# STRONGBRIDGE BIOPHARMA PLC

MAY 2020

# 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, anticipated timing of release of results from clinical trials, size of patient population potential, advantages of a product or product candidate, anticipated timing of activities related to the regulatory approval process for a product candidate, results of company-sponsored market research, plans, outcomes of product development efforts, intellectual property portfolio 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.

# Strongbridge Biopharma is a revenue-generating rare disease company


**Approved** for the treatment of Primary Periodic Paralysis (PPP)

2019 revenue of ~$21.7M;
Q1 2020 Revenue of $6.7M
(**up 54% from Q1 2019**)
**2020 revenue guidance: $22M - $26M**;
Product is contribution margin positive

Provides established and leverageable rare disease **commercial infrastructure**

## RECORLEV®
(levoketoconazole)

**Phase 3 asset** for the treatment of Cushing's syndrome (CS)

**Positive Phase 3 SONICS results** published in *The Lancet Diabetes and Endocrinology*

LOGICS Phase 3 trial **enrollment to close on or before May 14**
Top-line **results anticipated Q3 2020**;
NDA filing expected ~6 months thereafter

## VELDOREOTIDE
modified-release

**Next-generation** somatostatin (SST) analog

Immediate release formulation **studied in Phase 2** in acromegaly patients

Novel, patented, extended release formulation is **under evaluation** in nonclinical disease models potentially amenable to SST modulation

**$63M IN CASH EXPECTED TO FUND OPERATIONS THROUGH 3Q 2021***

*As of 3/31/2020, cash, cash equivalents, and marketable securities

The safety and efficacy of Recorlev (levoketoconazole) for treatment of endogenous Cushing's syndrome has not been established.

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# Management team

## John Johnson

**Executive Chairman**

Johnson & Johnson · Lilly · ImClone Systems Incorporated · Pfizer

## Richard Kollender

**Chief Operating Officer**

Quaker Partners · Rapid micro biosystems · GSK GlaxoSmithKline

## Robert Lutz

**Chief Financial Officer**

Shire · medgenics · Goldman Sachs

## Fred Cohen, MD

**Chief Medical Officer**

Aptalis · Johnson & Johnson · Eurand · Lilly

## Scott Wilhoit

**Chief Commercial Officer**

NPS Pharma · Johnson & Johnson · PTC · Marathon Pharmaceuticals · Auxilium

## Stephen Long

**Chief Legal Officer**

ReedSmith · BARD · Warner Lambert

## Brian Conner

**Chief Compliance Officer**

Huron · Shire

## Emily Doyle

**Chief Human Resources Officer**

Noramco Inc · Globus Medical · Shire · AstraZeneca

**EXPERIENCE IN RARE DISEASE DEVELOPMENT & COMMERCIALIZATION**

# RECORLEV

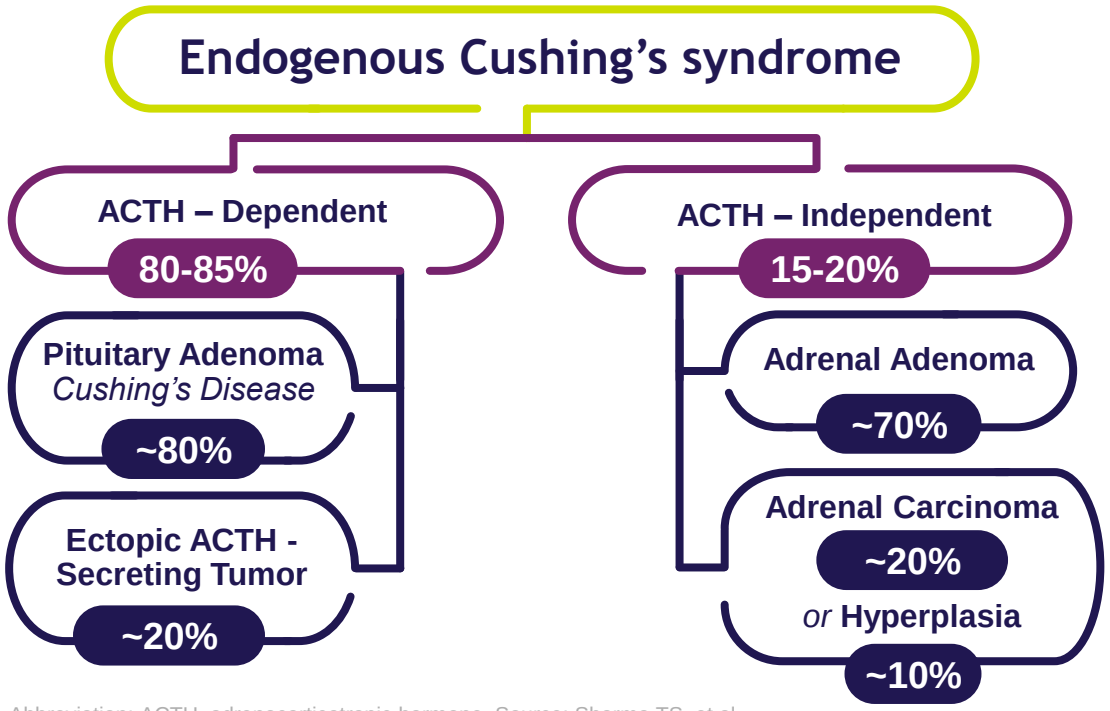
(levoketoconazole)



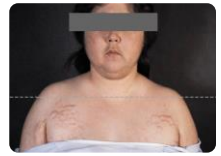
The safety and efficacy of Recorlev (levoketoconazole) for treatment of endogenous Cushing's syndrome has not been established.

# Endogenous Cushing's syndrome (CS) overview

Underlying cause is chronic exposure to
**EXCESS SERUM CORTISOL**
due to any of several etiologies




## Affects the whole body

| | | |
|---|---|---|
| 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 |  |

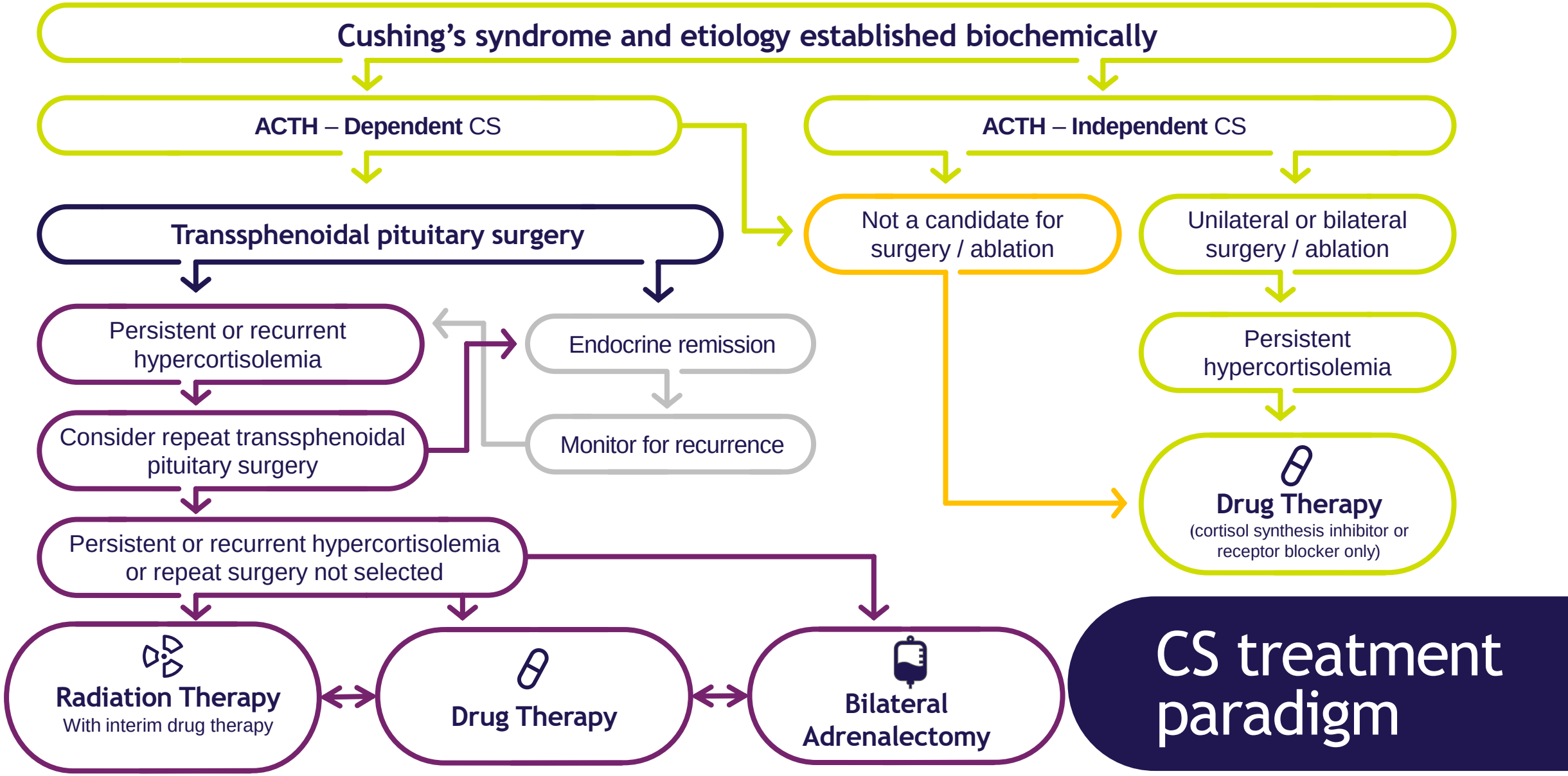
## Patients have*

**2-5x** higher incidence rates of comorbidities

**7x** higher medical costs

**4x** higher pharmacy costs

Abbreviation: ACTH, adrenocorticotropic hormone. Source: Sharma TS, et al. Clin Epidemiol. 2015;7:281–293.
*According to a retrospective analysis of claims from a large US commercial health plan (885 selected Cushing's disease cases and 2,655 matched controls without Cushing's disease) from 2007 to 2011.




The Journal of Clinical Endocrinology & Metabolism, Volume 100, Issue 8, 1 August 2015, Pages 2807–2831, https://doi.org/10.1210/jc.2015-1818
Tritos and Biller. Journal of Internal Medicine,
Volume: 286, Issue: 5, Pages: 526-541, First published: 11 September 2019, DOI: (10.1111/joim.12975)

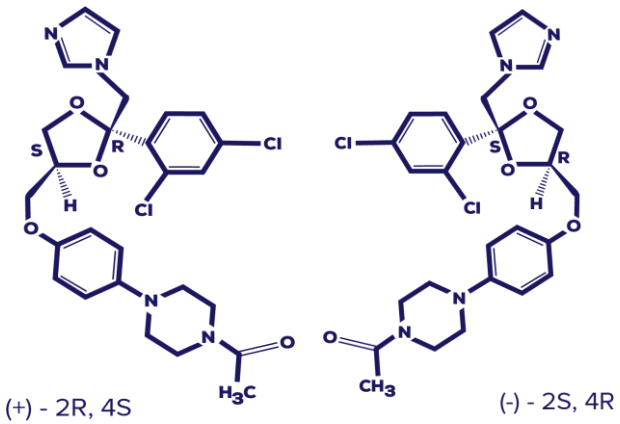
# Levoketoconazole appears to be the relevant enantiomer of ketoconazole for cortisol synthesis inhibition in humans

## DEXTROKETOCONAZOLE

**Right-handed**
enantiomer

Estimated to **contribute essentially <u>no</u> activity** towards the inhibition of adrenal cortisol synthesis by ketoconazole

## KETOCONAZOLE
Racemate with Two Enantiomers




## LEVOKETOCONAZOLE

**Left-handed**
enantiomer

Estimated to **provide essentially <u>all</u> of the cortisol synthesis inhibition** of ketoconazole in vivo

Auchus RJ, Wu JL, Peng HM "2S,4R-Ketoconazole is the Relevant Enantiomer of Ketoconazole for Cortisol Synthesis Inhibition: Steroidogenic P450s Inhibition Involves Multiple Mechanisms" Endocrine Society's Annual Meeting. University of Michigan, Chicago, IL. 18, March 2018. Poster presentation.

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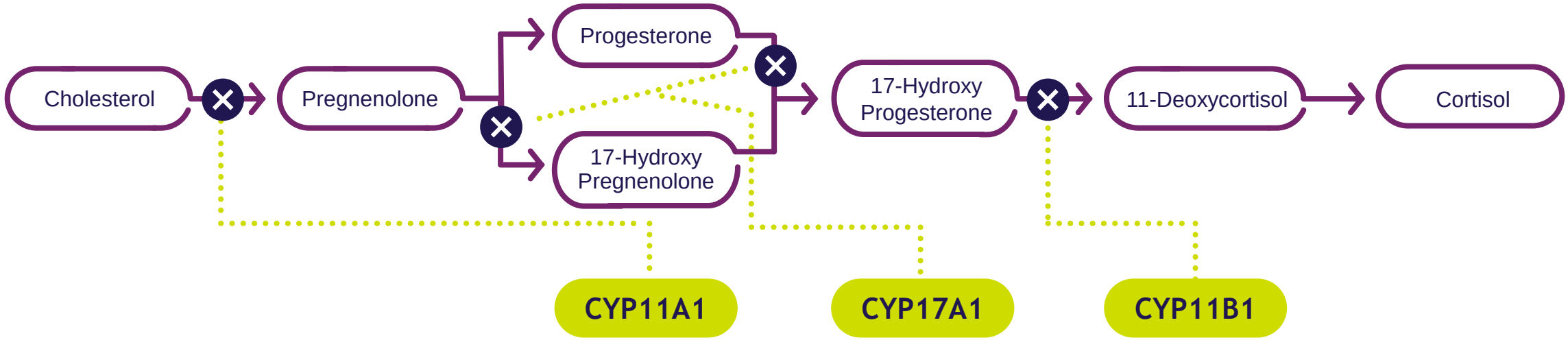
# Levoketoconazole is approximately twice as potent as ketoconazole for cortisol synthesis inhibition




| | CYP11A1 | CYP17A1 | CYP11B1 |
|---|---|---|---|
| **Ketoconazole, racemate** | 2,267 | 58 | 139 |
| **Dextroketoconazole** | 25,080 | 596 | 1,365 |
| **Levoketoconazole*** | 1,447 | 28 | 52 |

1.6-2.7x

10-26x

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


Auchus RJ, Wu JL, Peng HM "2S,4R-Ketoconazole is the Relevant Enantiomer of Ketoconazole for Cortisol Synthesis Inhibition: Steroidogenic P450s Inhibition Involves Multiple Mechanisms" Endocrine Society's Annual Meeting. University of Michigan, Chicago, IL. 18, March 2018. Poster presentation.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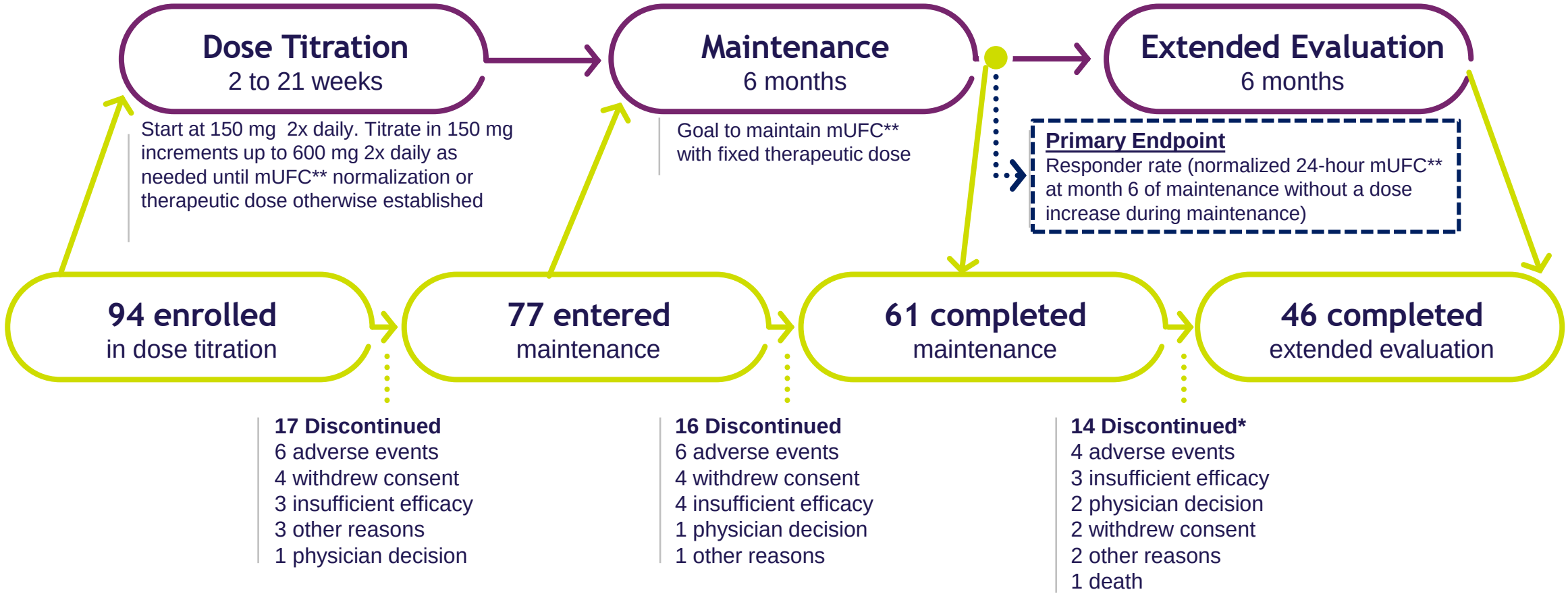
# SONICS, a successfully completed phase 3, multi-center, open-label, single-arm study



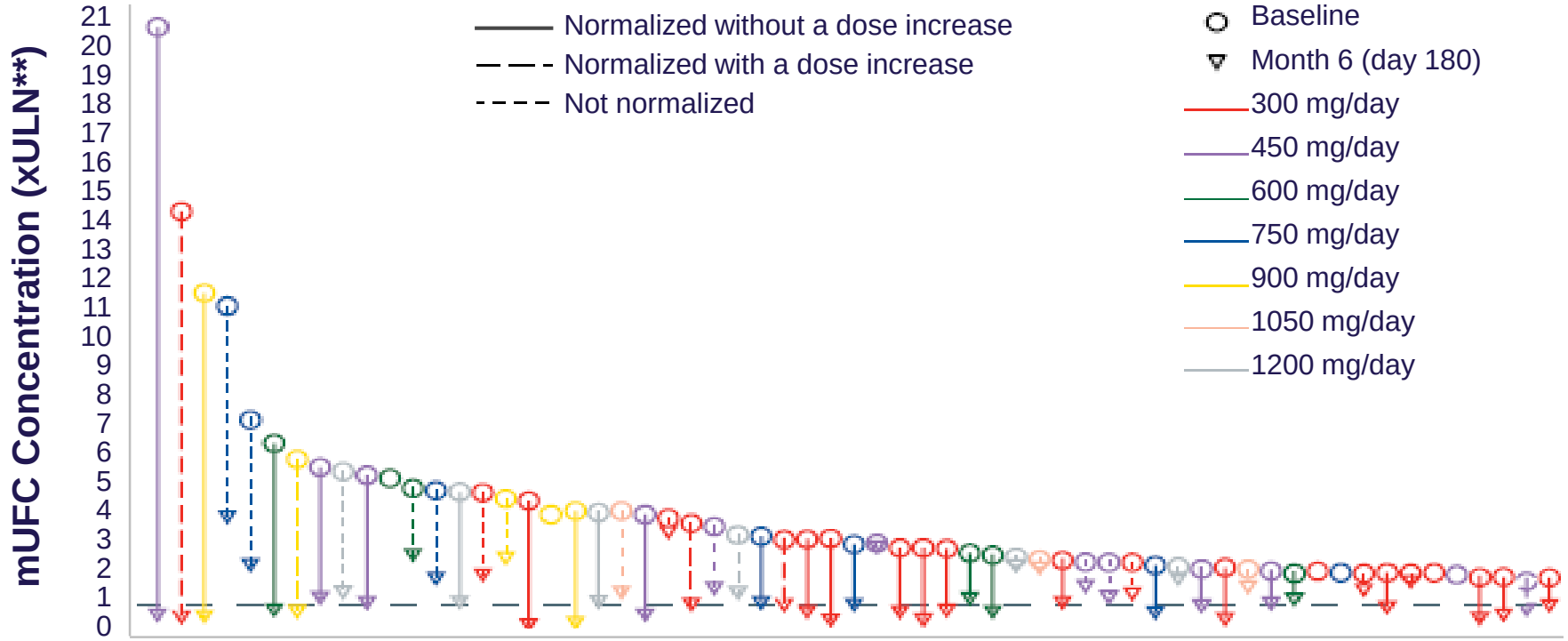

*1 subject did not enter extended evaluation
**mUFC= mean urinary free cortisol

The safety and efficacy of Recorlev (levoketoconazole) for treatment of endogenous Cushing's syndrome has not been established.

# SONICS achieved statistical significance of the primary endpoint

Primary endpoint was achieved with **statistical significance, with 30% of patients (29/94) achieving mean urinary free cortisol (mUFC) normalization** without a dose increase

(95% CI: 21%, 40%; p=.0154 vs null hypothesis of ≤ 20%), ITT** analysis*

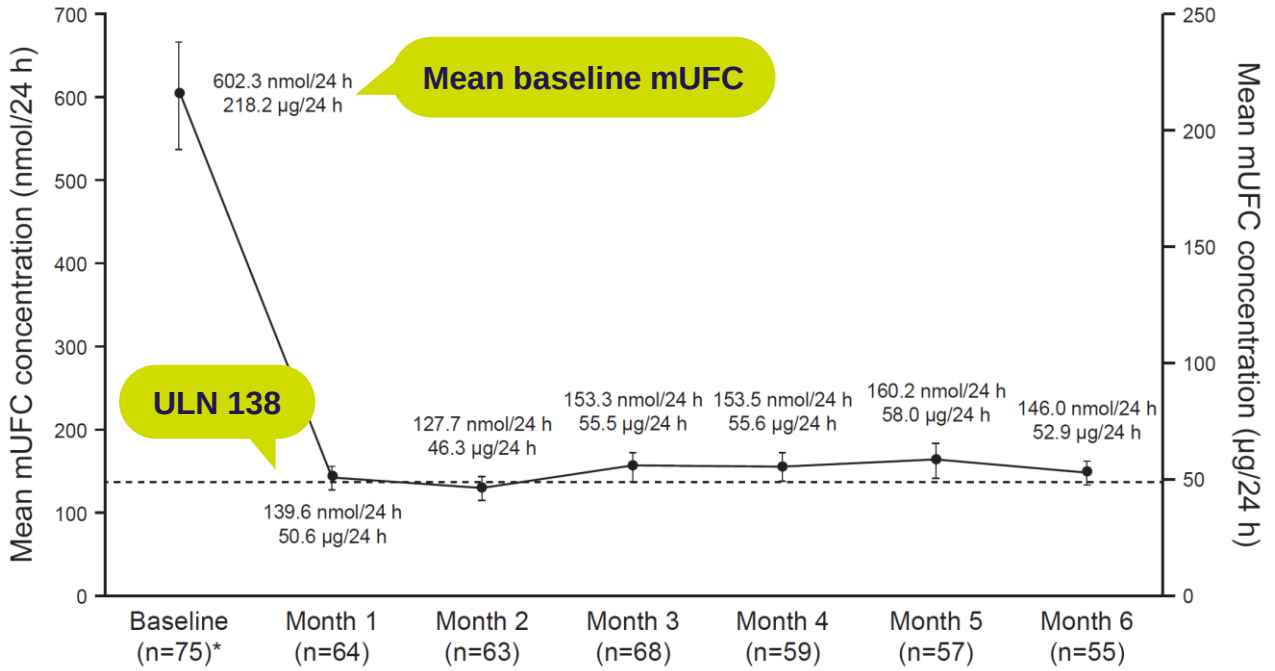



Change in individual mUFCs from baseline of the dose titration phase to the end of the maintenance phase (month 6; maintenance completers population [N=61]). Doses are from day 1 of the maintenance phase. Horizontal line represents ULN.

*Based on mixed-effects, repeated-measures model with underlying binomial distribution and logit link function, adjusted for baseline covariates.
**Abbreviations: CI= Confidence interval; ITT= Intent to Treat population; mUFC= mean urinary free cortisol; ULN= upper limit of normal

# Sensitivity analyses of SONICS primary endpoint support efficacy demonstration

## Mean mUFC remained at approximately the upper limit of normal (ULN) from month 1 through month 6 of maintenance (N=77)*




## Sensitivity analyses

**36%** (34/94)
mUFC normalization at month 6 irrespective of dose increase

**46%** (43/94)
≥50% mUFC decrease or normalization at month 6 irrespective of dose increase

**62%** (34/55)
Maintenance phase completers with mUFC data and mUFC normalization at month 6 irrespective of dose increase*

**78%** (43/55)
Maintenance phase completers with mUFC data and ≥50% mUFC decrease or normalization at month 6 irrespective of dose increase*

mUFC= mean urinary free cortisol level
ULN= upper limit of normal

*Data based on 77 maintenance phase subjects. Error bars represent Standard Error of the mean.

# SONICS achieved statistical significance in key secondary endpoints

## 5 KEY CARDIOVASCULAR (CV) SECONDARY ENDPOINTS WITH FAVORABLE CHANGES FROM BASELINE

| Outcome Measure | Baseline Mean (n) | Mean Change from Baseline at end of Maintenance phase† (n) | Adjusted* p-value of mean reductions from Baseline |
|---|---|---|---|
| **Fasting Blood Glucose** | 5.8 mmol/L (76) | -0.7 (50) | <0.0001 |
| **Hemoglobin A1c** | 6.0% (77) | -0.4 (55) | <0.0001 |
| **Total cholesterol** | 5.6 mmol/L (75) | -1.1 (53) | <0.0001 |
| **LDL-cholesterol** | 3.3 mmol/L (75) | -1.0 (53) | <0.0001 |
| **Body Weight** | 82.1 kg (77) | -5.1 (54) | <0.0001 |

HDL-cholesterol decreased by a mean of 0.2 mmol/L, an unfavorable mean change from baseline outweighed by the LDL-cholesterol mean improvement

Mean improvements in Hemoglobin A1c and fasting blood glucose were **more pronounced** among patients with diabetes mellitus

Mean scores for **quality of life (QoL), hirsutism, acne, peripheral edema and depression all significantly improved** at end of maintenance

*Hochberg adjustment applied to p-values to control type 1 error
†Mean reductions from baseline based on least squares mean changes from repeated-measures model adjusted for baseline covariates

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# Most commonly reported treatment emergent adverse events in SONICS

| Treatment Emergent Adverse Events (all phases combined) | N=94 |
|---|---|
| Nausea | **33%** |
| Headache | **29%** |
| Hypertension | **19%** |
| Peripheral edema | **19%** |
| Fatigue | **18%** |
| ALT increased* | **17%** |
| Diarrhea | **16%** |
| Arthralgia | **15%** |

Treatment-emergent events with incidence ≥15%

| Treatment Emergent Adverse Events of Special Interest (all phases combined) | |
|---|---|
| Liver-related | **7%** |
| QTc prolongation | **7%** |
| Adrenal insufficiency | **3%** |

*Includes all alanine aminotransferase (ALT) increases reported as an adverse event regardless of level or relationship to drug. A subset of these ALT increased events was also reported as adverse events of special interest.

The safety and efficacy of Recorlev (levoketoconazole) for treatment of endogenous Cushing's syndrome has not been established.

# Liver enzyme comparison

| | RECORLEV | KETOCONAZOLE* |
|---|---|---|
| **ALT >3X ULN** (includes those > 5x ULN) | 10.6% | 19% |
| **ALT >5x ULN** | 3.2% | 13% |
| **Total bilirubin values > 1.5x ULN** | 0% | NA |
| **Liver warning & precautions** | To be determined | Boxed warning & monitoring |
| **Monitoring** | SONICS Monitoring Protocol<br>At least once every two weeks during dose titration.<br>Monitored monthly for six months after the therapeutic dose is established; then every three months thereafter. | During the course of treatment, serum ALT should be **monitored weekly for the duration of treatment**. If ALT values increase to a level above the upper limit of normal or 30 percent above baseline, or if the patient develops symptoms, ketoconazole treatment should be interrupted. *** |

**Note: Data not from within the same study**

* Young et al. Eur J Endocrinol. 2018 Feb 22. pii: EJE-17-0886. doi: 10.1530/EJE-17-0886. [Epub ahead of print]; data from a substudy with patient who had no KTZ use in prior 4 weeks; ALT n=31.
*** Ketoconazole Prescribing Information;
Abbreviations: ALT, alanine aminotransferase; ULN, upper limits of normal; NA, Not Available

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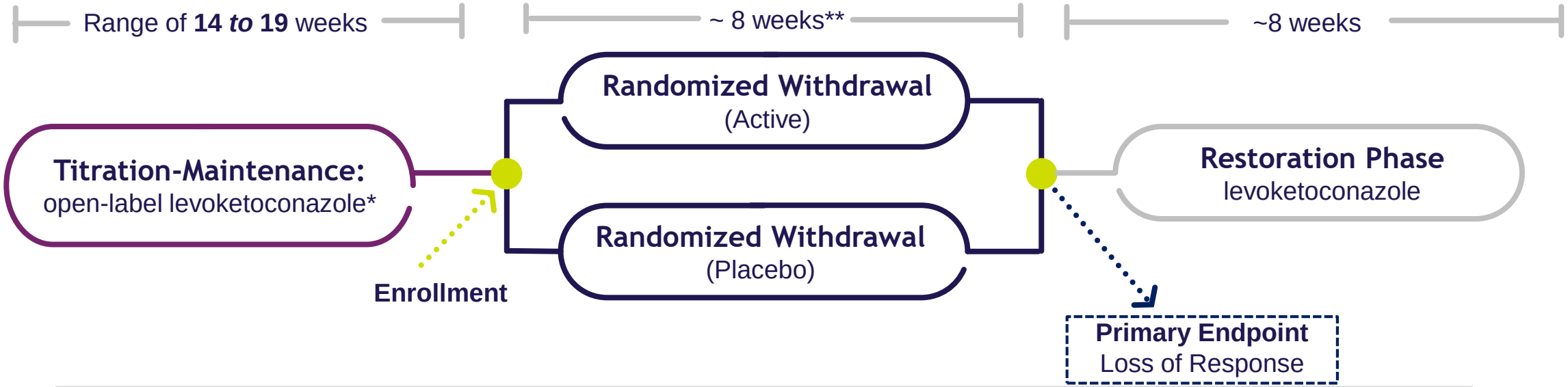
# LOGICS: a phase 3 placebo-controlled randomized withdrawal trial




## Design

Double-blind, placebo-controlled, randomized-withdrawal study intended to assess the efficacy and safety of levoketoconazole

## Study Rationale

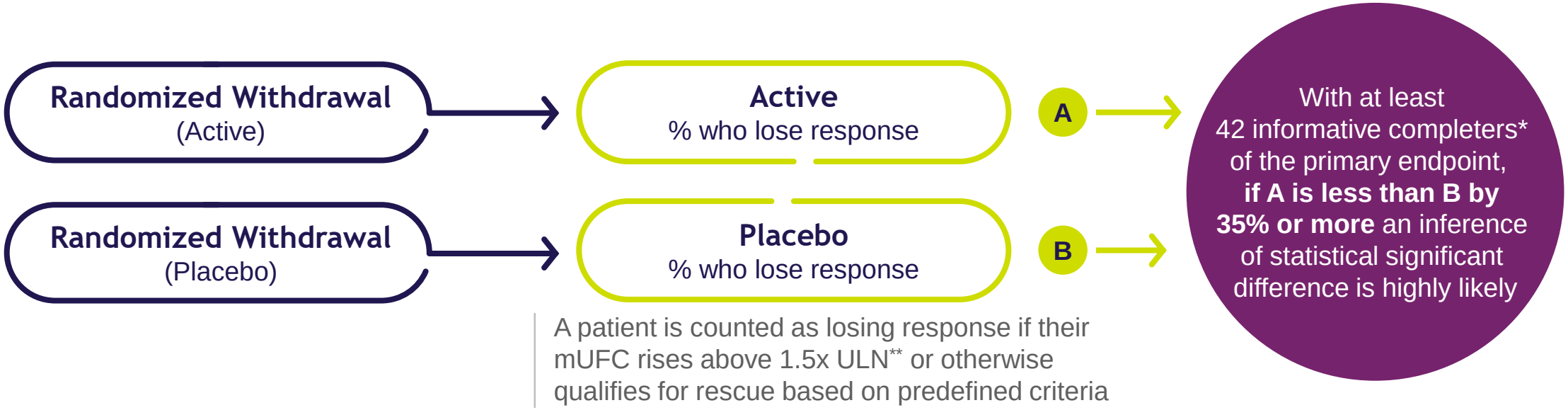
FDA had requested placebo-controlled data

## Primary endpoint

Comparison of the proportion of subjects with loss of therapeutic response upon withdrawing to placebo versus continuing treatment with levoketoconazole

* Subjects who directly rolled over into LOGICS from SONICS and were on a stable therapeutic dose for 12 weeks prior to screening did not require titration-maintenance
** Early rescue can happen at any time during randomized withdrawal

# LOGICS study sample size and power




A patient is counted as losing response if their mUFC rises above 1.5x ULN** or otherwise qualifies for rescue based on predefined criteria

With **43-44** enrolled

Expected to yield **42-43** completers*

**study power exceeds** **99%**

based on the null hypothesis of no difference in Loss of Response rate as compared with the alternative hypothesis of a 65% absolute Loss of Response rate difference

* Completers through at least the 4th visit in the randomized withdrawal phase. Currently 41 of 42 patients have completed the randomized withdrawal phase.
** Or mUFC more than 40% above the baseline (RW0) value for a patient from SONICS with RW0 value that is above ULN
Abbreviations: mUFC= mean urinary free cortisol; ULN=upper limit of normal

# Recorlev status and next steps

## LOGICS Top-line results
anticipated in Q3 2020

## LOGICS enrollment* to close on or before May 14, 2020 (n=43 or 44)

41 of 42 targeted study participants completed the randomized withdrawal phase; one additional patient is currently in the randomized withdrawal phase and one other patient is scheduled to be randomized imminently

## NDA submission
~6 months following reporting of top-line results

Pursuing 505(b)(2) approval pathway for a new active substance

10 month review expected

## Labeling

Certain parts of the Recorlev label concerning safety (e.g. toxicology) will derive from the current ketoconazole tablets label, potentially supplemented by newer information in the public domain and original data generated by Strongbridge

Clinical descriptions in the Recorlev label will necessarily reflect the clinical experience with Recorlev in CS patients at the time of NDA review, as the ketoconazole tablets label is specifically intended to guide prescribing of an antifungal treatment (not Cushing's syndrome)

*Defined by the number of patients who have been randomized (i.e. enrolled) towards a target randomization of 46 to 54 patients

The safety and efficacy of Recorlev (levoketoconazole) for treatment of endogenous Cushing's syndrome has not been established.

# RECORLEV MARKET ASSESSMENT



The safety and efficacy of Recorlev (levoketoconazole) for treatment of endogenous Cushing's syndrome has not been established.

# Cushing's syndrome (CS) market assessment and key findings

## Strongbridge CS Market Assessment

### Landscape Assessment + Primary Research

**Secondary Research**
*- Informed the market overview and competitive assessment*

**Analog Review**
*- Informed market dynamics and pricing assumptions*

**Qualitative HCP Research**
- *13 Endocrinologists*
- *Community and KOLs*
- *Avg. number of CS patients last 6mo's = 12 – 62*

**Quantitative HCP Research**
- *153 Endocrinologists*
- *Community and KOLs*
- *Avg. number of CS patients last 6mo's = 25-68*

**Qualitative Payer Research**
- *10 Payers*
- *Mix of National / Regional*
- *Avg. covered lives = 25M*

## Key Findings

**MARKET LANDSCAPE**

*of* **~ 8,000 patients**

**Endocrinologists interest in new therapies suggests**

**UNMET NEEDS EXIST**

**Results indicate Recorlev** has a

**POTENTIALLY COMPETITIVE PROFILE**

**Payers viewed Recorlev profile favorably**

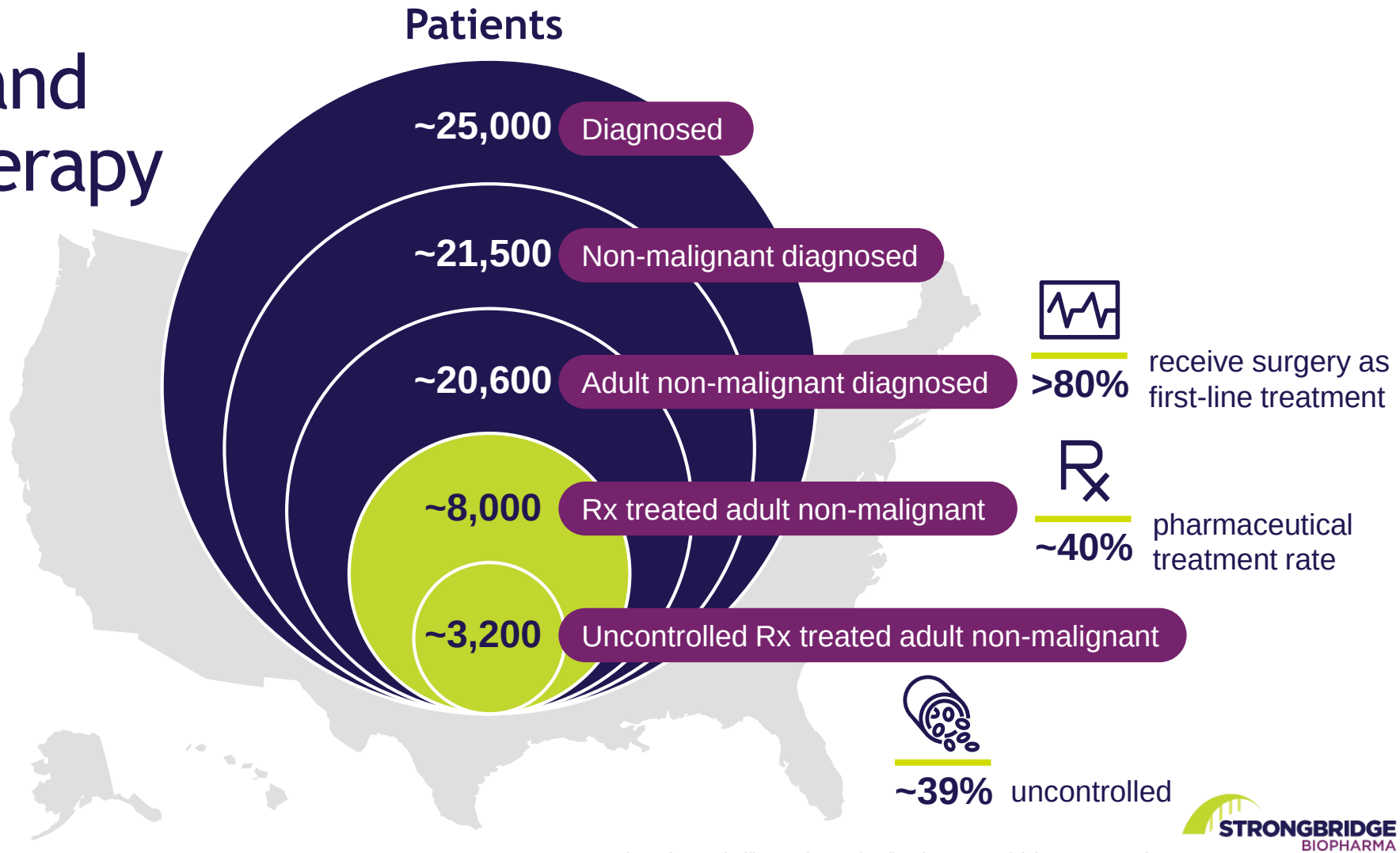
**PAYER ACCESS**



Source: Company sponsored research

The safety and efficacy of Recorlev (levoketoconazole) for treatment of endogenous Cushing's syndrome has not been established.

# U.S. Cushing's syndrome prevalence and pharmacotherapy landscape




Source: Secondary literature and company sponsored research
A07. Of your endogenous Cushing's patients currently receiving pharmacological therapy, what percent would you consider have their symptoms controlled vs. uncontrolled by their medication(s) for CS?

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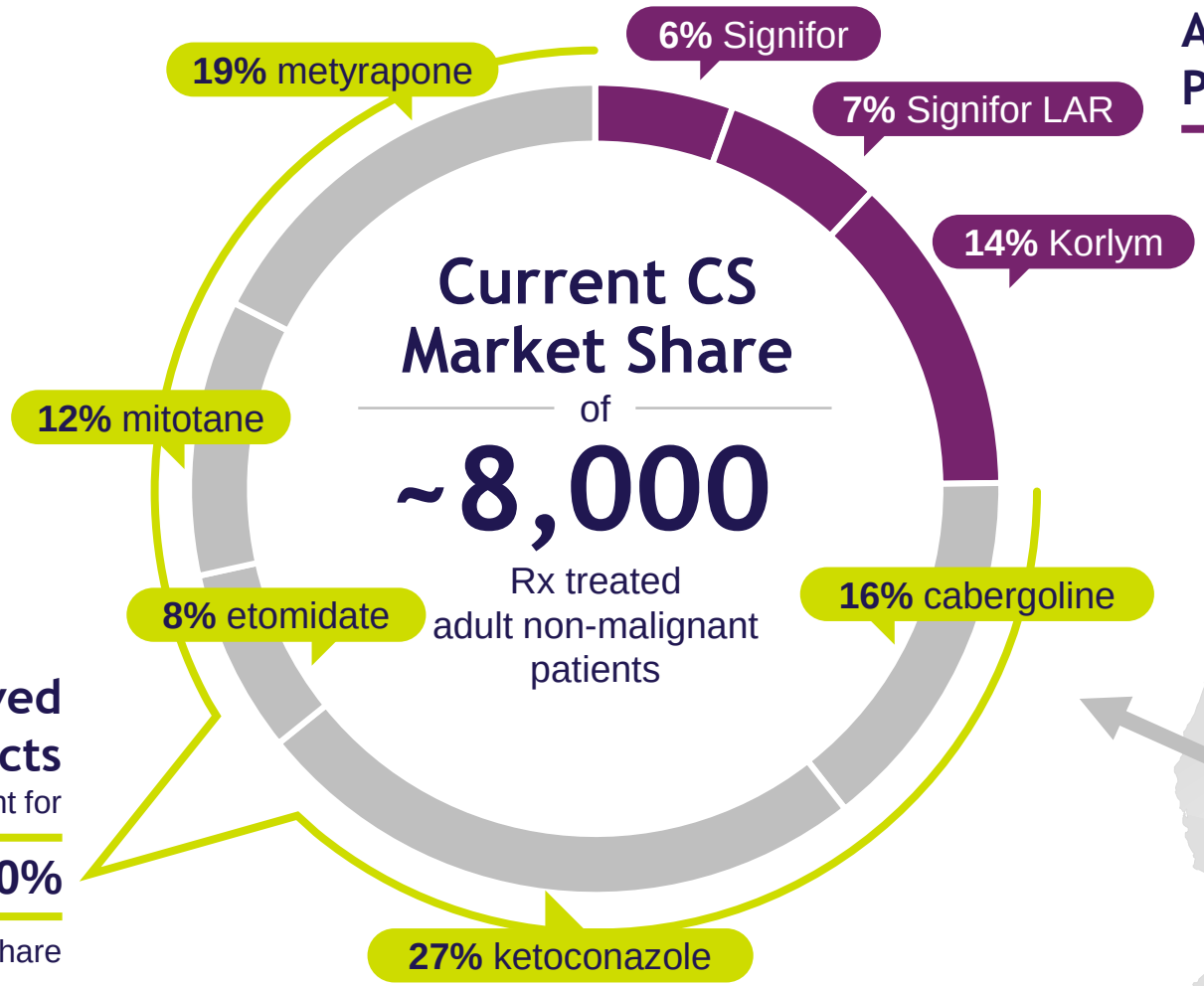
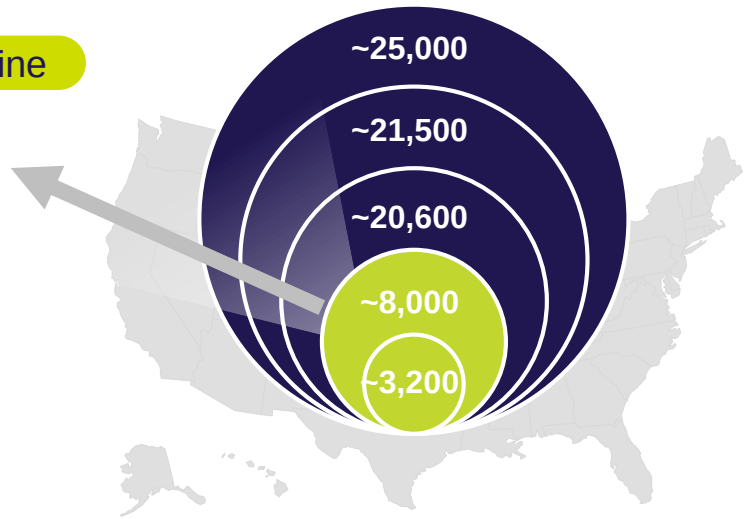
# Current Cushing's syndrome product market shares




**Note: Total utilization greater than 100% due to combination therapy**

Prevalence of Endogenous Cushing's syndrome in US

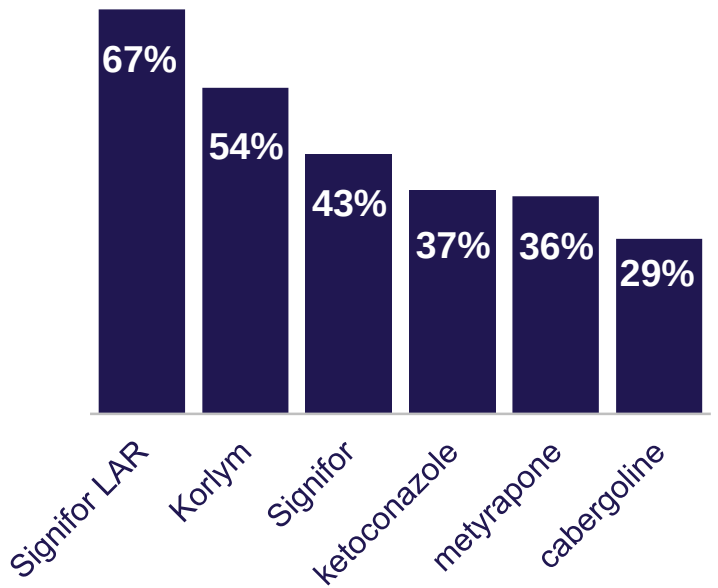



Signifor and Signifor LAR are owned by Recordati; Korlym is owned by Corcept
Source: Company sponsored research
B08. Of your endogenous Cushing's syndrome patients currently receiving a pharmacological therapy pre-surgery, what percent are receiving each pharmacological therapy listed below?
B09. Of your endogenous Cushing's syndrome patients currently receiving a pharmacological therapy post-surgery – 1st line, what percent are receiving each pharmacological therapy listed below?
B10. Of your endogenous Cushing's syndrome patients currently receiving a pharmacological therapy post-surgery – 2nd line or later, what percent are currently receiving each pharmacological therapy listed below?

# Endocrinologists interest in new treatment options

## Reported Product Satisfaction Level

% Reporting Satisfaction
**Level 7-9****




n=37 to 90 endocrinologists

## Endocrinologists Express a High Level of Interest in New Treatments

**9** | Extremely interested

Not at all interested **1** |


**81%** of endocrinologists have moderate to high interest in new treatments for CS

## Controlled vs. Uncontrolled on Pharmacological Therapy

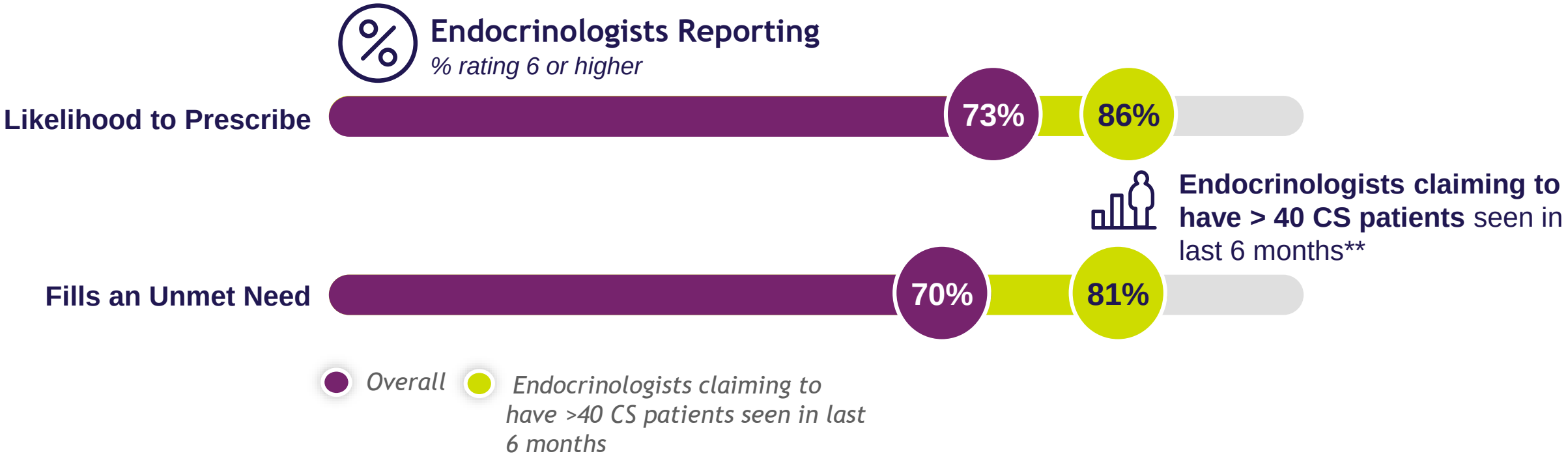

Source: Company sponsored research

B03. For each of the pharmacological therapies you currently use to treat endogenous Cushing's syndrome today, please rate your overall level of satisfaction with the ability of the following pharmacological therapies to manage endogenous Cushing's syndrome, using a 9-point scale where 1 = "Not at All Satisfied" and 9 = "Extremely Satisfied".

A07. Of your endogenous Cushing's patients currently receiving pharmacological therapy, what percent would you consider have their symptoms controlled vs. uncontrolled by their medication(s) for CS?

B15. In general, how interested are you in new treatments for endogenous Cushing's syndrome? Please rate on a 9-point scale, where 1 is "Not at all interested" and 9 is "Extremely interested".

# Endocrinologists reaction to the Recorlev target product profile




Source: Company sponsored research
D03. Based on this profile, what is your likelihood to prescribe Product Y? Please rate on scale from 1-9, with 1 being "Not at all likely" and 9 being "Very likely".
D02. To what extent does Product Y fill an unmet need in the treatment and management of endogenous Cushing's syndrome? Please rate on scale from 1-9, with 1 being "Not at all" and 9 being "Very much".
**Not statistically significant

# Recorlev and ketoconazole product profiles*

| | RECORLEV | KETOCONAZOLE |
|---|---|---|
| **Indication** | Anticipated labeling for the treatment of CS | Indicated as a last line anti-fungal;<br>*FDA label warns that the use of ketoconazole in Cushing's syndrome has not been approved* |
| **Clinical Data** | Will be well characterized in two Phase 3 clinical trials | Not well-studied prospectively in CS |
| **Liver Safety** | In SONICS, 3.2% of patients had an ALT elevation >5x ULN | In a registry study** of 47 keto-naïve patients, 13% had an ALT elevation > 5x ULN |
| **Liver Monitoring Scheme** | In SONICS, measured at least 1x every 2 weeks during dose titration; monthly for 6 months after therapeutic dose is established; every 3 months thereafter | FDA label indicates weekly liver monitoring |
| **Patient & Prescriber Support** | Fully leverage current Care Connection patient support program and planned specialty pharmacy distribution with expertise in Recorlev pharmacology and labeled monitoring scheme | No manufacturer support provided |
| **Dosage & Administration** | SONICS/LOGICS studied doses from 150 mg once daily up to 600 mg twice daily; Median treatment duration in SONICS was 383 days | 400-mg max dose, 200-mg strength, once daily; limited 6-month course |

* The data set forth above is not based on directly comparable trials and/or studies

** Source: 1. Young et al. Eur J Endocrinol. 2018 Feb 22. pii: EJE-17-0886. doi: 10.1530/EJE-17-0886. [Epub ahead of print]



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# Current branded Cushing's syndrome therapy pricing


| Annual Wholesale\ Acquisition Cost | SIGNIFOR/SIGNIFOR LAR | KORLYM |
|---|---|---|
| | ~$165k | ~$189k – ~$755k* |

Current analogues in CS category

Source: Red Book, Signifor Prescribing Info, Signifor LAR Prescribing Info, Korlym Prescribing Info as of January 2020
* Korlym has weight-based dosing

The safety and efficacy of Recorlev (levoketoconazole) for treatment of endogenous Cushing's syndrome has not been established.

# Branded CS products' coverage

| | KORLYM | | | SIGNIFOR | | |
|---|---|---|---|---|---|---|
| **Plan** | **Coverage** | **Prior Authorization** | **Step Edit*** | **Coverage** | **Prior Authorization** | **Step Edit*** |
| **Aetna** | Specialty | ✓ | | Specialty | ✓ | |
| **CVS Caremark** | Non-preferred | ✓ | ✓ | Non-preferred | ✓ | ✓ |
| **Express Scripts** | Non-preferred | ✓ | | Preferred** | ✓ | |
| **Humana** | Non-preferred | ✓ | Info not available | Non-preferred/Not covered | ✓ | Info not available |
| **Cigna** | Non-preferred | ✓ | Info not available | Non-preferred | ✓ | Info not available |
| **United Healthcare** | Non-preferred | ✓ | | Non-preferred | ✓ | |
| **Blue Cross** | Non-preferred/Specialty | ✓ | | Non-preferred/Specialty | ✓ | |
| **Premier Health** | Specialty | ✓ | ✓ | Specialty | ✓ | |
| **Medical Mutual** | Specialty | ✓ | ✓ Step through generic or Signfor | Specialty | ✓ | |
| **Geisinger** | Preferred | ✓ | | Preferred | ✓ | |

**PA CRITERIA CAN INCLUDE**

Must be prescribed by an endo | Must have failed/not be a candidate for surgery | Must have confirmed endogenous Cushing's diagnosis

Source: Finger Tip Formulary and Health Plan Drug Policies as of 2019
* Must have failed or be intolerant to ketoconazole, metyrapone, and/or etomidate
** LAR on 2020 exclusion list

The safety and efficacy of Recorlev (levoketoconazole) for treatment of endogenous Cushing's syndrome has not been established.

# Summary results of Recorlev payer research

Payers viewed Recorlev Sonics' clinical efficacy and safety benefits profile favorably

Payers expressed initial willingness to provide coverage throughout a tested price range of $200K - $400K

Payers expect to use existing utilization management restrictions to ensure only appropriate patients receive access

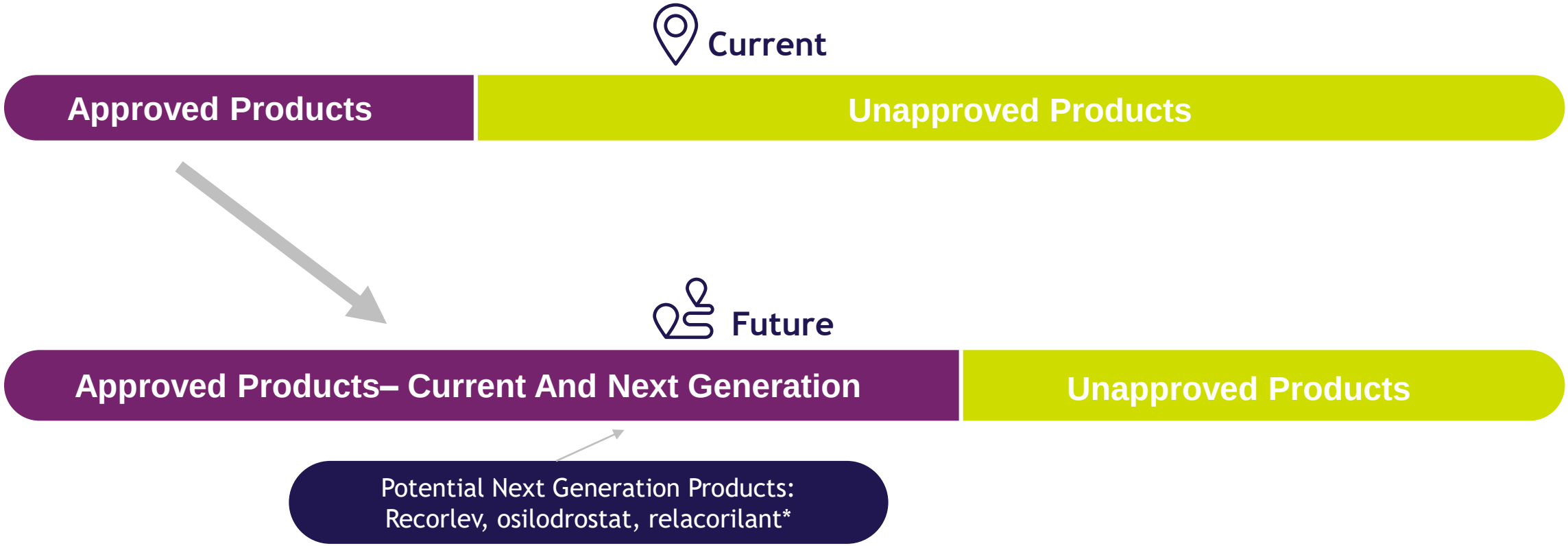
In more highly restrictive payers, new product entries may be subjected to step-edit requirement

## How interested would you be to cover/include on formulary?


Source: Company sponsored research

The safety and efficacy of Recorlev (levoketoconazole) for treatment of endogenous Cushing's syndrome has not been established.

# Next generation products are expected to lead the market in the future




Source: Company sponsored research
* Next generation products were deidentified in the market research

# Upon approval, fully leverage existing Strongbridge infrastructure and rare disease experience to commercialize Recorlev

## AWARENESS & EDUCATION

- **Maximize existing MSL team** KOL Relationships
- Foster established **Advocacy Group relationships**
- Identify and profile centers of excellence

## MARKETING & ANALYTICS

- **Leverage data and analytics** to target key endocrinologists
- Establish Recorlev as a significantly differentiated next generation CS treatment option
- Communicate Recorlev's unique efficacy, safety, and QoL profile

## FIELD FORCE

- Prioritize target customer segments relative to keto usage
- Target high volume CS practices
- Engage key centers
- **Opportunity to leverage current field team**

## ACCESS & PATIENT SUPPORT

- Engage top payers to create additional choices for endocrinologists
- **Leverage CareConnection suite of patient services to educate providers and patients**
- Establish forums that support patient interactions and empowerment

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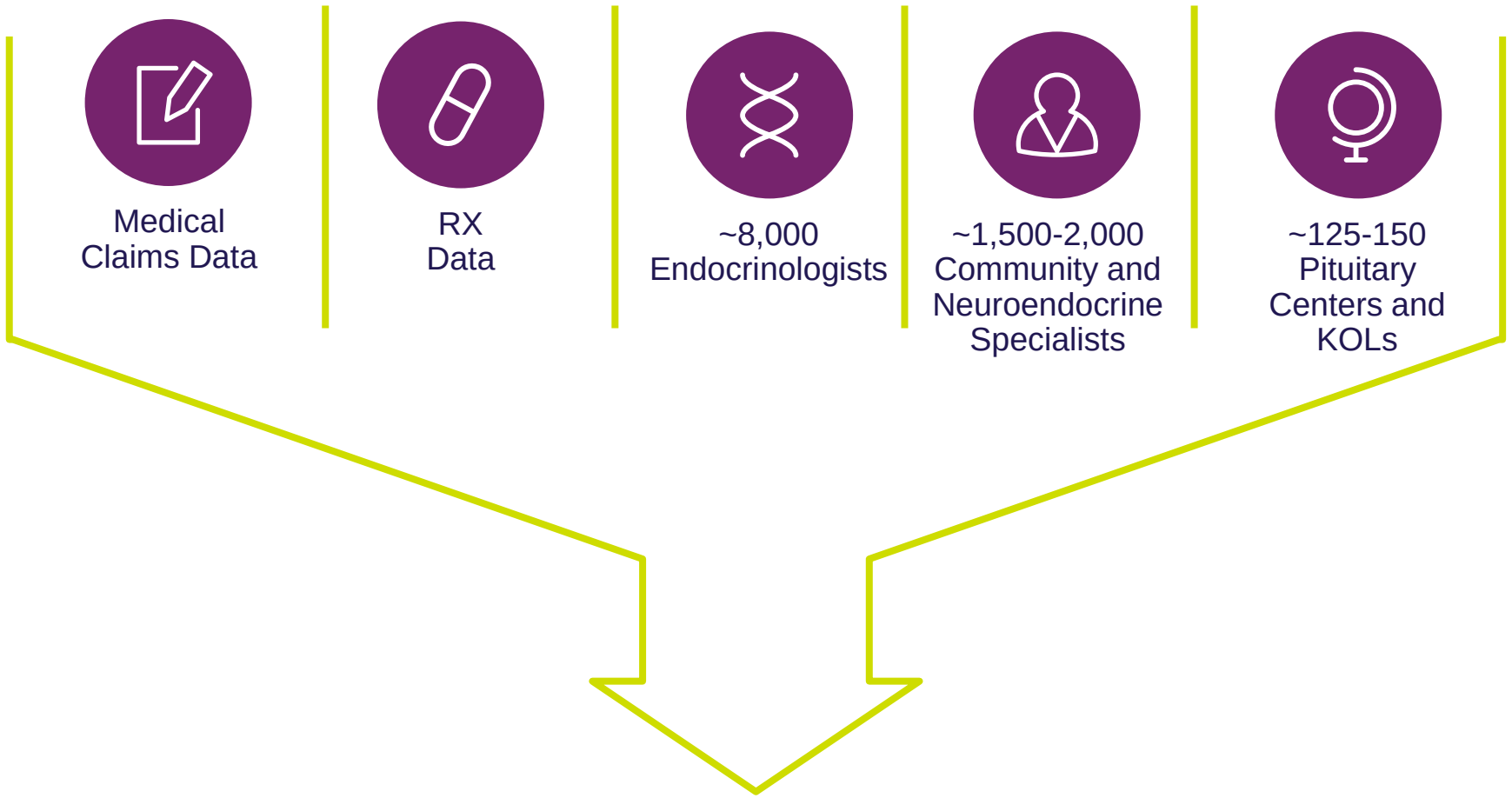
# Cushing's syndrome stakeholder targeting approach




*Customer facing positions may include sales, access managers, and MSLs

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# KEVEYIS
(dichlorphenamide)

The first and only FDA-approved therapy for primary periodic paralysis*

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

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

## PPP

Causes recurrent, progressive, and debilitating episodes of muscle weakness and temporary paralysis[2-4]

## Symptoms/Triggers

**Symptoms**
clumsiness, extreme fatigue, weakness, palpitations, pain

**Triggers**
potassium, carbohydrates, rest after exercise, cold exposure, stress

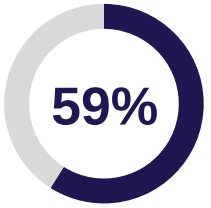
## Impact of Attacks

Paralytic attacks are acute episodes that can be debilitating[4]

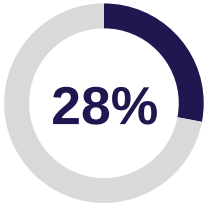
Attacks may last from one hour to several days[1]

As patients age, muscle weakness can become permanent[3]

## Frequency




have **weekly** attacks




have **daily** attacks


1. Charles G, Zheng C, Lehmann-Horn F, Jurkatt-Rott, Levitt J. Characterization of hyperkalemic periodic paralysis: a survey of genetically diagnosed individuals. J Neurol. 2013;260:2606-2613.
2. 2. Cannon SC. Channelopathies of skeletal muscle excitability. Compr Physiol. 2015;5:761-790.
3. 3. Cavel-Greant D, Lehmann-Horn F, Jurkat-Rott K. The impact of permanent muscle weakness on quality of life in periodic paralysis: a survey of 66 patients. Acta Myol. 2012;31:126-133.
4. 4. Sansone V, Meola G, Links TP, Panzeri M, Rose MR. Treatment for periodic paralysis. Cochrane Database Syst Rev. 2008; Jan 23;(1):CD005045.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# Keveyis approved for the treatment of PPP in the US

**~4,000 - 5,000***

diagnosed PPP patients in the United States

## The first and only FDA-approved therapy

indicated for the treatment of primary hyperkalemic and hypokalemic periodic paralysis and related variants

## Dosing in an oral tablet formulation

Starting dose is

**50 mg**

**1X *or* 2X daily**

Can be titrated up to

**100 mg**

**2X daily**

*Based on Strongbridge analysis of medical claims database.
Identified patients were required to have medical activity of any type in the last 12-36 months.

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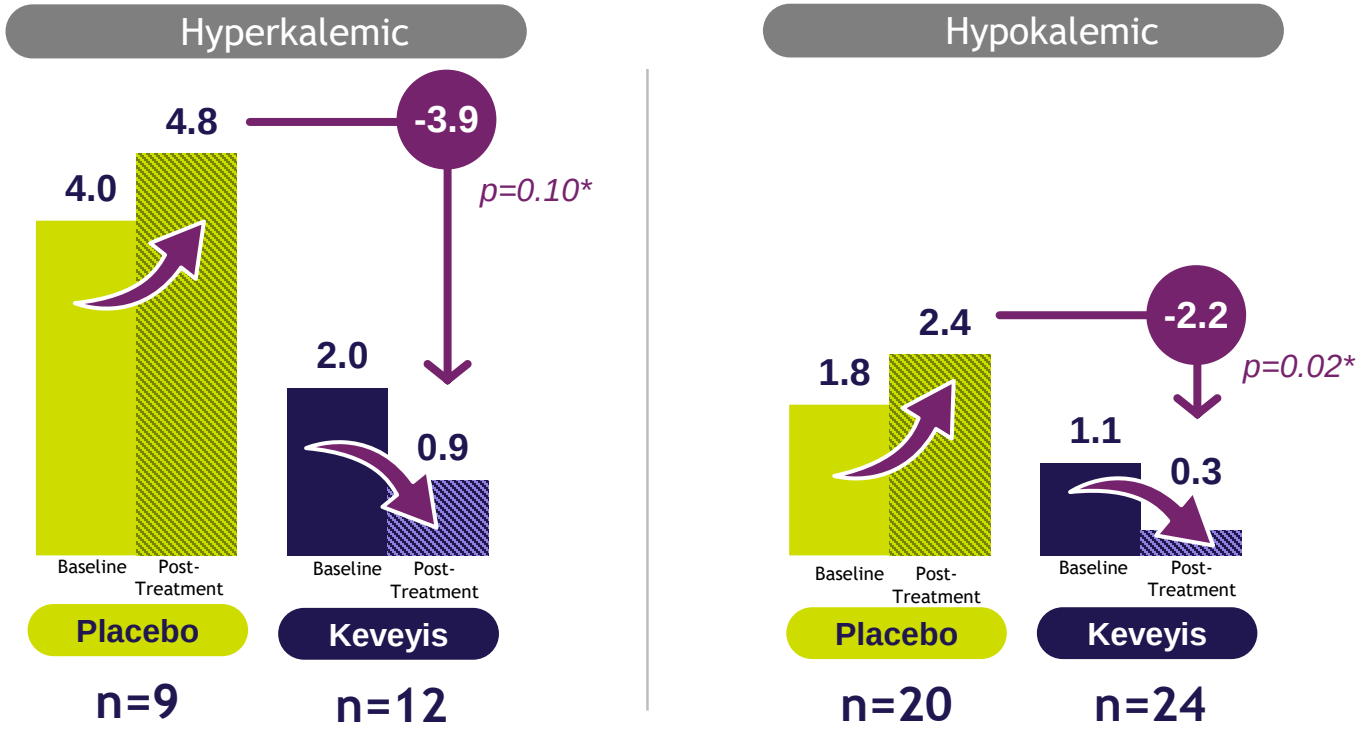
# Treatment with Keveyis decreased weekly attack rates

## Study 1

### Decreased weekly attack rates from baseline to week 9




## Study 2

### 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

# Our commitment to primary periodic paralysis

STRONGBRIDGE™ CareConnection

**Suite of Patient Services**

- Personalized Support
- Community Connection
- Access Assistance
- Education & Public Awareness
- Uncovering Periodic Paralysis Genetic Testing

**29-MEMBER CUSTOMER FACING* TEAM DEPLOYED**

*Customer facing positions include sales, access managers, and MSLs

# Strategic priorities & revenue guidance

## Continue to Drive Revenue Growth

and increase positive contribution margin

## Pursue Life Cycle Opportunities

to extend exclusivity runway beyond 2022

14 US patent applications filed

## 2020 Revenue guidance of $22-$26 Million

Building off 2019 revenue of $21.7 Million

Q1 2020 revenue of $6.7M up 54% from Q1 2019

# IP & FINANCIALS

# Intellectual property and orphan exclusivity

| | Intellectual Property | | Orphan Exclusivity | |
|---|---|---|---|---|
| | US | EU | US | EU |
| RECORLEV | 2026 – Method of use: treating Cushing's syndrome | 2026 – Method of use: treating Cushing's syndrome | 7 years | 10 years |
| VELDOREOTIDE | 2037 – Modified release formulation | Filed patent application for modified release formulation | 7 years | 10 years |
| KEVEYIS | Filed 14 patent applications | Strongbridge has US rights only | Aug **2022** | Strongbridge has US rights only |

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# Summary financial information*

**~$63M**
in cash, cash equivalents, and marketable securities

No debt

**~54.2M**
shares outstanding

**Cash runway**
through
**3Q 2021**

**2019**
revenue of
**$21.7 million**
for Keveyis
*29% growth from 2018*

**2020**
full-year Keveyis revenue guidance of
**$22–$26 million**

* As of 03/31/2020

# Strongbridge COVID-19 Response

## Corporate

All employees successfully working virtually since March 9th

Our offices will remain closed until at least May 31

## R&D

Extreme efforts and innovative measures from the clinical team, investigators, our CRO, and vendors are mitigating potential impacts of COVID-19 on LOGICS

Innovative measures include home health visits, special transportation arrangements, telemedicine, direct-to-patient drug supply delivery, and regulatory authority-sanctioned protocol and monitoring flexibility

## Commercial

Field team is working virtually and adapting to novel ways of seeking new patients

Focused effort on retaining existing base of patients has been successful to date

# 2020 key priorities

## 2020 Key Priorities

**RECORLEV®** (levoketoconazole)

- **Report top-line results for the Phase 3 LOGICS study of RECORLEV™ in endogenous Cushing's syndrome in Q3 2020**
- **Submit an NDA for Recorlev to the U.S. FDA approximately 6 months after reporting top-line LOGICS results**

**KEVEYIS®** dichlorphenamide 50 mg tablets

- **Execute on life cycle opportunities underway for KEVEYIS**
- **Continue growing the revenue and contribution margin of Keveyis and achieve or exceed $22-26 million revenue guidance**

**Corporate**

- **Manage expenses to achieve or exceed runway guidance of having cash through at least Q3 2021**
- **Continue search for a CEO**

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# STRONGBRIDGE BIOPHARMA PLC