
Akebia®
THERAPEUTICS
A FULLY INTEGRATED BIOTECH COMPANY FOCUSED ON KIDNEY DISEASE
John P. Butler
President and CEO
(Nasdaq: AKBA)

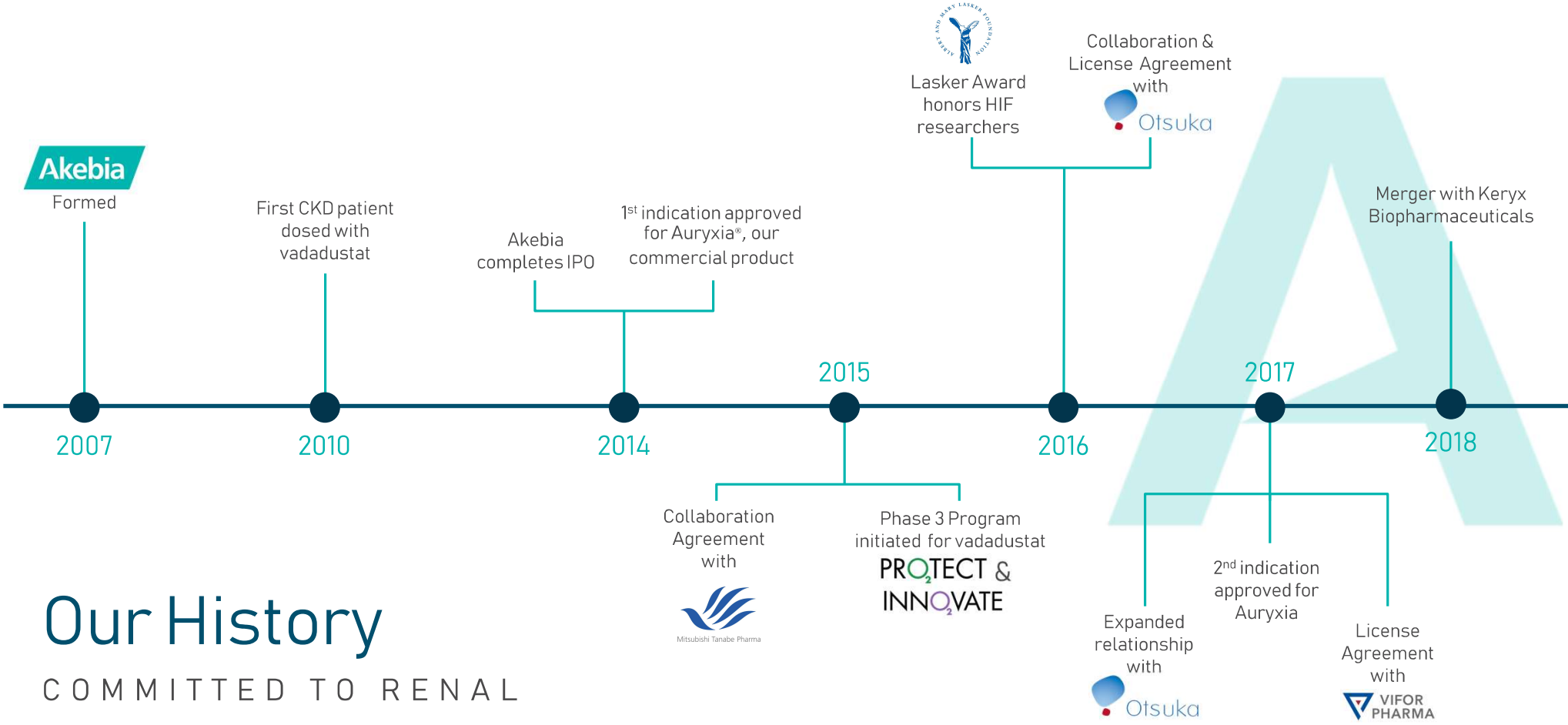
CAUTIONARY NOTE ON FORWARD-LOOKING STATEMENTS

Statements in this presentation regarding Akebia's strategy, plans, prospects, expectations, beliefs, intentions or goals are forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including but not limited to statements regarding the expected period of time our resources can fund operations, and the components of such resources; the optimization of cash resources; the timing, availability and presentation of clinical trial data and results; development, launch, commercial availability and the commercial potential, growth potential and market opportunity for our product and, if approved, our product candidates, and the drivers, timing, impact and results thereof; our strategy, mission and objectives; potential for our product candidates to set a new standard of care; the potential benefits of our product candidates; our relationships with the nephrology community; and the anticipated timing of preclinical, clinical and regulatory developments and the design, timing and results of our studies; the assessments and evaluations we expect from our clinical programs. The terms "estimate," "expect," "growth," "momentum," "mission" "opportunity," "positioned," "potential," "vision" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Each forward-looking statement is subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement, including the actual product revenues for Auryxia (ferric citrate); the timing of generic entrants for Auryxia, vadadustat or any other product candidates; the rate of enrollment in clinical studies of vadadustat; the risk that clinical trials may not be successful; the risk that existing preclinical and clinical data may not be predictive of the results of ongoing or later clinical trials; manufacturing risks; the quality and manner of the data that will result from clinical studies of vadadustat; the actual funding required to develop and commercialize our product candidates and operate the company, and the actual expenses associated therewith; efficacy, safety and tolerability of our products and product candidates; the risk that clinical studies need to be discontinued for any reason, including for safety, tolerability, enrollment, manufacturing or economic reasons; early termination of any of Akebia's collaborations or license agreements, and the parties' ability to satisfy their obligations under such agreements; the timing and content of decisions made by regulatory, judicial or similar authorities; the timing of any additional studies initiated for vadadustat; the actual time it takes to initiate and complete research and clinical studies; the success of competitors in developing product candidates for diseases for which we are currently developing our product candidates; the scope, timing, and outcome of any ongoing legal proceedings; changes in the economic and financial conditions of our or our partners' businesses; and our ability to obtain, maintain and enforce patent and other intellectual property protection for Auryxia, vadadustat and our other product candidates. Other risks and uncertainties include those identified under the heading "Risk Factors" in Akebia's Quarterly Report on Form 10-Q for the quarter ended September 30, 2019, filed with the SEC, and other filings that Akebia may make with the SEC in the future. These forward-looking statements (except as otherwise noted) speak only as of the date of this presentation, and Akebia does not undertake, and specifically disclaims, any obligation to update any forward-looking statements contained in this presentation. Vadadustat is an investigational drug and has not yet been approved by the U.S. Food and Drug Administration (FDA) or any regulatory authority.

Akebia THERAPEUTICS

OUR PURPOSE

To Better the Life of Each Person Impacted by Kidney Disease



Our History

COMMITTED TO RENAL

Vadadustat is an investigational HIF PH inhibitor that is not approved by the FDA or any other regulatory authority.


2019 MILESTONES
Positive Phase 3 Study Results for Vadadustat in Japan
Expanded License Agreement with
VIFOR PHARMA
JNDA Filing for Vadadustat (Japan)
Secured
$100M
Non-Dilutive Term Loan
Funding into Q1 2021*
CLINICAL LEADERSHIP
Full enrollment of global Phase 3 studies for vadadustat: INNO2VATE and PRO2TECT
AURYXIA PATENT SETTLEMENT
Reinforces strength of intellectual property
Nobel Prize in Medicine
KERYX
Integration of Keryx

2019


2020 A DEFINING YEAR FOR AKEBIA
The countdown to our global Phase 3 data for vadadustat has begun!
INNOVATE
TOPLINE DATA EXPECTED Q2 2020*
INNO2VATE enrolled 3,923 DD-CKD patients
PROTECT
TOPLINE DATA EXPECTED MID 2020*
PRO2TECT enrolled 3,513 NDD-CKD patients
ANTICIPATE APPROVAL OF VADADUSTAT IN JAPAN
JNDA for marketing approval of vadadustat submitted in July 2019
PRE-COMMERCIALIZATION ACTIVITIES UNDERWAY
Leveraging existing nephrology-focused commercial organization to increase disease awareness and aligning manufacturing resources

2020

2019 MILESTONES

Positive Phase 3 Study Results for Vadadustat in Japan

Mitsubishi Tanabe Pharma

Expanded License Agreement with

VIFOR PHARMA

Mitsubishi Tanabe Pharma

JNDA Filing for Vadadustat (Japan)

Secured

$100M

Non-Dilutive Term Loan

Funding into Q1 2021*

CLINICAL LEADERSHIP

Full enrollment of global Phase 3 studies for vadadustat: INNO$_2$VATE and PRO$_2$TECT

AURYXIA PATENT SETTLEMENT

Reinforces strength of intellectual property

Nobel Prize in Medicine

Awarded to HIF Researchers

KERYX BIOPHARMACEUTICALS, INC

Integration of Keryx

* Includes $145.6 million of cash, cash equivalents and available for sale securities as of 9/30/19, $100 million tranched term loan, committed research and development funding from collaborators and receipt of a regulatory milestone from MTPC, assuming approval of vadadustat in Japan.

2020 A DEFINING YEAR FOR AKEBIA

The countdown to our global Phase 3 data for vadadustat has begun!

INNO$_2$VATE

TOPLINE DATA EXPECTED Q2 2020*

INNO$_2$VATE enrolled 3,923 DD-CKD patients

PRO$_2$TECT

TOPLINE DATA EXPECTED MID 2020*

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Mitsubishi Tanabe Pharma

ANTICIPATE APPROVAL OF VADADUSTAT IN JAPAN

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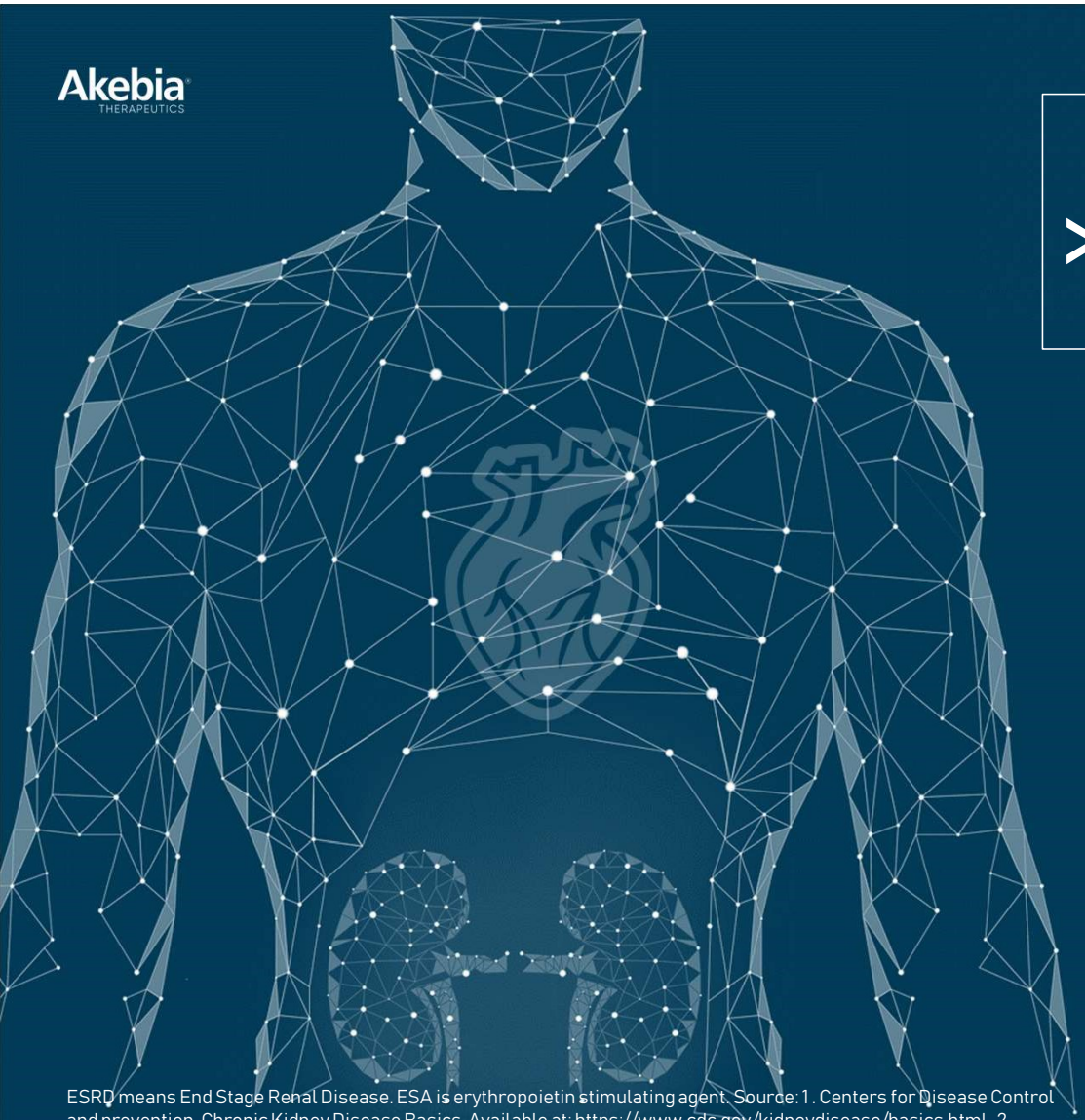
Leveraging existing nephrology-focused commercial organization to increase disease awareness and aligning manufacturing resources

*Subject to accrual of MACE.

Akebia THERAPEUTICS



KIDNEY DISEASE COSTS US MEDICARE

>$120B

annually [1]

PATIENT POPULATION

5.7M

People in the US with anemia due to CKD [2]

ANEMIA DUE TO CKD: Unmet Needs

BURDEN OF DISEASE

Quality of life: fatigue, weakness, dizziness, shortness of breath.

CLINICAL IMPACT

Anemia due to CKD can contribute to risk of ESRD, Cardiovascular (CV) disease, stroke, cognitive impairment, CV-related complications and death

CURRENT STANDARD OF CARE
Consists of injectable ESAs, which have been associated with significant CV risk

ESRD means End Stage Renal Disease. ESA is erythropoietin stimulating agent. Source: 1. Centers for Disease Control and prevention. Chronic Kidney Disease Basics. Available at: https://www.cdc.gov/kidneydisease/basics.html. 2. Stauffer et al. PLOSONE, 2014.

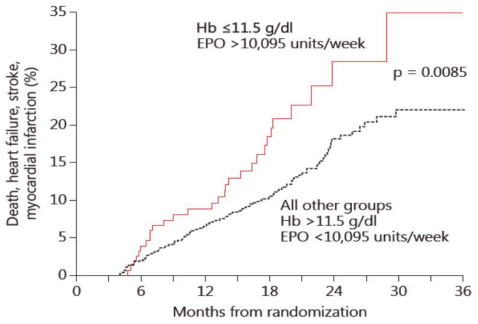
LIMITATIONS of ESAs

in Treating and Managing Anemia Due to CKD

HIGH EPO LEVEL IS ASSOCIATED WITH INCREASED CV RISK

Kaplan-Meier Survival Curves[1]

Death, Heart Failure, Stroke, Myocardial Infarction

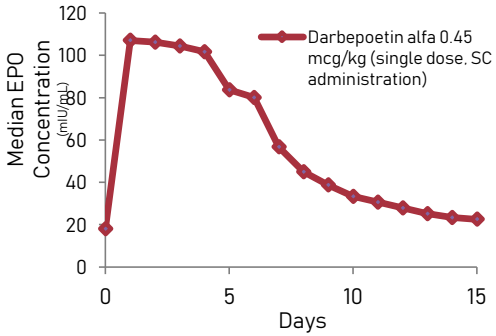


Study Methods: post hoc analysis from the CHOIR trial. Inclusion criteria were Hb <11.0 g/dl and eGFR of 15–50 ml/min/1.73 m². To be included in the present analysis, subjects needed to be free of the composite event at 4 months, receive epoetin-alfa, and have ≥1 postbaseline Hb measurement.

ESAs RESULT IN SUPRA-PHYSIOLOGICAL EPO LEVELS

EPO vs Time with Darbepoetin Alfa (SC)

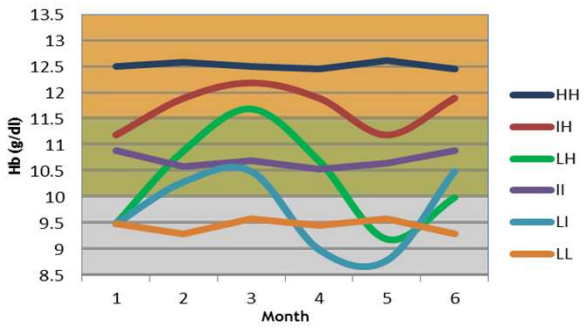
PK-PD Model in CKD Subjects[2]



HGB VARIABILITY REMAINS[3]

Variability in Hemoglobin Levels in Hemodialysis Patients in the Current Era[3]

Figure 1. Idealized example of Hb variability groups



Distribution of demographics and comorbidity by Hb variability groups in 2012

	Overall	LL	II	HH	LI	IH	LH
Total patients, N	200,728	2,200	18,999	10,552	48,029	60,525	60,423

Study Methods: The study population consisted of maintenance HD patients as of October 1, 2012, with Medicare as primary payer during the baseline period (April 1 – September 30, 2012). Monthly Hb values were categorized as low (L), intermediate (I), or high (H), where L and H were based on monthly Hb values below or above the 25th and 75th percentiles, respectively. Hb variability was then classified into six groups based on the lowest and highest category during the six month observation period (LL, consistently low; II, consistently intermediate; HH, consistently high; LI, low-intermediate; IH, intermediate-high; LH, low-high).

HGB is hemoglobin. eGFR is estimated glomerular filtration rate. EPO is erythropoietin.. Sources: 1. McCullough PA et al. Am J Nephrol 2013;37:549-558 (DOI:10.1159/000351175). Permission granted by S. Karger AG, Basel.; 2. Doshi S et al. J Clin Pharmacol. 2010;50(9 Suppl):75S-90S (DOI: 10.1177/0091270010377201). Original figure redrawn to depict darbepoetin alfa serum concentration (ng/mL/(mcg/kg)) converted to mU/mL. Data from 6 clinical studies conducted with extensive PK sampling in CKD patients following subcutaneous (SC) administration of a single dose or first dose of a monthly dosing regimen ranging from 0.4-0.6mcg/kg, dose normalized to 0.45 mcg/kg.; 3. Chronic Disease Research Group. Gilbertson D et al. Variability in Hemoglobin Levels in Hemodialysis Patients in the Current Era. Presented at: American Society of Nephrology Kidney Week; Nov 2015; San Diego, CA. Funded by Akebia Therapeutics, Inc.

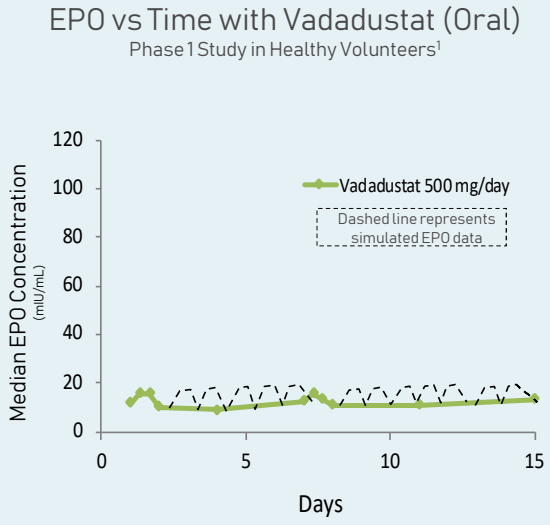
Akebia THERAPEUTICS

VADADUSTAT

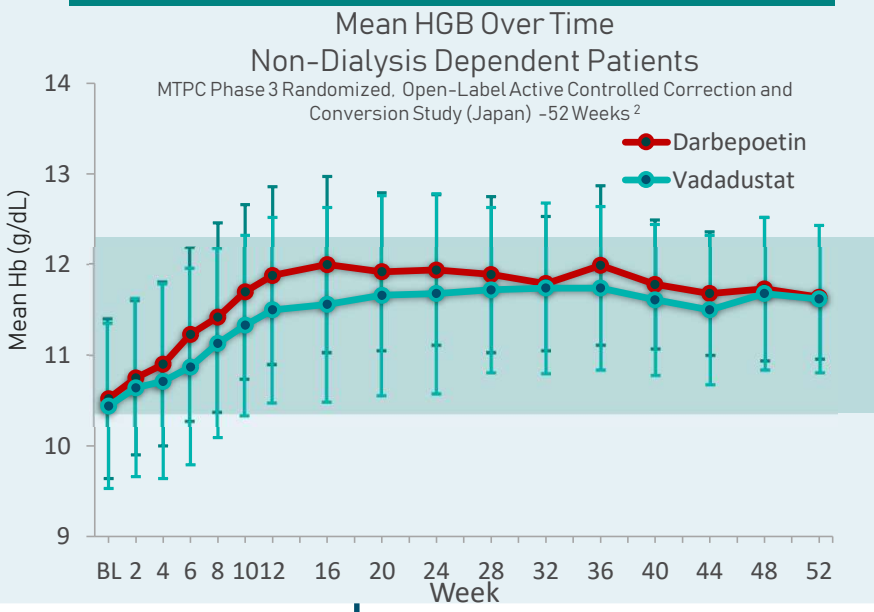
An investigational oral HIF PH inhibitor designed to stimulate endogenous EPO production

Sources: 1. Akebia Therapeutics, Inc. Data on File (2010). Data from Phase 1 study in healthy volunteers with vadadustat once daily dosing for 10 days. Pre-dose EPO concentrations were evaluated on Days 1, 4, 7, 11, 15 and 22. Post-dose data to assess acute rise in EPO following vadadustat dosing was only completed on Day 1 and Day 7 (8 and 16 hours post-dose). Dashed line represents estimated EPO levels based on post-dose data from Day 1 and Day 7.; 2. Nangaku M et al. Randomized, Open-Label, Active-Controlled (Darbepoetin Alfa), Phase 3 Study of Vadadustat for Treating Anemia in Nondialysis-Dependent Chronic Kidney Disease Patients in Japan. Presented at: American Society of Nephrology Kidney Week; Nov 9 2019; Washington, DC. (Mitsubishi Tanabe Pharma Corporation's (MTPC) Phase 3 randomized, open-label, active-controlled correction and conversion study assessed the efficacy and safety of vadadustat compared to darbepoetin alfa in 304 Japanese non-dialysis dependent subjects with anemia due to CKD, with a treatment duration of 52 weeks.)

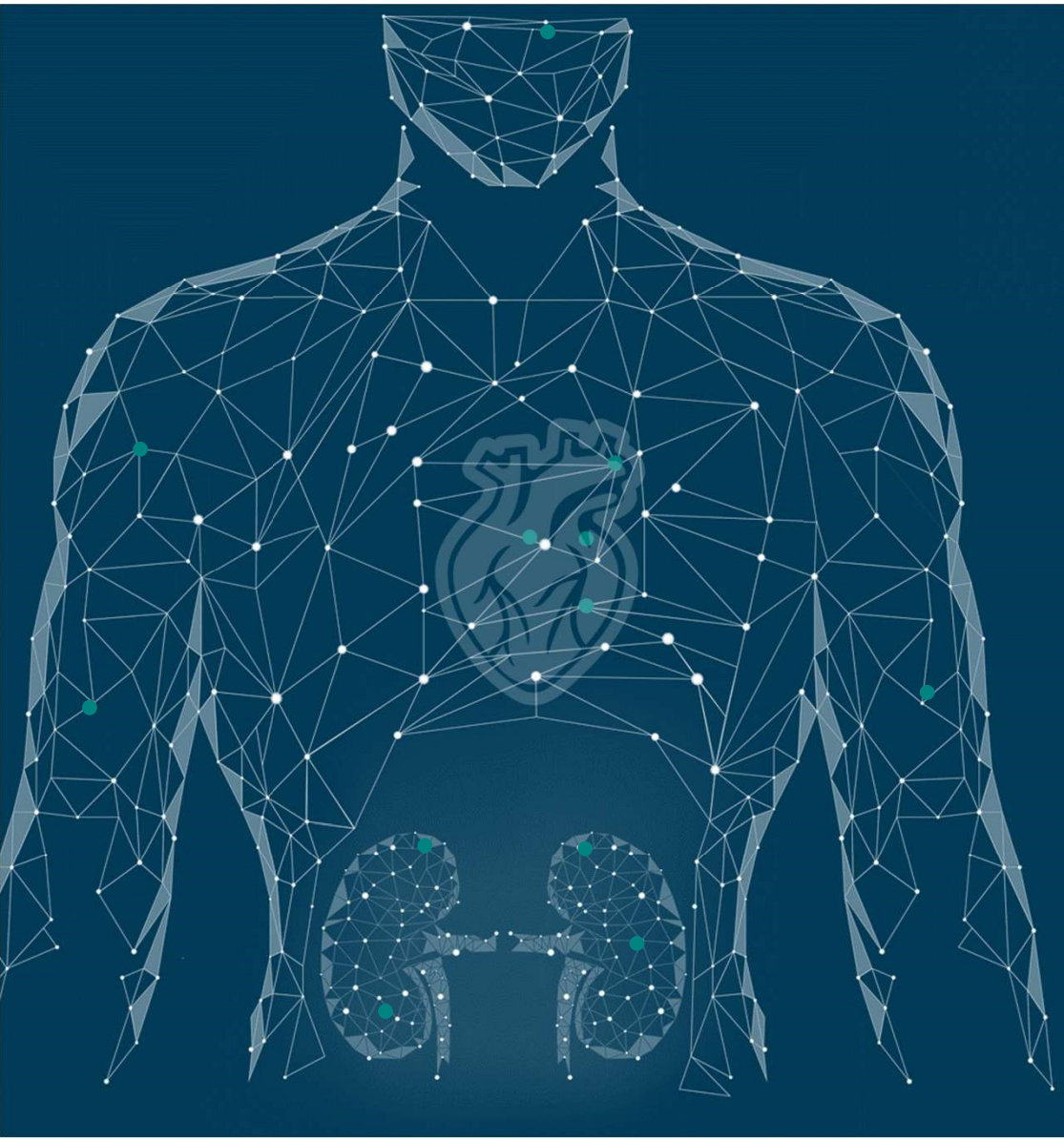
VADADUSTAT AVOIDED SUPRA-PHYSIOLOGICAL EPO LEVELS



VADADUSTAT'S EFFECT ON HEMOGLOBIN (HGB) WAS SUSTAINED THROUGH TO 52 WEEKS



Vadadustat's ongoing Phase 3 cardiovascular outcomes trials for anemia due to CKD in non-dialysis and dialysis patients are now fully enrolled and will be analyzing these parameters. Vadadustat is an investigational HIF PH inhibitor that is not approved by the FDA or any other regulatory authority.



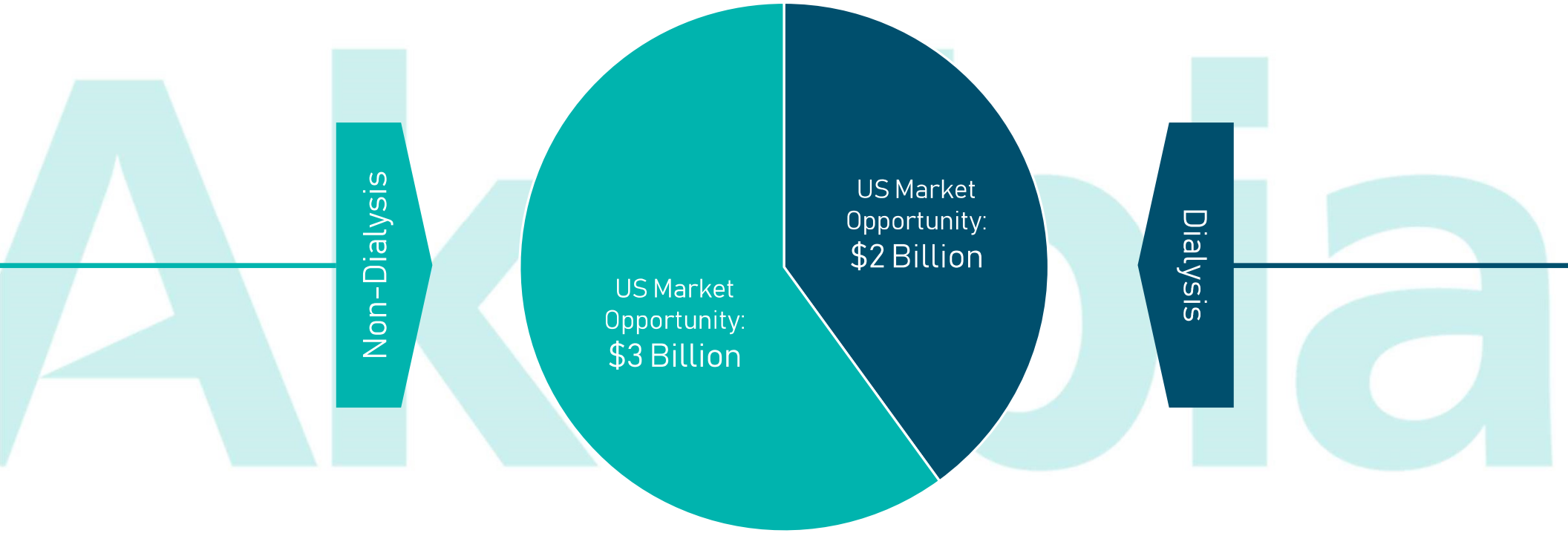
VADADUSTAT

POTENTIAL NEW ORAL STANDARD OF CARE FOR ANEMIA DUE TO CKD

Unique focus and potential opportunity to address unmet needs with a differentiated safety profile compared with the standard of care

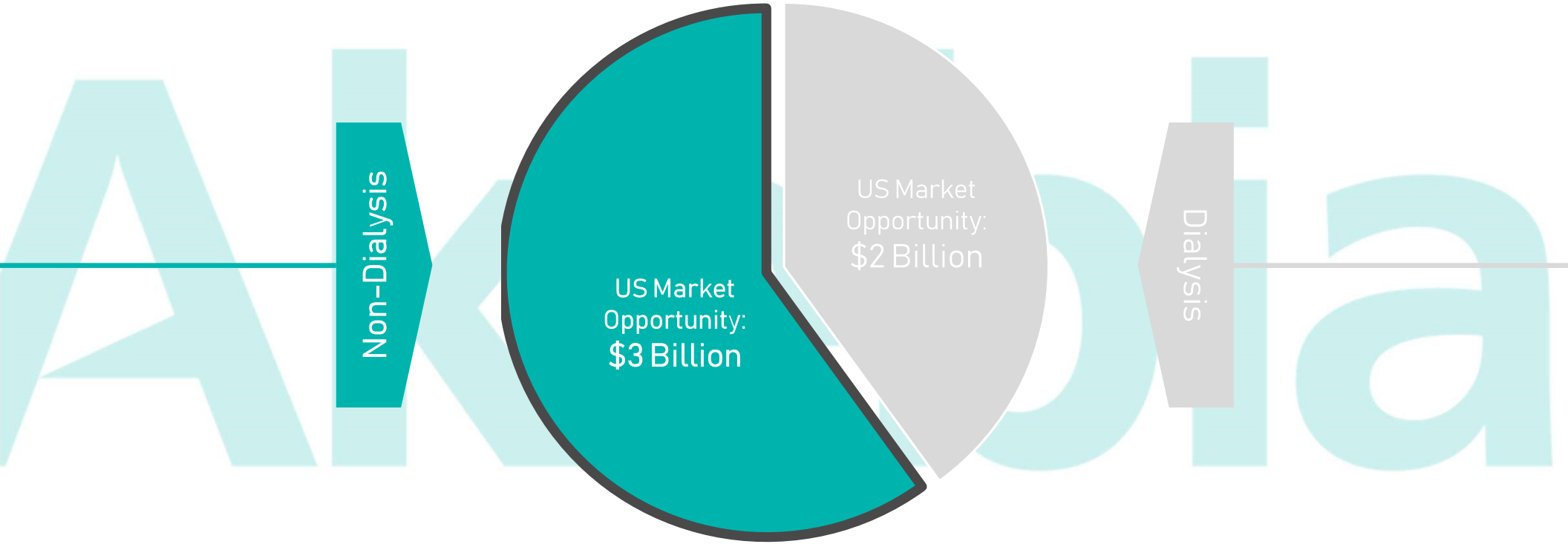
Vadadustat's ongoing Phase 3 cardiovascular outcomes trials for anemia due to CKD in non-dialysis and dialysis patients are now fully enrolled. Vadadustat is an investigational HIF PH inhibitor that is not approved by the FDA or any other regulatory authority.

Focused on $5B+ MARKET OPPORTUNITY



Akebia THERAPEUTICS

Focused on $5B+ MARKET OPPORTUNITY



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Non-Dialysis

VADADUSTAT

Significant Opportunity to Address Unmet Needs of Non-Dialysis Dependent Patient Population with Anemia Due to CKD (US Market), Upon Approval

37 Million
People with CKD[1]

5.7 Million
People with anemia due to CKD[2]

CURRENT POPULATION

250K
Size of non-dialysis dependent patient population currently treated for anemia due to CKD[3]

POTENTIAL TO AT LEAST DOUBLE x2

POTENTIAL POPULATION

400–500K
Estimated size of addressable non-dialysis dependent patient population with anemia due to CKD[4]

$3 Billion

Estimated US Market Opportunity within Non-Dialysis Patient Population[4]

Vadadustat is an investigational HIF PH inhibitor that is not approved by the FDA or any other regulatory authority.

Slide shows estimated US market. Sources: 1. 2018 USRDS Annual Data Report: https://www.usrds.org/2018/download/v2_c01_IncPrev_18_web.xlsx. 2. Stauffer et al. PLOSONE, 2014. 3. Spherix RealWorld Dynamix Renal Anemia market research survey and chart review, Feb 2018. 4. Based on internal estimates for pts with Hb<11 and not treated with ESA and industry reports estimating ESA pricing.

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Non-Dialysis

To Have the Potential to Change the Standard of Care, You Must Compare to the Standard of Care.

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NDA/MAA CORE PACKAGE

Phase 3 Studies of Vadadustat for Treatment of Anemia due to CKD in Non-Dialysis Dependent Patients

FULLY ENROLLED

PROTECT CORRECTION
Not ESA Treated

Vadadustat vs
Darbepoetin Alfa

N ≈ 3,513

PROTECT CONVERSION
ESA Treated

Vadadustat vs
Darbepoetin Alfa

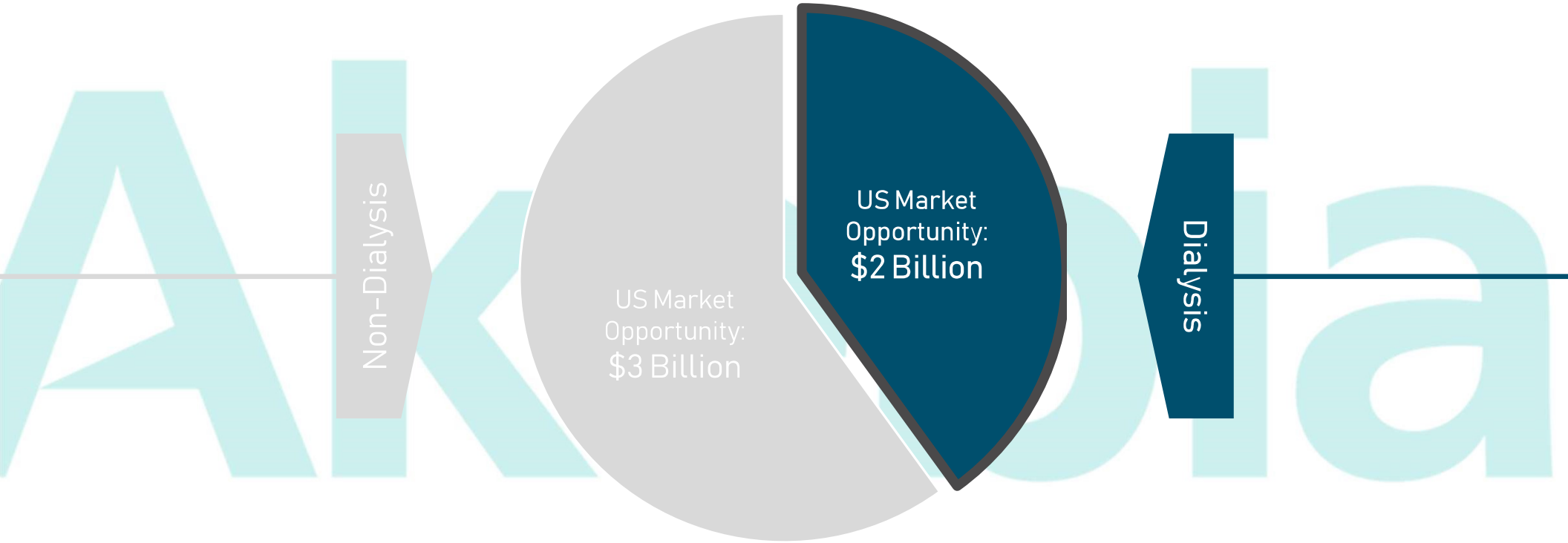
PRIMARY EFFICACY ENDPOINT:
Change in hemoglobin from baseline

PRIMARY SAFETY ENDPOINT:
Major Adverse Cardiovascular Events (MACE)

TOP-LINE RESULTS
Expected mid 2020*

*Subject to accrual of MACE.

Focused on $5B+ MARKET OPPORTUNITY



Akebia THERAPEUTICS

Dialysis

VADADUSTAT

Significant Opportunity to Address Unmet Needs of Dialysis Dependent Patient Population with Anemia Due to CKD (US Market), Upon Approval

37 Million
People with CKD[1]

5.7 Million
People with anemia due to CKD[2]

510K
Dialysis patient population[1]

90%
Of dialysis patient population is treated for anemia due to CKD[3]

$2 Billion
Estimated US Market Opportunity within Dialysis Dependent Patient Population[4]

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Slide shows estimated US market. Sources: 1. 2018 USRDS Annual Data Report: https://www.usrds.org/2018/download/v2_c01_IncPrev_18_web.xlsx. 2. Stauffer et al, PLOSONE, 2014. 3. Based on internal estimates and industry reports. 4. Based on internal estimates and industry reports estimating ESA pricing.

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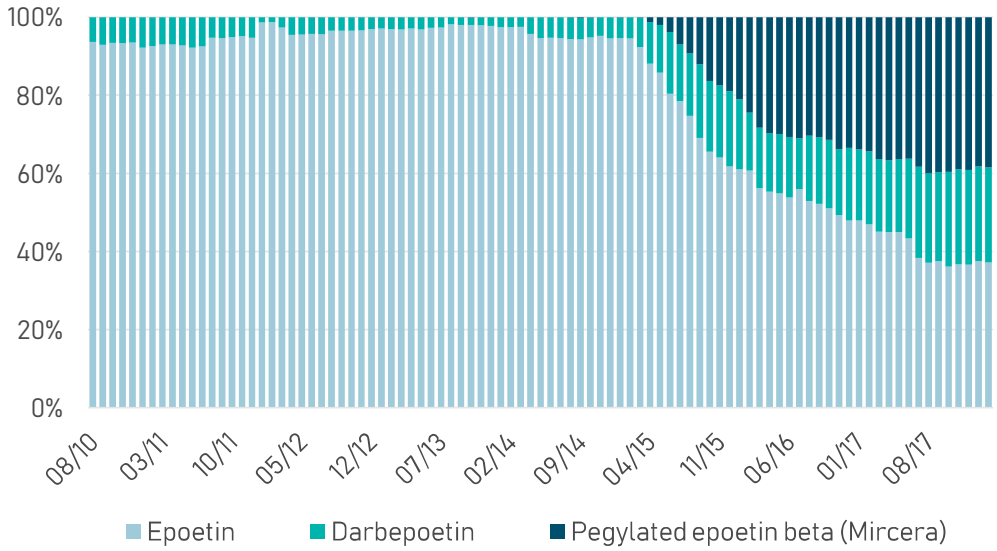
Dialysis

STRONG DISTRIBUTION CHANNEL to US Dialysis Network with VIFOR PHARMA

- Unique market dynamics with dialysis center clinical protocols
- Agreement with Vifor positions vadadustat for rapid uptake in Fresenius LDO subject to FDA approval*
- 2019 amendment expands agreement, facilitating access to up to 60% of US dialysis patients
- TDAPA[2] creates additional opportunity for value creation

Chart Shows Uptake of Mircera into Fresenius Dialysis Centers[1] which represent approx. 40% of US Dialysis Market



*Subject to the earlier of reimbursement under TDAPA (defined below) or inclusion in the ESRD bundle. Sources: 1 ESA Use, by type. DOPPS Practice Monitor. https://www.dopps.org/dpm/DPMSlideBrowser.aspx. Accessed 12/19/2018.; 2 TDAPA: Transitional drug add-on payment adjustment, CMS Ruling CMS-1691-F. Medicare Program; End-Stage Renal Disease Prospective Payment System, Payment for Renal Dialysis Services Furnished to Individuals with Acute Kidney Injury, End-Stage Renal Disease Quality Incentive Program, Durable Medical Equipment, Prosthetics, Orthotics and Supplies (DMEPOS) Competitive Bidding Program (CBP) and Fee Schedule Amounts, and Technical Amendments to Correct Existing Regulations Related to the CBP for Certain DMEPOS.

Dialysis

To Have the Potential to Change the Standard of Care, You Must Compare to the Standard of Care.

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NDA/MAA CORE PACKAGE

Phase 3 Studies of Vadadustat for Treatment of Anemia due to CKD in Dialysis Dependent Patients

FULLY ENROLLED

INNOVATE
CORRECTION
CONVERSION
New-Onset Dialysis

Vadadustat vs
Darbepoetin Alfa

N ≈ 3,923

INNOVATE
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ESA Treated

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Change in hemoglobin from baseline

PRIMARY SAFETY ENDPOINT:
Major Adverse Cardiovascular Events (MACE)

TOP-LINE RESULTS
Expected Q2 2020*

*Subject to accrual of MACE.

Design of Global Phase 3 Program

POSITIONS VADADUSTAT FOR SUCCESS

CLINICAL

Active control enables clear data readouts for efficacy and safety as compared with current standard of care

REGULATORY

Prospectively defined and agreed upon non-inferiority margin and key components of statistical analysis plan

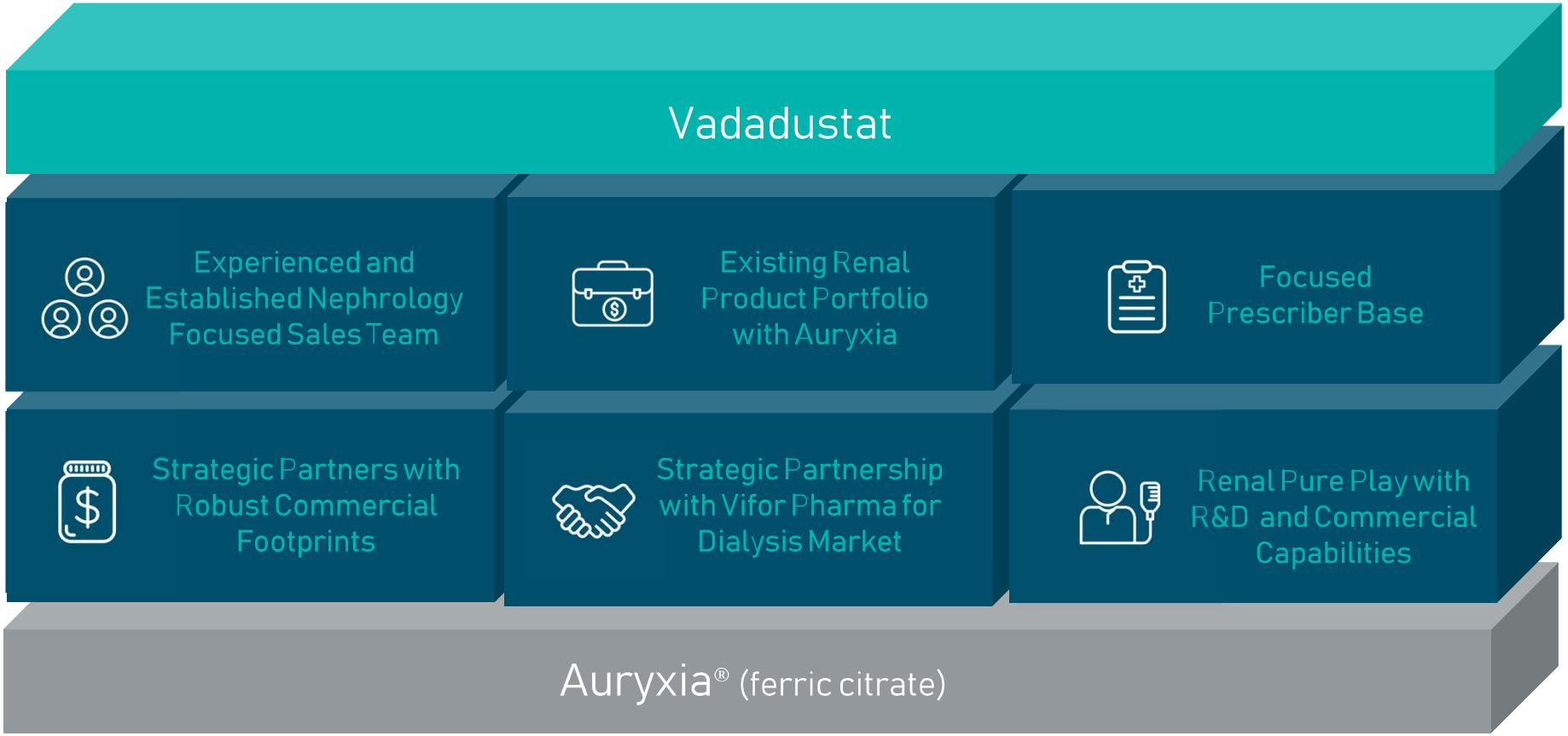
COMMERCIAL

Key secondary efficacy and safety endpoints to assess important areas of differentiation from current standard of care

Vadadustat's ongoing global Phase 3 cardiovascular outcomes trials for anemia due to CKD in non-dialysis and dialysis patients are now fully enrolled. Vadadustat is an investigational HIF PH inhibitor that is not approved by the FDA or any other regulatory authority.

Akebia® THERAPEUTICS

WELL POSITIONED UPON VADADUSTAT APPROVAL AND LAUNCH



2020 A Defining Year for Akebia

STRATEGIC OBJECTIVES

1 EXECUTE ON GLOBAL PHASE 3 DATA READOUT FOR VADADUSTAT

- Topline data for INNO$_2$VATE expected Q2 2020*
- Topline data for PRO$_2$TECT expected mid 2020*
- NDA/MAA readiness

2 SECURE APPROVAL OF VADADUSTAT IN JAPAN

3 DEEPEN RELATIONSHIPS WITH NEPHROLOGY COMMUNITY

4 OPTIMIZE CASH RESOURCES

*Subject to accrual of MACE.

Important Safety Information

CONTRAINDICATION

AURYXIA® (ferric citrate) is contraindicated in patients with iron overload syndromes, e.g., hemochromatosis

WARNINGS AND PRECAUTIONS

Iron Overload: Increases in serum ferritin and transferrin saturation (TSAT) were observed in clinical trials with AURYXIA in patients with chronic kidney disease (CKD) on dialysis treated for hyperphosphatemia, which may lead to excessive elevations in iron stores. Assess iron parameters prior to initiating AURYXIA and monitor while on therapy. Patients receiving concomitant intravenous (IV) iron may require a reduction in dose or discontinuation of IV iron therapy

Risk of Overdosage in Children Due to Accidental Ingestion: Accidental ingestion and resulting overdose of iron-containing products is a leading cause of fatal poisoning in children under 6 years of age. Advise patients of the risks to children and to keep AURYXIA out of the reach of children

ADVERSE REACTIONS

The most common adverse reactions reported with AURYXIA in clinical trials were:

Hyperphosphatemia in CKD on Dialysis: Diarrhea (21%), discolored feces (19%), nausea (11%), constipation (8%), vomiting (7%) and cough (6%)

Iron Deficiency Anemia in CKD Not on Dialysis: Discolored feces (22%), diarrhea (21%), constipation (18%), nausea (10%), abdominal pain (5%) and hyperkalemia (5%)

SPECIFIC POPULATIONS

Pregnancy and Lactation: There are no available data on AURYXIA use in pregnant women to inform a drug-associated risk of major birth defects and miscarriage. However, an overdose of iron in pregnant women may carry a risk for spontaneous abortion, gestational diabetes and fetal malformation. Data from rat studies have shown the transfer of iron into milk, hence, there is a possibility of infant exposure when AURYXIA is administered to a nursing woman

To report suspected adverse reactions, contact Akebia at 1-844-445-3799.
Please see full Prescribing Information
Learn more at AURYXIA.com.

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THERAPEUTICS
THANK YOU | QUESTIONS?