Diagnostics & Pharmaceuticals for Large Markets with Unmet Needs

September 2017

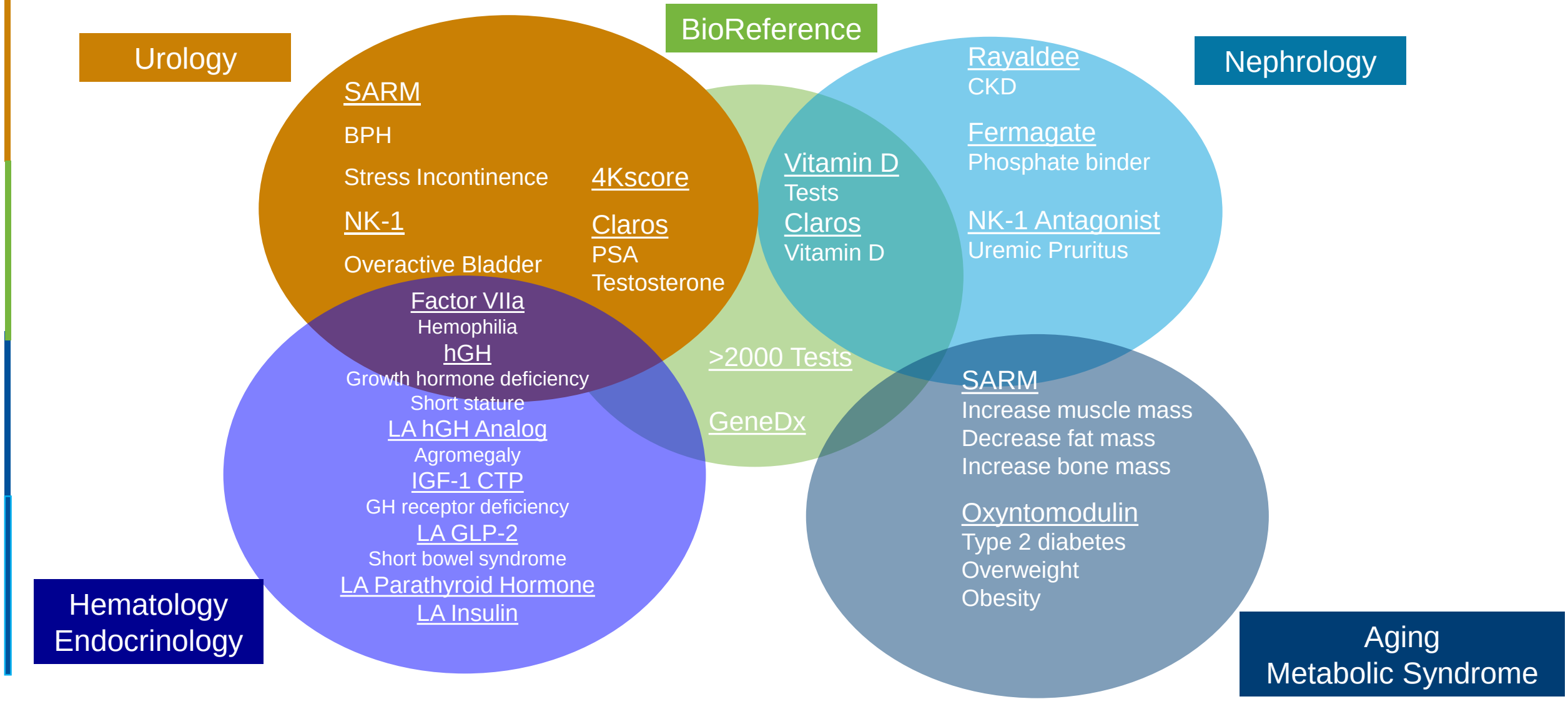
NASDAQ: OPK

FORWARD–LOOKING STATEMENTS

This presentation contains “forward-looking statements,” as that term is defined under the Private Securities Litigation Reform Act of 1995 (PSLRA), which statements may be identified by words such as “expects,” “plans,” “projects,” “will,” “may,” “anticipates,” “believes,” “should,” “intends,” “estimates,” “potential,” and other words of similar meaning, including statements regarding our estimated revenues and financial projections, expected milestones and royalties from the outlicense of our products, our ability to achieve high levels of growth, the potential for our products under development, the potential of the 4Kscore® to influence 89% of biopsy decisions and predict the risk of aggressive prostate cancer, the expected timing of the clinical studies and regulatory approval for our products under development, the outcome of our clinical trials and validation studies and that such outcomes will support marketing approval or commercialization, the expected market penetration and size of the market for our products, including without limitation, Rolapitant, Rayaldee®, hGH-CTP, the 4Kscore, Factor VIIa-CTP, Alpharen, oxyntomodulin, the SARM candidate, our point-of-care diagnostic products and our animal health products, the potential benefits of our products under development, including whether the 4Kscore will predict the risk of 20 year metastasis free survival and result in 40-55% cost savings, the expected submission dates for the PMA for PSA and 510k for testosterone and expected launch date for each, that oxyntomodulin will provide superior long-term therapy for obesity and Type II diabetes patients, our ability to successfully commercialize our product candidates such as Rolapitant, the 4Kscore, hGH-CTP, Rayaldee, Alpharen, the SARM, and oxyntomodulin, and whether Rayaldee will take significant market share in stage 3 and 4 CKD patients with SHPT, whether Rayaldee will raise serum total 25-hydroxyvitamin D (25D) more effectively than any over-the-counter (OTC) or prescription (Rx) products currently marketed without the risk of hypercalcemia, our ability to obtain commercial and Part D coverage for 70% of U.S. covered lives by end of 2017, our ability to develop Rayaldee for new indications including stage 5 CKD and the timeline for doing so, whether data from adult clinical studies of hGH-CTP will support submission or approval of a biologics License Application, whether we will be required to make any changes to our development plans for hGH-CTP, expectations regarding patent coverage, the expected timing for commencing, completing and announcing results for our clinical trials, the timing for release of trial data and seeking and obtaining FDA and European regulatory approvals as well as reimbursement coverage for our products, expectations regarding growth in sales of our 4Kscore and Rayaldee, our ability to obtain a positive coverage determination for the 4Kscore and whether we have enough scientific and clinical data to justify a positive coverage determination, expectations about our animal health business, and the timing of commercial launch of our product candidates. These forward-looking statements are only predictions and reflect our views as of the date they were made, and we undertake no obligation to update such statements. Such statements are subject to many risks and uncertainties that could cause our activities or actual results to differ materially from the activities and results anticipated in forward looking statements, including integration challenges with Bio-Reference and other acquired businesses, risks inherent in funding, developing and obtaining regulatory approvals of new, commercially viable and competitive products and treatments, the success of our collaboration with Pfizer, general market factors, competitive product development, product availability, federal and state regulations and legislation, delays associated with development of novel technologies, unexpected difficulties and delays in validating and testing product candidates, the regulatory process for new products and indications, manufacturing issues that may arise, the cost of funding lengthy research programs, the need for and availability of additional capital, the possibility of infringing a third party’s patents or other intellectual property rights, the uncertainty of obtaining patents covering our products and processes and in successfully enforcing them against third parties, and the potential for litigation or government investigations, among other factors, including all of the risks identified under the heading Risk Factors in our Annual Report on Form 10-K and other filings with the Securities and Exchange Commission.

A multinational biopharmaceutical and diagnostics company establishing important positions in large markets by leveraging its extensive health care industry expertise and experience.

MULTI-FACETED GROWTH STRATEGY



DIVERSIFIED INVESTMENT HIGHLIGHTS

Diagnostics

- Bio-Reference Laboratories revenue of more than $1Billion in 2016
- 400-person sales and marketing team drives industry-leading esoteric testing, ~70% of revenues
- Facilitates uptake of 4Kscore® prostate cancer test and Claros® 1 in office platform
- Completed clinical study on Claros® 1 for PSA – Preparing PMA submission to FDA

Pharmaceuticals

- Rayaldee addresses unmet need in ~$12 billion CKD market, ~9 million patients
- Rayaldee license (VFMCRP); Up to $837 million in milestones, double digit royalties
- Phase 2 for higher dosage Rayaldee in Stage 5 CKD patients initiating 4Q17
- VARUBI™ partnered with Tesaro; Up to $85 million in milestones, double digit royalties, ~$1 billion market
- hGH-CTP is a 1x/week hGH, Partnered with Pfizer, $570 million pre-commercial milestones; double digit royalties and profit-sharing, ~$3 billion growing market
  - Pediatric Phase 3 clinical trial underway and completed adult Phase 3 study in hGH deficiency
- Initiating a multitude of Phase 2 clinical studies in various areas of unmet need in:
  - Hemophilia
  - Benign Prostatic Hypertrophy
  - Obesity/Diabetes
  - Pruritus
  - Dravet Syndrome

Strategy & Execution

- Management team with a track record of success and access to capital
- Commitment to opportunistic business development
- Production and distribution assets expanding worldwide, multiple strategic investments

OPKO DIAGNOSTICS: NEAR–TERM OPPORTUNITIES

LEVERAGING NATIONAL MARKETING, SALES AND DISTRIBUTION RESOURCES TO DRIVE RAPID AND WIDESPREAD UPTAKE OF OPKO DX PLATFORMS

- BioReference Labs is the third largest full service reference laboratory in the U.S.
  - ~400 person sales and marketing team
  - ~5,000+ people working together to support the needs of clients and patients
  - ~200+ patient service centers located throughout the U.S.
- Over 12 million patients served during 2016
- Continued investment in new systems provides better financial data and more information about customers, products and sales
- New leadership team introducing new programs that are expected to benefit all aspects of the business
- Revenue of more than $1Billion in 2016; 2Q17 revenue of $256.7 million
- GeneDx is a genomics leader known for its expertise in rare disease and whole exome testing
  - Continues to further develop it relationship with health care providers and systems
  - Continues to actively expand its innovative tests and service offerings

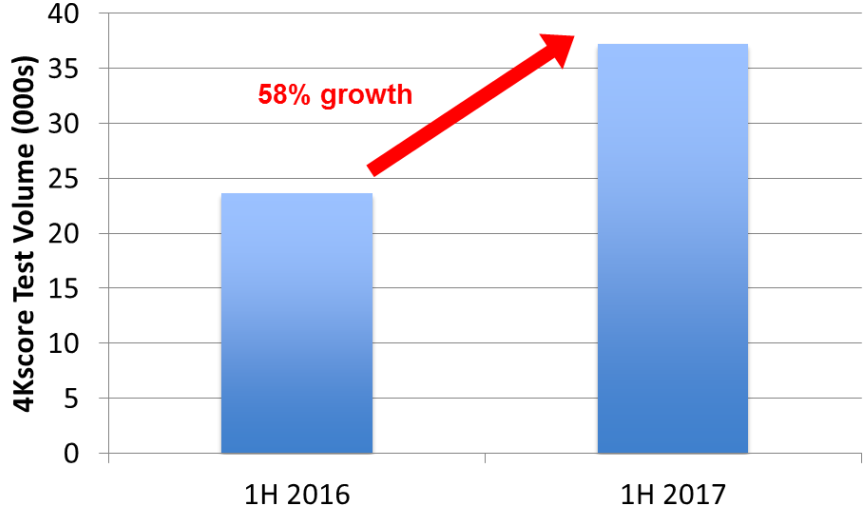
4KSCORE TEST: BLOOD TEST ALTERNATIVE TO BIOPSY

MORE THAN 2 MILLION PROSTATE BIOPSIES PER YEAR WORLDWIDE

- ***4Kscore is the only blood test that accurately identifies risk for aggressive prostate cancer***
- Clinical utility based on decades of biomarker research and >20,000 men tested in Europe and U.S.
- In long-term outcome data 4Kscore test predicts 20 year metastasis free survival for individual patient
- Included in the 2015-2017 NCCN and 2016-2017 EAU Prostate Cancer Guidelines
- Category I CPT published and effective January 1, 2017
- >5,000 physicians have used the 4Kscore in practice; > 18,700 tests performed during 2Q17
- Health economics study shows 40–55% cost savings by avoiding unnecessary MRI, prostate biopsy, and additional treatment or monitoring of indolent cancer
    - 80% of men undergoing prostate biopsy based on PSA are found to have no cancer or indolent cancer
- Clinical utility study shows 4Kscore influences 89% of decisions about performing prostate biopsy

CLINICAL AND COMMERCIAL UPDATE

- Significant YOY growth
- Novitas Solutions (Medicare Administrative Contractor for OPKO Elmwood Park, NJ facility)
  - CMS national rate for 2017 $602.10
  - ***Novitas has been and continues to pay for 4Kscore Medicare submissions***
- Strong showing at the recent AUA meeting
- Data from study at VA hospitals confirming the 4Kscore's ability to accurately predict aggressive prostate cancer presented
  - Demonstrated equally effective and vital clinical test for African American men, who have the highest rates of prostate cancer mortality
  - Preparing manuscript for publication in major urology journal
- Deploying specialized sales force for Urology
- Initiating new sales incentive plan in U.S.
- Initiating TV advertisement in Northeast



CLAROS 1 PLATFORM ADDRESSES LARGE POINT OF CARE TEST MARKET

25M PSA TESTS IN THE US ANNUALLY; $625M MARKET OPPORTUNITY

- Claros 1 can run immunoassay tests in the physician's office or hospital nurse's station using a single draw of blood from a finger stick
- Negates the need for a full blood draw or a centralized reference lab for many common tests
- Completed clinical study for PSA test in August 2017
- Filing modular PMA with FDA for PSA test expected in 4Q17 and expect testosterone 510(k) filing in 2018
- Claros 1 point of care platform will leverage BioReference Labs distribution and marketing
- Menu expansion following initial FDA filings

ROBUST & LATE-STAGE DRUG PIPELINE

PRODUCT	INDICATION	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	MARKETED	MILESTONE
VARUBI (Rolapitant)	CINV	Out-licensed to Tesaro					IV Launch 4Q 2017 EU Oral Launch 2H 2017
Rayaldee® (CTAP101)	SHPT (CKD stage 3-4)	Partnered with Vifor Fresenius Ex U.S.					68% of U.S. potential lives have access; anticipate 70% by year end
Rayaldee® (CTAP101)	SHPT (CKD stage 5)	Partnered with Vifor Fresenius					Ph 2 in 4Q 2017
hGH-CTP (Somatrogon)	hGH deficiency	Collaboration with Pfizer					Adult Ph 3 completed YE16 Pediatric Ph 3 Initiated YE16
Alpharen™ (Fermagate)	Hyperphosphatemia (CKD stage 5 patients)						Ph 3 in 1H 2018
OPK88003 (Oxyntomodulin)	Diabetes, obesity						Ph 2 in 1H 2018
OPK88004 (SARM)	BPH						Ph 2 in 4Q 2017
OPK88002 (NK1)	Pruritus (itching)						Ph 2a 4Q 2017
OPK88005 (Factor VIIa-CTP)	Hemophilia A & B						Ph 2a ongoing
OPK88001 (AntagoNAT)	Dravet Syndrome						Ph 2a in 4Q 2017

CHRONIC KIDNEY DISEASE – THE SILENT KILLER

- CKD is the 9th leading cause of death, ahead of breast and prostate cancer
- CKD prevalence is expected to increase due to obesity, diabetes and hypertension
- Most CKD patients die from cardiovascular disease (CVD), precipitated by secondary hyperparathyroidism (SHPT)
- SHPT is driven by vitamin D insufficiency (VDI) and characterized by elevated blood levels of parathyroid hormone (PTH)
- High PTH levels promote calcification (hardening) of vascular and renal tissues, the major cause of CKD mortality
- Nutritional vitamin D is ineffective for treating VDI and SHPT in CKD, but is the current “standard of care”
- Vitamin D receptor activators (VDRAs) are approved for SHPT in CKD but drive vascular calcification.
- The new KDIGO Clinical Practice Guidelines recommend against routine use of VDRAs in CKD and highlight the unproven effectiveness of vitamin D supplementation
- **Healthcare providers have no good options to treat SHPT in stage 3-4 CKD except for RAYALDEE**

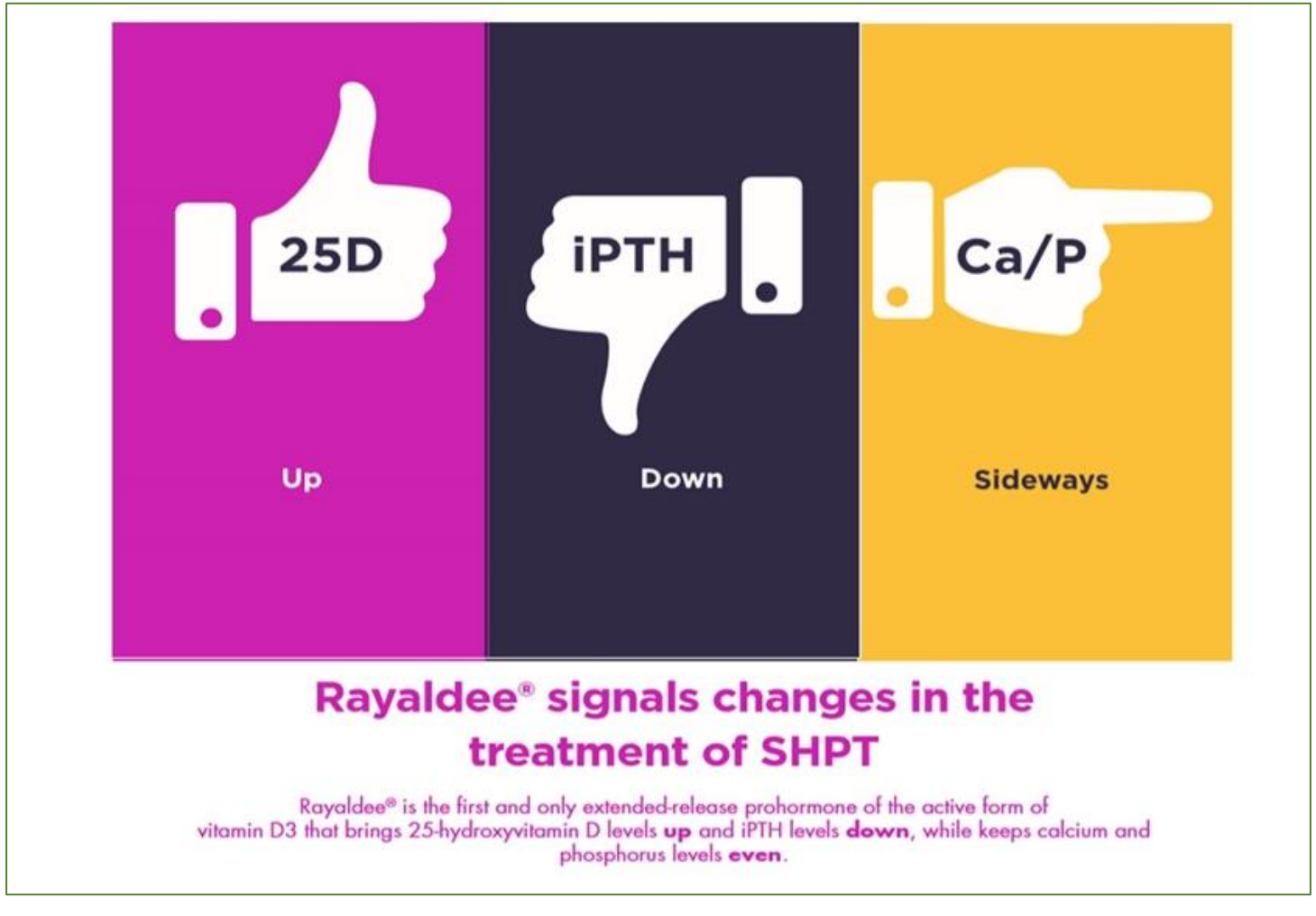
RAYALDEE OVERVIEW

PRODUCT LAUNCHED NOVEMBER 29, 2016

- Extended-Release (1x daily) oral formulation of $25D_3$* addresses significant unmet need
- FDA-approved for SHPT (elevated PTH) in patients with stage 3-4 CKD and VDI
- Reduces plasma PTH and increases serum 25D with a safety profile similar to placebo
- Minimal effects on serum calcium or phosphorus (key drivers of vascular calcification)
- Expected to take significant market share in stage 3-4 CKD patients with SHPT & VDI (~12M patients in US)
- Potential for new indications including stage 5 CKD, institutionalized elderly, osteoporosis and cancer

* 25-Hydroxyvitamin D_3 or Calcifediol

RAYALDEE SENDS A CLEAR MESSAGE



RAYALDEE COMMERCIALIZATION

- Total prescriptions in Q2 increased by 140% compared to 1Q 2017
- Number of nephrologists prescribing Rayladee in Q2 has almost doubled compared to Q1
- 50-person sales and marketing team launched Rayaldee on November 30, 2016
  - Increasing field sales force from 35 to 70 reps – roll out expected October 2017
- Comprehensive ongoing market education campaign highlighting the unmet need re: SHPT
- Leveraging KOL advocates in community outreach (i.e., Speaker Bureaus and Patient Advocacy)
- Commercial and Part D insurance under contract for >68% of U.S. covered lives
  - Growing to more than 70% by end of 2017
- Initial line extension plans
  - Clinical trials for stage 5 CKD to begin 4Q17

SARM–SELECTIVE ANDROGEN RECEPTOR MODULATOR
OPK–88004 –BENIGN PROSTATIC HYPERTROPHY (BPH)

ONCE DAILY ORAL TABLET

- Phase 2 study of 350 male subjects for another indication showed significantly increased lean body mass and muscle strength and significant fat mass reduction with no change or lower prostate specific antigen (PSA) levels
- Animal studies resulted in decreased size of prostate
- Currently in Phase 2 study in prostate cancer patients who have undergone radical prostatectomy
- BPH affects approximately 50 million men in the U.S.

NEXT STEP:

- Begin Phase 2 trial in 4Q2017 to determine optimal dose to treat BPH
- Study will be a 4 month treatment period involving 80 to 120 BPH patients
- Will also examine for improvement in secondary endpoints:
  - PSA
  - Lean body mass
  - Fat mass
  - Physical function

hGH–CTP COMPETITIVE ADVANTAGES

PARTNERED WITH PFIZER

- New molecular entity (NME) that maintains natural native sequence of growth hormone
- Once weekly injection vs. current products requiring daily injections
- Human growth hormone is used for:
  - Growth hormone deficient children and adults
  - SGA, PWS, ISS
- Final presentation:
  - Refrigerated, liquid, non viscous formulation
  - Disposable easy to handle pen injection device with thin needle and small injection volume
- Phase 3 study in growth hormone deficient adults completed at the end of 2016
- Phase 3 study in naive growth hormone deficiency pediatric population underway
- Orphan drug designation in the U.S. and the EU for children and adults

hGH–CTP PROGRAM STATUS

- **Initiated Phase 3 pediatric hGH-CTP study in December 2016**
  - 220 patients, non-inferiority comparison of weekly hGH-CTP to daily growth hormone
  - Global study CROs selected; sites initiated in December
  - Easy-to-use, disposable, refrigerated pen device
- **Phase 3 adult hGH-CTP**
  - In December 2016 reported that primary endpoint of change in trunk fat mass from baseline to 26 weeks did not demonstrate a statistical significance between the hGH-CTP treated group and placebo
  - Completed post hoc outlier analysis in June 2017 to assess the influence of outliers on the primary endpoint results
  - Analyses which excluded outliers showed a statistically significant difference between hGH-CTP and placebo on the change in trunk fat mass; additional analyses that did not exclude outliers showed mixed results
  - No safety concerns
  - OPKO and Pfizer have agreed that OPKO may proceed with a pre-BLA meeting with FDA to discuss a submission plan
  - OPKO plans to carry out an additional study in adults using a pen device
- **Initiated pediatric hGH-CTP registration study in Japan**
  - 44 patients, comparison of weekly hGH-CTP to daily growth hormone
  - Same pen device, dosage and formulation used in global study

LONG-ACTING FACTOR VIIA-CTP FOR HEMOPHILIA A & B

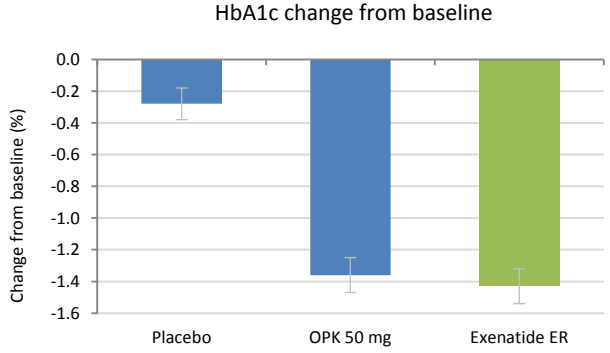
$1.7 billion market growing 7% annually and only 25% of patients are treated

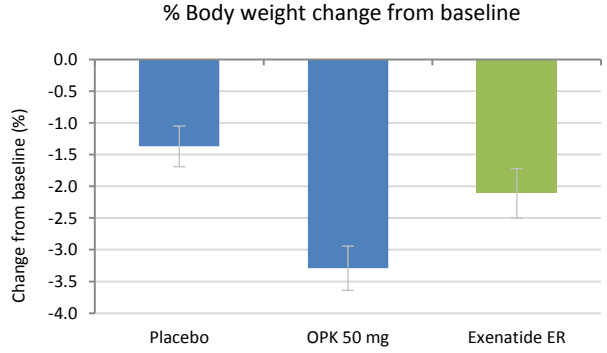
- Current product (NovoSeven®) requires frequent IV doses
  - 3-4 times a day during bleeding episodes
  - 1-2 times a day for prophylactic treatment
- In pharmacological studies in hemophilic mice and dogs, Factor VIIa-CTP:
  - Demonstrated potential for subcutaneous administration
  - Reduced frequency of injection during on-demand therapy
  - Enabled prophylactic treatment while reducing the injection frequency to 2-3 times a week
- Commenced a Phase 2a single intravenous injection (IV) dose escalating study in January 2016 to evaluate the safety, PK and PD properties of Factor VIIa
- Commenced a Phase 1 single dose subcutaneous (SC) administered, dose escalating study in December 2016 to evaluate the safety, PK and PD properties of Factor VIIa when administered by SC injection
- Top line data from both studies as well as preclinical data presented at the International Society on Thrombosis and Haemostasis in June
  - Factor VIIA-CTP in both IV and SC administration demonstrated a favorable safety profile and local tolerance following single administration.
  - No unexpected adverse events related to the drug
- Orphan drug designation in the U.S. and the EU

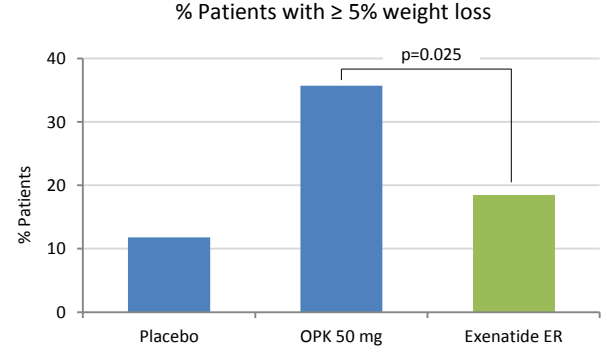
OPK–88003 OXYNTOMODULIN ANALOG

FOR THE TREATMENT OF TYPE 2 DIABETES AND OBESITY

- 26 million diabetics in US: drug development focused on: **blood glucose control** and **reducing body weight**
- OPK-88003 is an once-weekly analog with both GLP-1 and glucagon activity
- Data support that combining GLP-1 and glucagon activity provides superior weight loss
- Data from phase 2 study in 420 type 2 diabetes patients (week 12):







OPK-88003 CLINICAL DEVELOPMENT

- Phase 2b dose-escalation study expected to start 1H18
- Preparation of pen and formulation for phase 3 is ongoing

OPK-88002 NK 1 ANTAGONIST

FOR THE TREATMENT OF PRURITUS IN DIALYSIS PATIENTS

PRURITUS

- Acute and chronic pruritus (“itching”) occurs in 10% to 15% of the population
  - Most prevalent in skin, kidney and liver diseases
- Substance P is implicated in pruritus
- NK-1 antagonists block substance P activity and have been shown to reduce itching in human trials

PRURITUS IN DIALYSIS PATIENTS

- Major medical need and requires management
- 70 to 90% of patients Kidney dialysis patients suffer from Pruritus

PHASE 2a CLNICAL STUDY

- Expected to begin in 4Q2017

SELECT FINANCIAL INFORMATION

Balance sheet at 6/30/2017

- Cash, cash equivalents & marketable securities: $130.5 million
- Net investments: $34.5 million
- Current portion of line of credit and notes payable: $14.5 million
- Senior notes (net of embedded derivatives): $34.8 million

Capital structure at 6/30/2107

- Common shares outstanding: 559,995,118

Revenues

- Three months ended June 30, 2017 were $314.2 million compared to $357.1 million for the comparable period of 2016.
  - 2016 period includes a $50 million upfront payment for Rayaldee from Vifor Fresenius

UPCOMING MILESTONES

PROGRESS ACROSS MULTIPLE BUSINESS AREAS

▪ Expand Rayaldee field sales force	October 2017
▪ SARM Phase 2	Initiate in 4Q17
▪ Claros 1 PSA clinical study	PMA filing 4Q17
▪ Claros 1 testosterone clinical study	Initiate 1H18
▪ Oxyntomodulin Phase 2b	Initiate 1H18
✓ hGH-CTP Phase 3 Pediatric	Enrollment ongoing
▪ Rayaldee Stage 5 CKD Phase 2	Initiate 4Q17
▪ AntagoNAT Dravet Phase 2b	Initiate 4Q17
▪ NK-1 Antagonist Pruritus Phase 2a	Initiate 4Q17
✓ Pediatric hgh-CTP registration study in Japan	Initiated 3Q17

THANK YOU