilepsy Epilepsy Res. 2017 Jul;133:98-102. doi: 10.1016/j.eplepsyres.2017.04.016. Epub 2017 Apr 27
- 6. Diastat administration and disposal instructions www.diastat.com

### **ANAPHYLAXIS (SLIDE 14)**

- 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

