

Strongbridge Biopharma plc

November 2017

Forward-looking Statements

This document contains forward-looking statements relating to the Company's strategy, objectives, business development plans and financial position. All statements other than statements of historical facts included in this document, including, without limitation, statements regarding the Company's future financial position, strategy, anticipated investments, costs and results, status and results of clinical trials, size of patient population, plans, outcomes of product development efforts, and objectives of management for future operations, may be deemed to be forward-looking statements. You can identify forward-looking statements by words such as "anticipate," "believe," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "will," "would" or the negative of those terms, and similar expressions that convey uncertainty or future events or outcomes.

These forward-looking statements involve known and unknown risks, uncertainties, and other factors that may cause the Company's actual results, performance, or achievements or industry results to be materially different from those contemplated, projected, forecasted, estimated or budgeted, whether expressed or implied, by these forward-looking statements. Given these risks and uncertainties, investors should not place undue reliance on forward-looking statements as a prediction of actual results. A discussion of certain of these risks may be found in the filings the Company makes with the U.S. Securities and Exchange Commission. None of these forward-looking statements constitutes a guarantee of the future occurrence of such events or of actual results. These statements are based on data, assumptions, and estimates that the Company believes are reasonable.

The forward-looking statements contained in this document are made only as of the date hereof. Except as otherwise required by law, the Company expressly disclaims any obligation or undertaking to release publicly any updates of any forward-looking statements contained in this document to reflect any change in its actual results, assumptions, expectations or any change in events, factors, conditions, or circumstances on which any forward-looking statement contained in this document is based.

Global rare disease biopharmaceutical company with commercial and late-stage portfolio



COMMERCIAL

1st, only FDA-approved drug for ultra-rare Primary Periodic Paralysis*

ORPHAN

RECORLEV™
levoketoconazole

PHASE 3

Potential next-generation cortisol inhibitor for Cushing's Syndrome

ORPHAN

Veldoreotide

PHASE 2 (SHORT-ACTING FORMULATION)

Potential next-generation somatostatin analog, Acromegaly

ORPHAN

Well capitalized to fund planned operations to cash flow positive

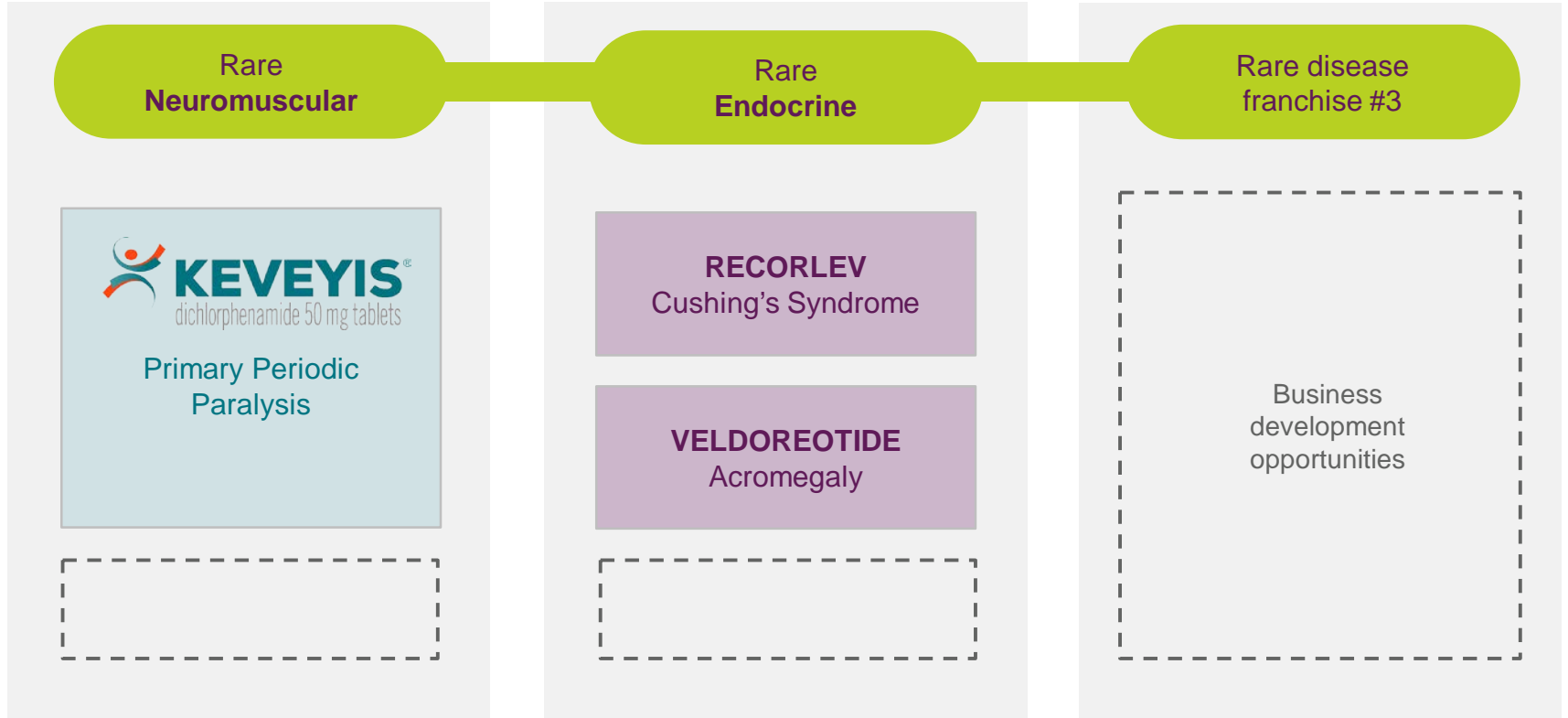
Highly **experienced** rare disease team: clinical and commercial

*FDA-approved treatment for hyperkalemic, hypokalemic, and related variants of primary periodic paralysis

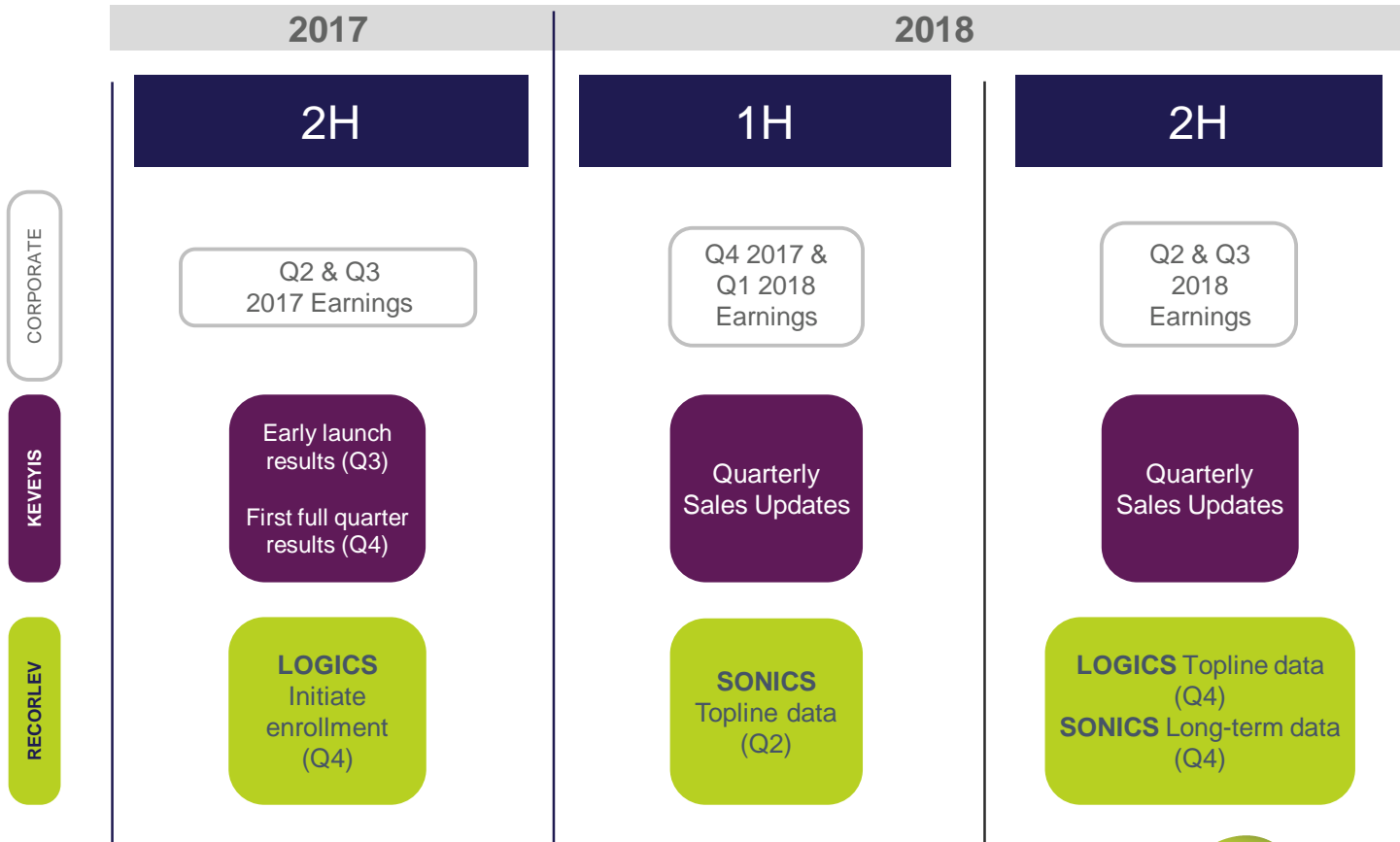
The management team is highly experienced in managing orphan and ultra-rare disease assets

<p>Matthew Pauls President, CEO, Director</p>	
<p>Fred Cohen, M.D. Chief Medical Officer</p>	
<p>Brian Davis Chief Financial Officer</p>	
<p>Stephen Long Chief Legal Officer</p>	
<p>Robert Lutz Chief Business Officer</p>	
<p>Dave Bonnell SVP Sales & Marketing</p>	
<p>Peter Valentinsson SVP, Global Technical Operations</p>	
<p>Scott L. Wilhoit SVP, Global Market Access & Patient Services</p>	

Building a portfolio of therapeutically-aligned vertical franchises in rare diseases



Anticipated milestones – 2H17 & 2018



Keveyis

dichlorphenamide

Keveyis: the first and only FDA-approved therapy for primary periodic paralysis

KEVEYIS[®]
dichlorphenamide 50 mg tablets

GREATER CONTROL. LESS LIMITATION.

FDA approval for PPP in Aug 2015 → Taro launched in Sept 2015 → Strongbridge acquired US rights in Dec 2016, launched in April 2017

FDA-approved treatment for hyperkalemic, hypokalemic, and related variants of primary periodic paralysis

Primary periodic paralysis: a spectrum of rare, chronic, genetic neuromuscular disorders

Causes recurrent, progressive, and debilitating episodes of muscle weakness and temporary paralysis

Triggers may include potassium, carbohydrates, rest after exercise, cold exposure, stress

Symptoms: clumsiness, extreme fatigue, weakness, palpitations, pain. As patients age, muscle weakness can become permanent

59%

have **weekly** attacks

28%

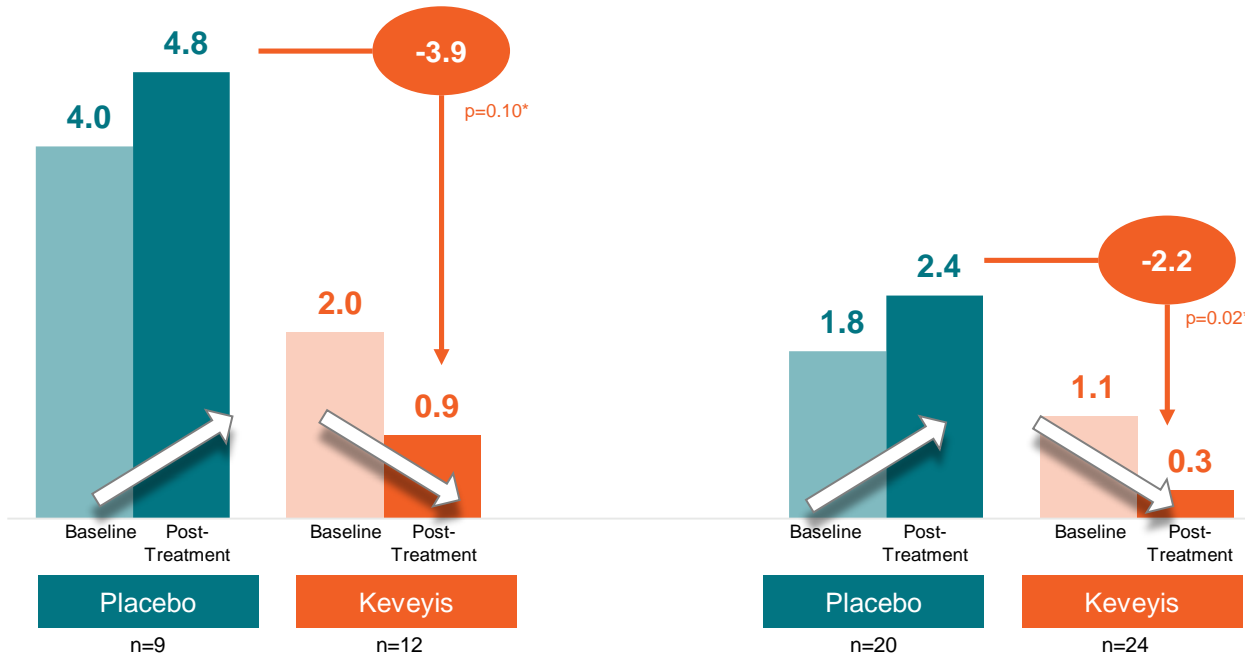
have **daily** attacks

Treatment with Keveyis decreased weekly attack rates

Study 1: decreased weekly attack rates from baseline to week 9

Hyperkalemic

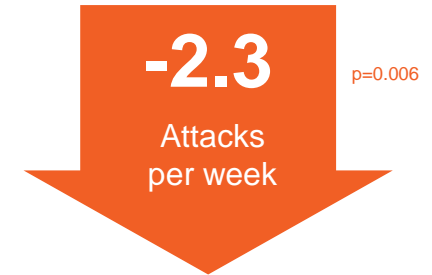
Hypokalemic



Study 2

Hyperkalemic

Mean decrease in attack rates relative to placebo



Mean weekly attack rate at baseline was 3.8 (n=31)

Study 1: Sansone VA, et al. Neurology 2016;86:1408-1416
 Study 2: Tawil R, et al. Ann Nuerol. 2000; 47:46-53.

*Treatment effects (DCP-placebo) are computed as the median of the bootstrap distribution of the treatment group difference in median response

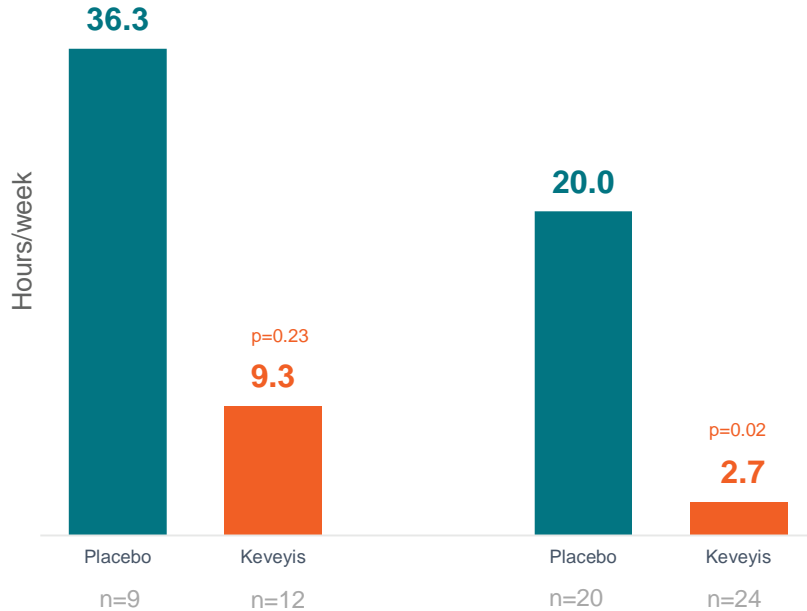
Reduced duration of weekly attacks and decreased attack severity

Average values for each group over weeks 2-9

Decreased weekly attack duration

Study 1: Hyperkalemic

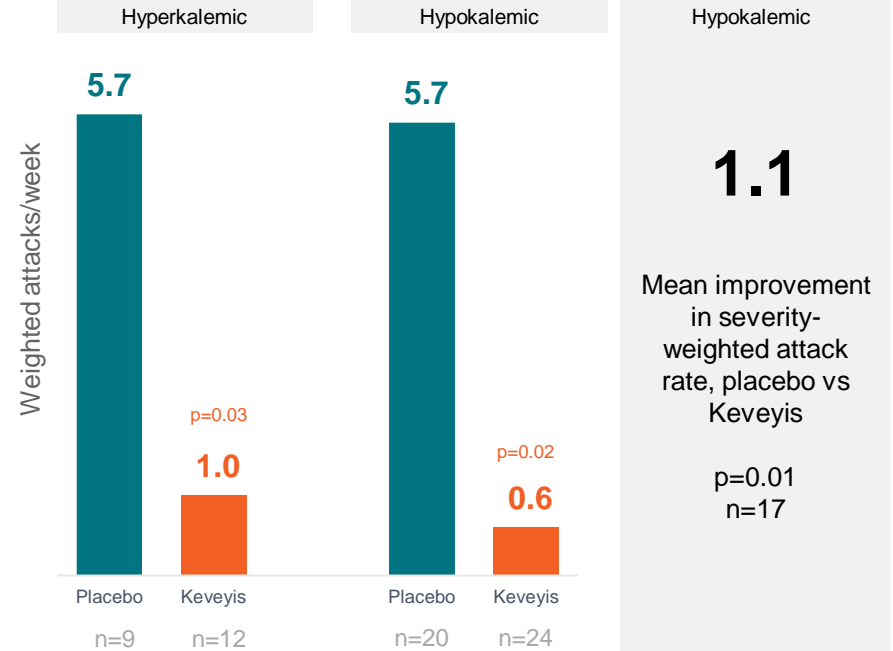
Study 1: Hypokalemic



Decreased severity-weighted attack rate

Study 1

Study 2



Study 1: Sansone VA, et al. Neurology 2016;86:1408-1416
 Study 2: Tawil R, et al. Ann Nuerol. 2000; 47:46-53.

Safety and tolerability

Adverse reactions (≥5% and more common than in patients treated with placebo in Study 1)

	Adverse Reaction	Keveyis n=36 (%)	Placebo n=29 (%)
Nervous system disorders	Paresthesia	44	14
	Cognitive disorder	14	7
	Dysgeusia	14	0
	Confusional state	11	0
	Headache	8	7
	Hypoesthesia	8	0
	Lethargy	8	0
	Dizziness	6	0
Gastrointestinal disorders	Diarrhea	6	3
	Nausea	6	0
General disorders and administration site conditions	Fatigue	8	0
	Malaise	6	0
Investigations	Weight decreased	6	0
Musculoskeletal and connective tissue disorders	Muscle spasms	8	0
	Arthralgia	6	3
	Muscle twitching	6	0
Respiratory	Dyspnea	6	0
	Pharyngolaryngeal pain	6	0
Skin	Rash	8	0
	Pruritus	6	0

PPP market opportunity

Patient population

Approximately 4,000 – 5,000 diagnosed patients*
(2x previous estimate)

Diagnosis can be challenging, often takes 20+ years from symptom onset

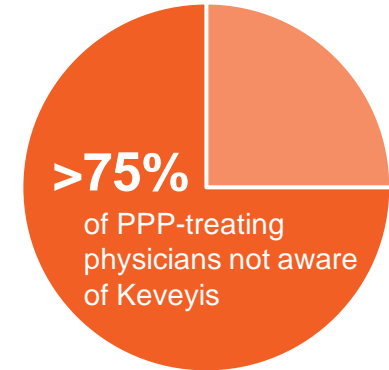
Underdeveloped market

Limited historical investment in disease awareness / education

No other FDA-approved treatment options**

Low Keveyis awareness

Unaided awareness



* Based on Strongbridge Biopharma analysis of medical claims database

**FDA-approved treatment for hyperkalemic, hypokalemic, and related variants of primary periodic paralysis

Keveyis: The formula for early launch success

SALES FORCE



- 12-person team
- Combined 20 orphan drug launches
- Nearly 80 years of collective rare disease sales experience

ADVANCED ANALYTICS



- Multiple data sources
- Predictive modeling
- Identify diagnosed
- Identify undiagnosed

PATIENT SERVICES



- Case management
- Financial assistance
- Adherence/Compliance
- Advocacy connections

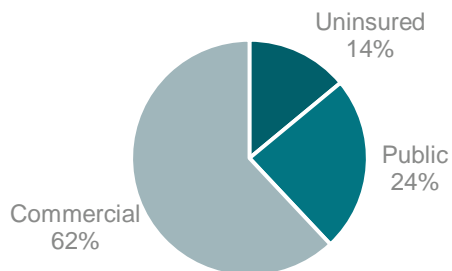
HCP EDUCATION



- Conference presence
- Speaker bureau
- Branded webinars
- Disease education tools

Keveyis market access status: current patients

Payer mix



Broad Payer Coverage

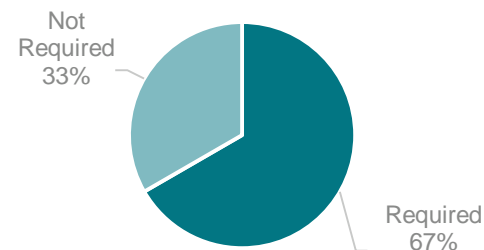
Top 5 national insurers covering > 48 million lives

Largest PBM's covering > 111 million lives

Over 8 Medicare Part D plans covering > 23 million lives

Top regional plans covering > 27 million lives

Payer Prior Authorization Requirements



Dosing

AVERAGE
124 mg/day
2.48 tabs/day

Average Age of Patients

43 Years

Annualized Price

Starting dose:
100 mg per day

\$109,500

Max dose:
200 mg per day

\$219,000

Early launch results and current trends are promising, leading to immediate increased commercial investment

Pre-Existing Patients	New Start Forms	Net Product Sales
72 of 80 Converted to Strongbridge Commercial Supply and PAP	>80 New Start Forms	Q2 2017: \$1.5M Q3 2017: \$2.5M

Significant Market Demand + Expanded Market Size = Immediate Increased Commercial Investment

- Expanding experienced, high-performing sales team from 12 to 21 employees by end of November 2017

- Increasing near-term investment in:
 - PPP disease-state education tactics
 - Keveyis branded promotional initiatives

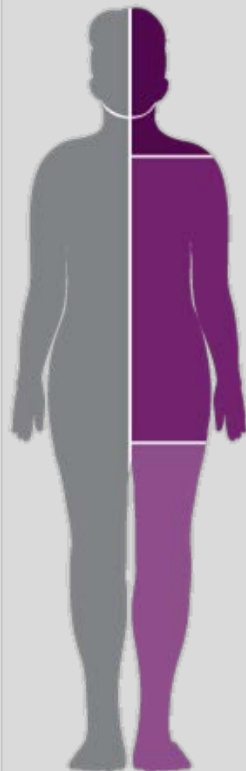
- Nationwide rollout of large-scale PPP genetic testing program (no cost to eligible patients)
- Launched in October 2017

Recorlev

levoketoconazole

Cushing's syndrome: rare endocrine disorder defined by elevated cortisol

Typically caused by pituitary adenomas



Psychosis, impaired memory, sleep disturbance, depression, anxiety



Heart attacks, stroke, high blood pressure, high cholesterol, vein clots



Overweight/obesity, facial, neck and abdominal fat accumulation, diabetes



Muscle and skin atrophy



Osteoporosis

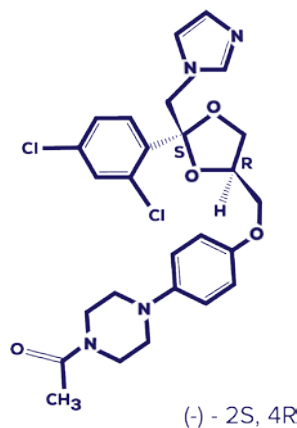
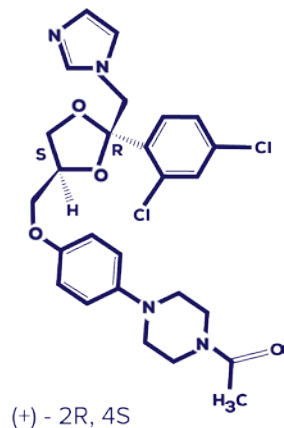
2-4X
increased mortality rate

Recorlev (levoketoconazole) is the pure 2S,4R enantiomer of ketoconazole

KETOCONAZOLE

Two enantiomers combined

Not approved in the US to treat Cushing's Syndrome



RECORLEV

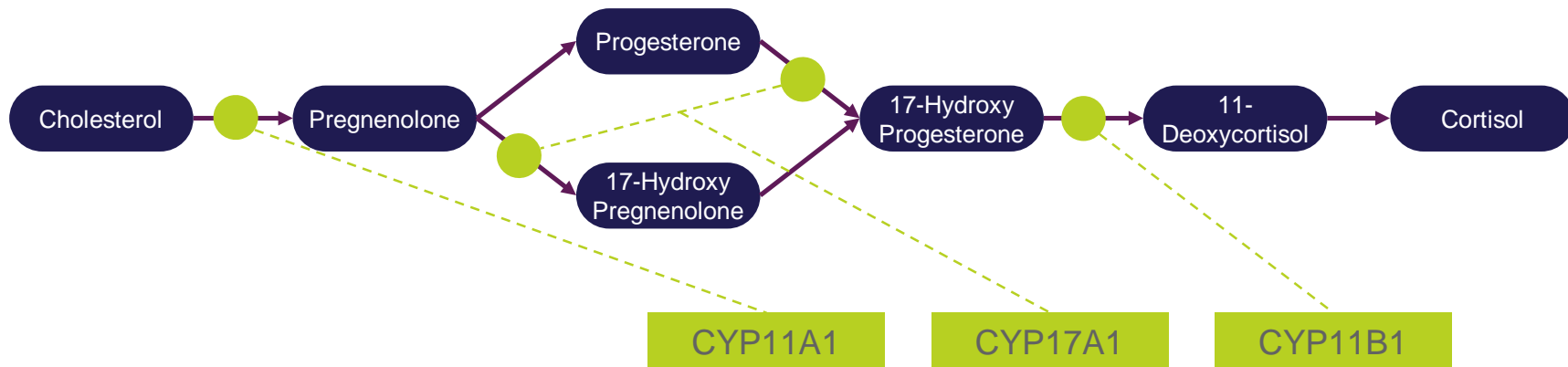
Single enantiomer only

US/EU orphan designation for Cushing's

New chemical entity
FDA 505(b)(2)

Previously in Phase 2 for diabetes (n=118)

Levoketoconazole is the active half of ketoconazole responsible for cortisol synthesis inhibition



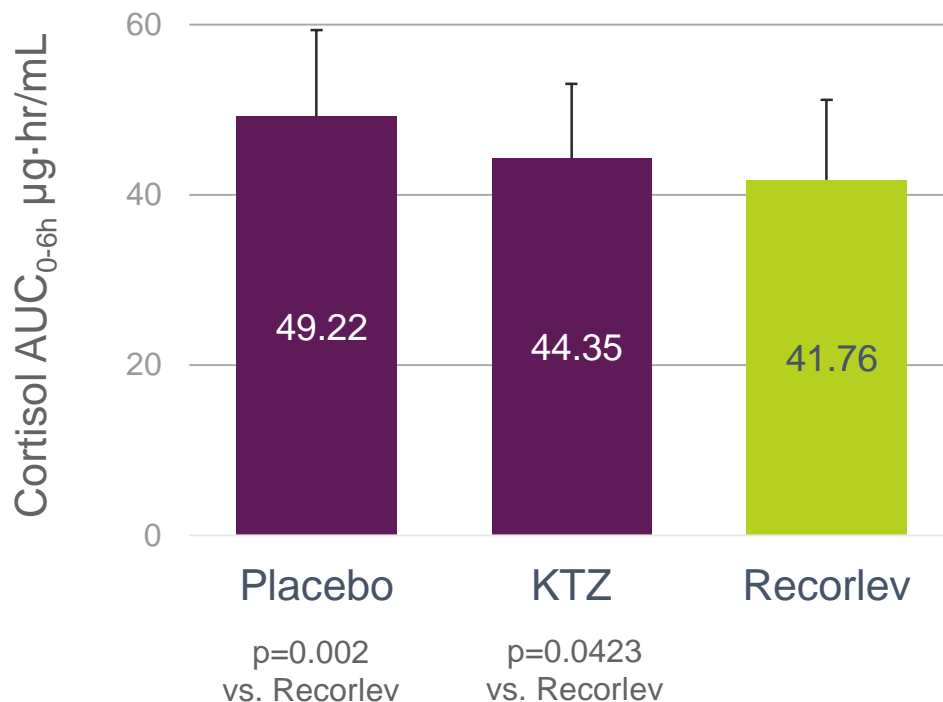
	CYP11A1	CYP17A1	CYP11B1
2R,4S-ketoconazole enantiomer	25,080	595.7	1,365
Ketoconazole, racemate	2,267	57.77	138.6
Levoketoconazole*	1,447	27.94	51.65

10-26x (comparing Levoketoconazole to Ketoconazole, racemate for CYP11B1)
1.6-2.7x (comparing Levoketoconazole to Ketoconazole, racemate for CYP17A1)

50% inhibitory concentration, nmol/L; lower number indicates greater inhibition potency

Source: Auchus RJ, U. of Michigan, data on file; *The active ingredient in RECORLEV

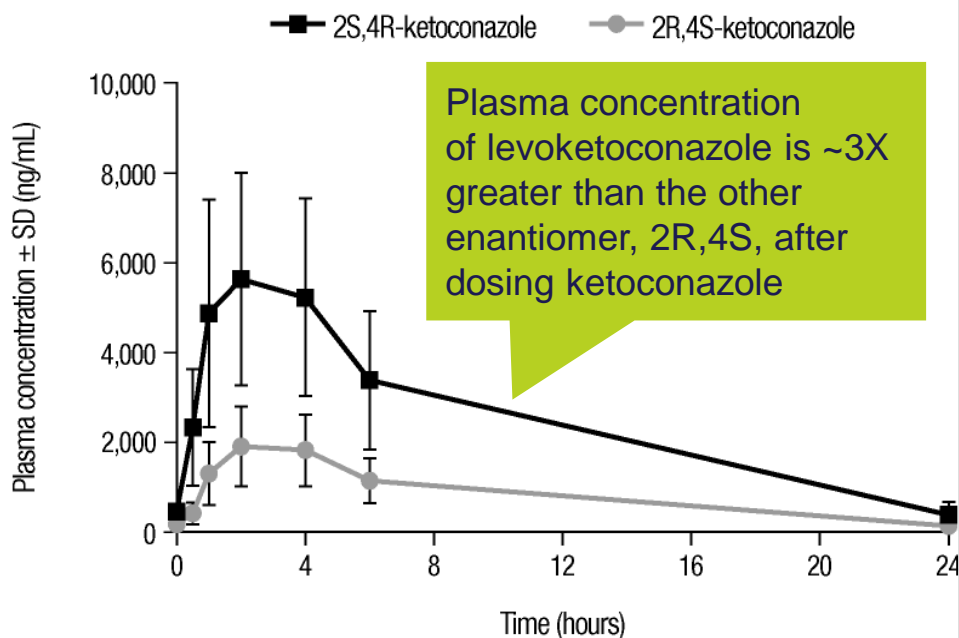
Recorlev significantly suppresses serum cortisol in healthy subjects



Source: AA34510, 24 subjects dosed with 400 mg Recorlev or ketoconazole for 4 days; mean serum cortisol AUC +SD

Recorlev has potential for reduced liver toxicity

PK implies less liver extraction of levoketoconazole



Less potent inhibition of CYP7A

50% inhibition concentration, nmol/L

Levo-KTZ

2,400

2R,4S-KTZ

195

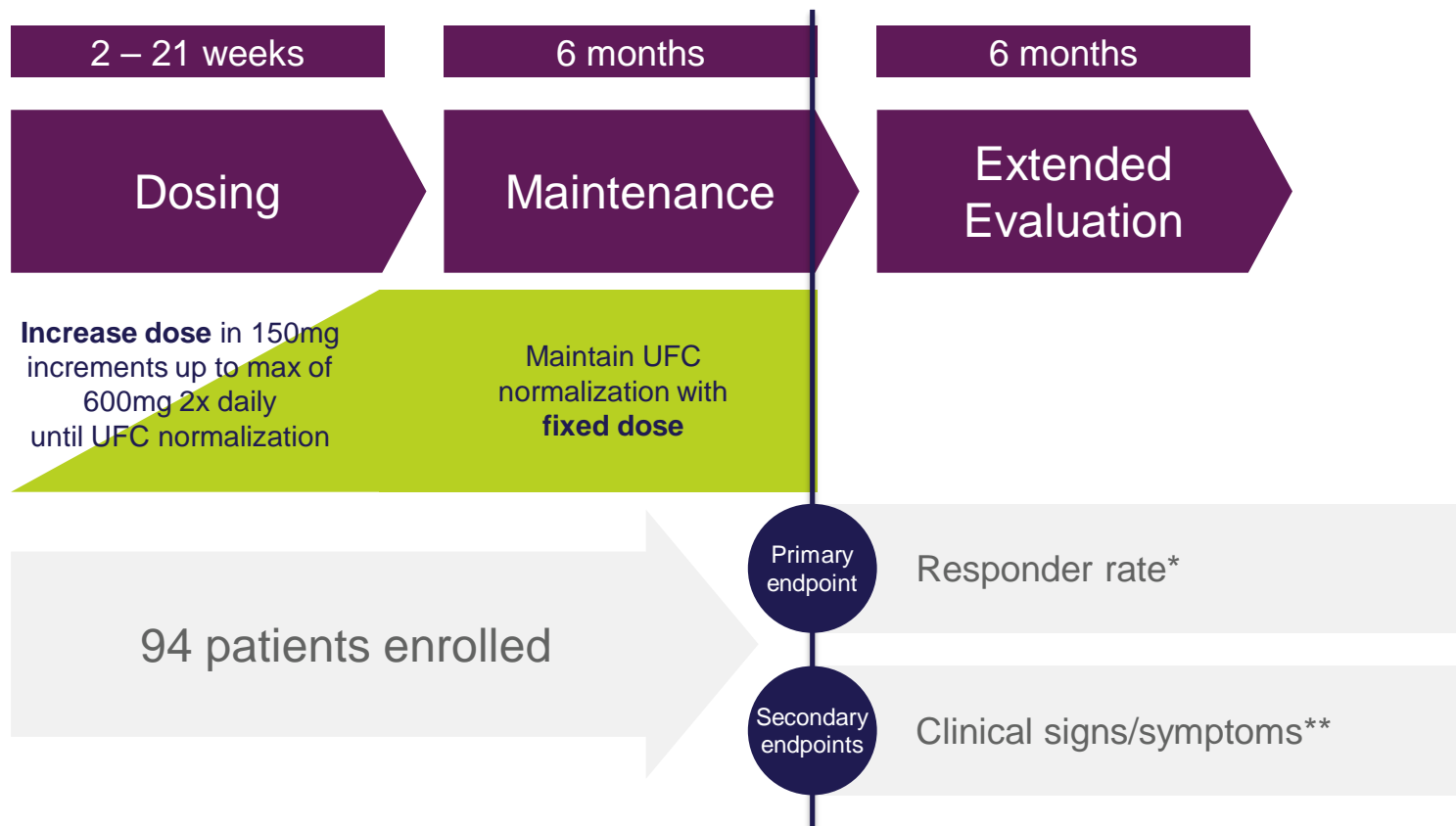
12x

Levoketoconazole is a 12-fold **less potent** inhibitor of CYP7A, the rate-limiting enzyme for bile acid synthesis.

Bile acids aid fat and vitamin absorption and help eliminate toxins and drugs, including Recorlev.

Recorlev Phase 3 Clinical Trials

SONICS - single arm, open-label study in 94 patients

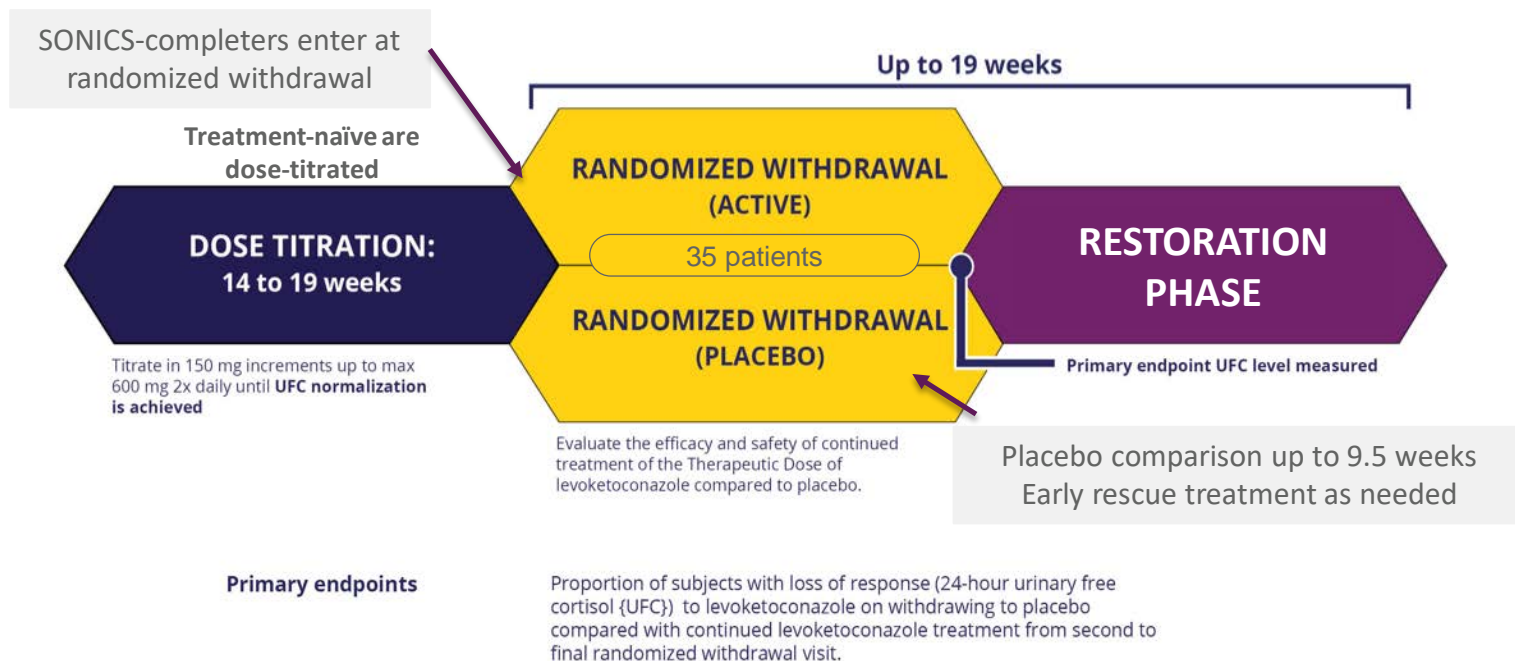


* Normalized 24-hour urinary free cortisol (UFC) after 6 months of maintenance without dose increase

** HbA1c, glucose, blood pressure, lipid profile, CRP, weight, quality of life measures

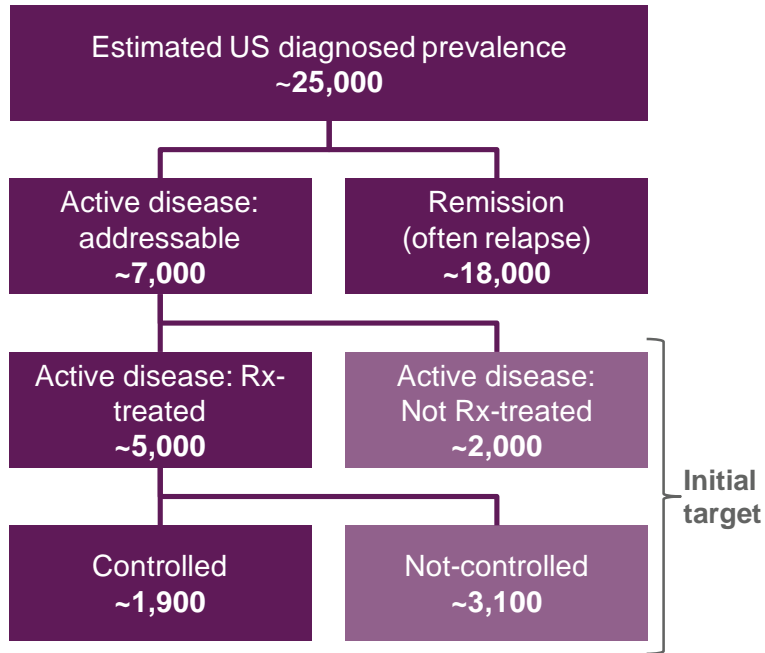
Recorlev Phase 3 Clinical Trials

LOGICS: double-blind, randomized, placebo-controlled study in 35 patients



Cushing's syndrome market opportunity

Patient population



Suboptimal treatments

Highly fragmented market, significant off-label use

Unmet need for new treatment

Broad FDA-approved indication

Low rates of hyperglycemia, reproductive disorders, etc.

Simple dosing and titration

RECORLEV

Potential next-generation cortisol inhibitor

Pure single enantiomer of ketoconazole

Potential for lower liver toxicity

Early engagement with Cushing's Syndrome community & market development initiatives

Patient advocacy



Multi-language patient education brochures



Sponsor of the Annual Patient Summit

Disease awareness



Cushing Syndrome on "The Balancing Act"

Attend major endocrine conferences

Create/implement market development initiatives for Cushing's Syndrome

KOL engagement

Scientific Advisory Board

Richard Auchus MD PhD University of Michigan	Beverly Biller MD Massachusetts General Hospital	Thierry Brue MD PhD University of Marseille
Frederic Castinetti MD PhD University of Marseille	Maria Fleseriu MD Oregon Health & Science University	Eliza Geer MD Memorial Sloan-Kettering Cancer Center
Anthony Heaney MD PhD University of California, Los Angeles	Aart Jan van der Lely MD PhD Erasmus University	Shlomo Melmed MBChB Cedars-Sinai Medical Center
Richard Feelders MD PhD Erasmus University	Christian Strasburger MD Charite University, Berlin	Susan Webb MD PhD University of Barcelona

Veldoreotide

Veldoreotide LAR: A novel, multi-receptor somatostatin analog

BACKGROUND

Acquired immediate-release formulation of veldoreotide in 2015 and focused initial R&D on long-acting reformulation

10/2016: Successfully formulated for convenient, at-home, subcutaneous administration using PLGA microspheres

Unique formulation may provide additional IP

Data through Phase IIa: potential differentiated benefits from currently approved somatostatin analogues

Comparable maximal GH suppression to octreotide

Reduced impact on gallbladder function, bile acid production, and GI motility in rodents

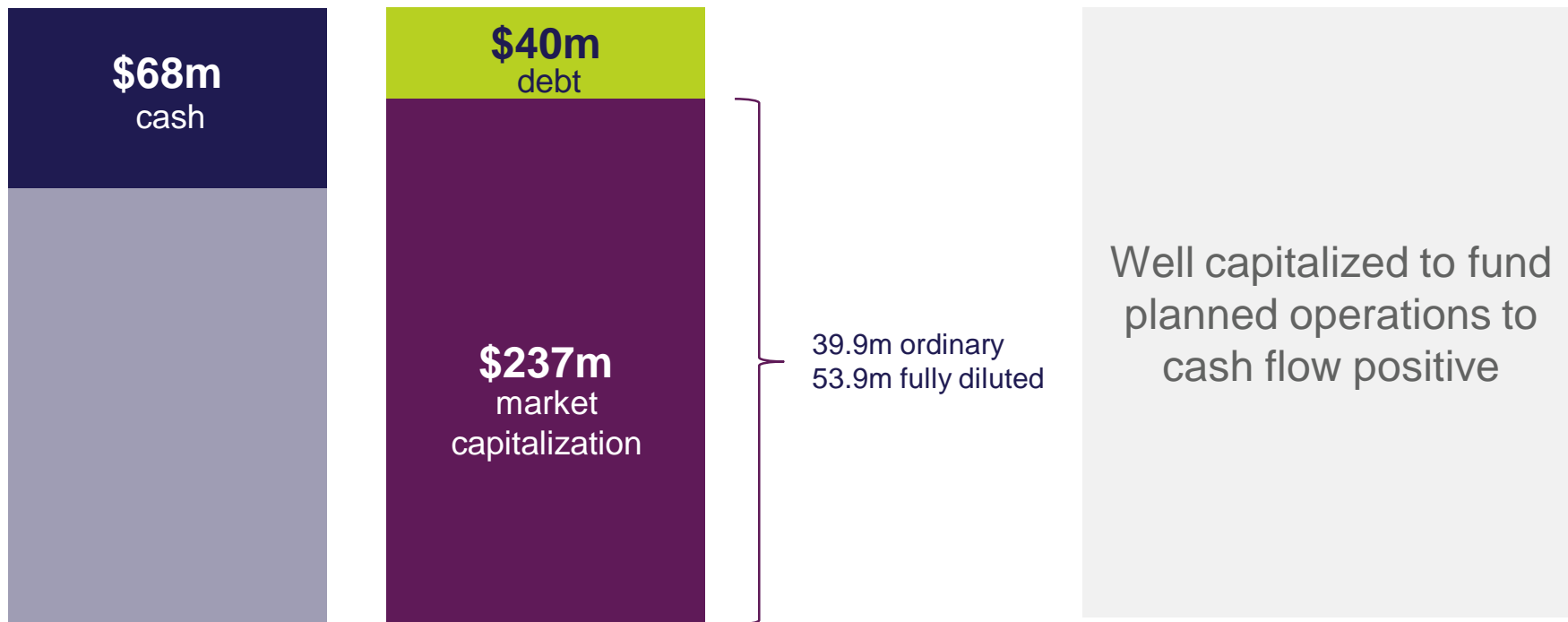
Reduced impact on hormonal responses to mixed meals in healthy subjects

About Strongbridge

Intellectual property and orphan exclusivity

	IP		Orphan exclusivity	
	US	EU	US	EU
Keveyis	Exploring options	US rights only	Aug 2022	US rights only
Recorlev	<p>2030 Method of use: reducing CRP levels and systemic inflammation</p> <p>Under review Method of use: reducing cortisol levels</p>	<p>2026 Method of use: treating Cushing's syndrome</p>	7 years	10 years
Veldoreotide	Filed patent application for novel formulation		7 years	10 years

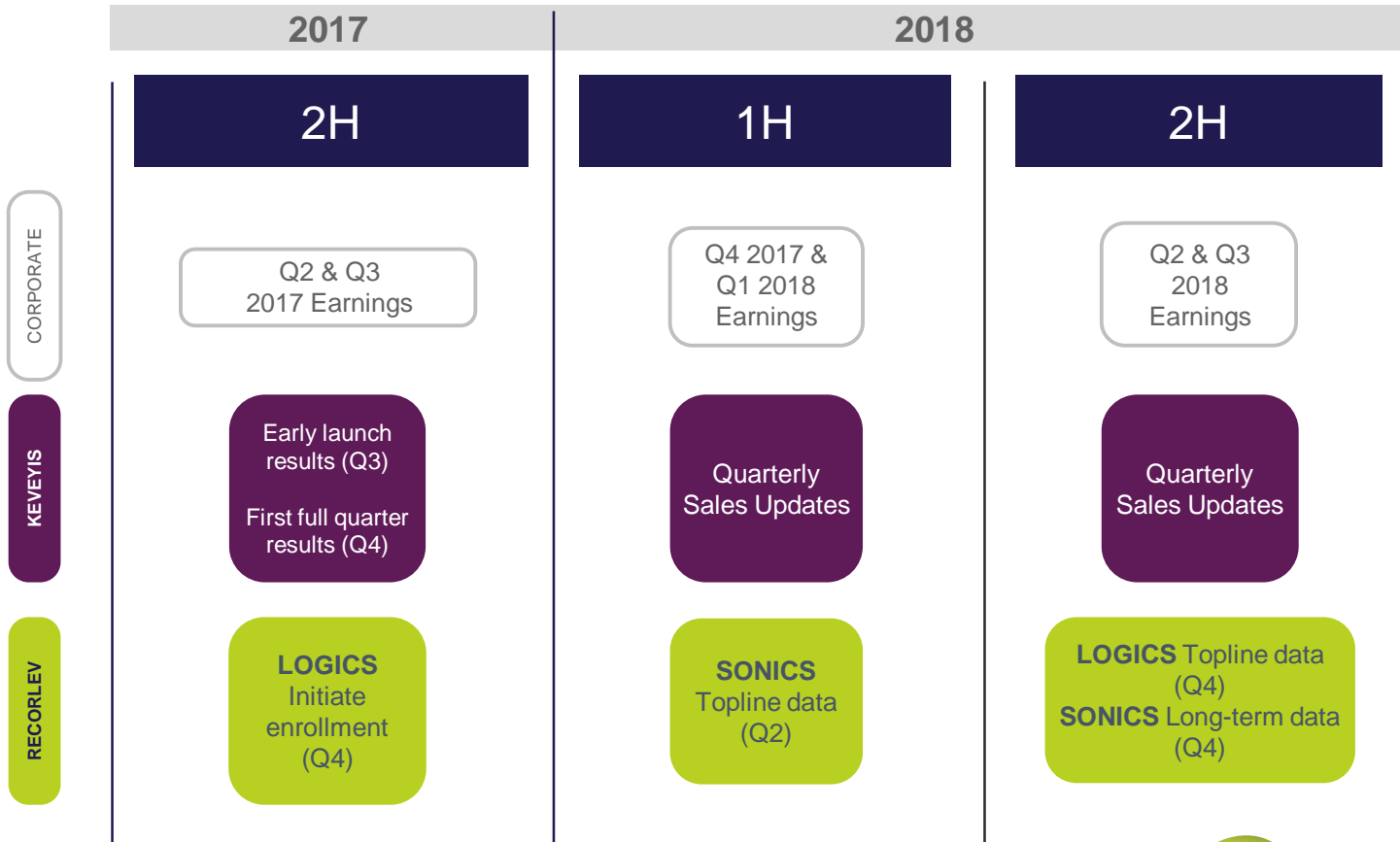
Strong balance sheet to support operations



Market capitalization as of November 13, 2017.

Pro forma cash as of September 30 and shares outstanding includes impact of October 4 equity financing.

Anticipated milestones – 2H17 & 2018



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1st, only FDA-approved drug for ultra-rare Primary Periodic Paralysis*

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