Diagnostics & Pharmaceuticals for Large Markets with Unmet Needs

May 2017

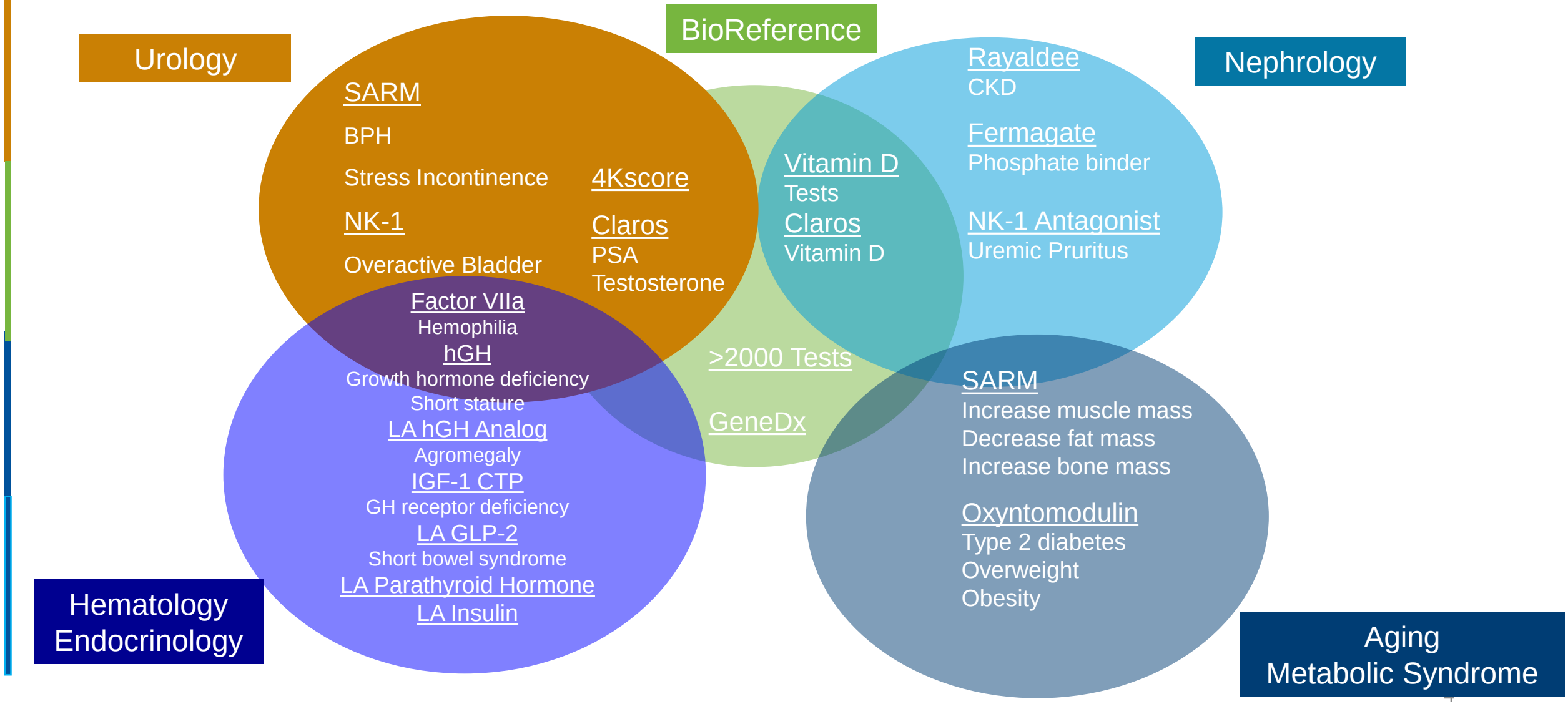
NASDAQ: OPK

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 75% 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, expectations surrounding the sensitivity analysis for primary and secondary endpoints for the adult hGH-CTP study, whether the results of the analysis will be positive, whether the FDA would consider the analysis and whether the drug will be approvable, 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, 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 recommendations in the KDIGO guidelines, expectations about our animal health business and the introduction of several prescription products this year for the animal market, 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
- Initiated clinical study on Claros® 1 for PSA

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 2H17
- VARUBI™ partnered with Tesaro; Up to $95 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
- OPK88003 SARM initiating a Phase 2 study to treat benign prostate hypertrophy in 2H17
- OPK88005 Long acting Factor VIIa-CTP in an ongoing Phase 2a study to treat hemophilia, ~$1.7B market
- OPK88004 Oxyntomodulin initiating a Phase 2 study as a treatment for diabetes/weight loss in 1H18
- OPK88002 NK-1 Antagonist initiating a Phase 2a study to treat pruritis (itching) in 2H17
- OPK88001 AntagoNat initiating a Phase 2a study as a treatment for Dravet Syndrome in 2H17

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
- Revenue of more than $1Billion in 2016; 1Q17 revenue of $255.3 million
- GeneDx is a genomics leader known for its expertise in rare disease and whole exome testing
- Utilizing BRL commercial infrastructure to drive 4Kscore and Claros 1 adoption

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 and 2016 NCCN and 2016 EAU Prostate Cancer Guidelines
- Category I CPT published and effective January 1, 2017
- >5,000 physicians have used the 4Kscore in practice; > 18,600 tests performed during 1Q17
- 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

4KSCORE TEST: REIMBURSEMENT UPDATE

POSITIVE PROGRESS WITH COMMERICIAL PAYORS; MEDICARE LOCAL COVERAGE DECISIONS EXPECTED

- Obtained positive coverage decision from one national payer
- Obtained pricing agreements from several regional payers
- CMS national rate for 2017 $602.10
- Novitas Solutions (Medicare Administrative Contractor for OPKO Elmwood Park, NJ facility)
  - Initial draft positive coverage determination (LCD) tentatively retired due to a potential conflict with another MAC
  - ***Novitas has been and continues to pay for 4Kscore Medicare submissions***
  - Expect Novitas to include 4Kscore in an upcoming review cycle for a draft local coverage determination.
- Palmetto, GBA Noridian and GSS Administrators
  - Issued negative coverage determinations
  - OPKO addressing concerns largely around clinical study protocols and clinical utility
- OPKO confident has now supplied sufficient scientific and clinical data to justify positive LCD by any MAC

CLAROS 1 PLATFORM ADDRESSES LARGE POINT OF CARE TEST MARKET

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

- Initiated clinical study for PSA test in January 2017
- Filing modular PMA with FDA for PSA test expected in 2H17 and expect testosterone 510(k) filing 2H17
- 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 1H 2017 EU Oral Launch 1H 2017
Rayaldee® (CTAP101)	SHPT (CKD stage 3-4)	Partnered with Vifor Fresenius Ex U.S.					60% of U.S. potential lives have access; anticipate 75% by year end
Rayaldee® (CTAP101)	SHPT (CKD stage 5)	Partnered with Vifor Fresenius					Ph 2 in 2H 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 2H 2017
OPK88004 (Oxyntomodulin)	Diabetes, obesity						Ph 2 in 1H 2018
OPK88003 (SARM)	BPH						Ph 2 in 2H 2017
OPK88002 (NK1)	Pruritis (itching)						Ph 2a in 2H 2017
OPK88005 Factor VIIa-CTP	Hemophilia A & B						Ph 2a ongoing
OPK88001 (AntagoNAT)	Dravet Syndrome						Ph 2a in 2H 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 forthcoming KDIGO Clinical Practice Guidelines are expected to recommend against routine use of VDRAs in CKD and to 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**

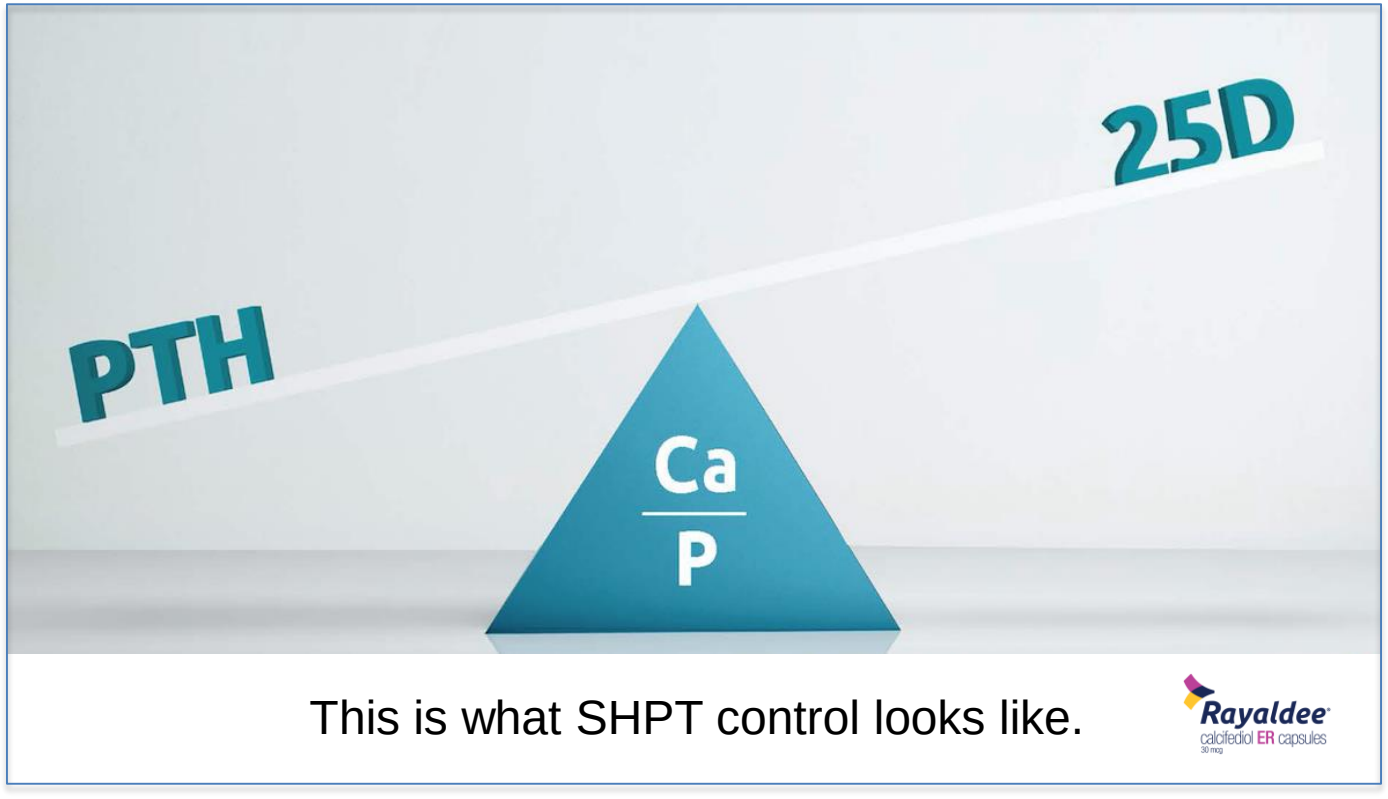
RAYALDEE OVERVIEW

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: SIMPLE POSITIONING



RAYALDEE COMMERCIALIZATION

- 50-person sales and marketing team launched Rayaldee on November 30, 2016
  - Plans to increase the sales and marketing team in 2H17
- 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 >60% of U.S. covered lives
  - Growing to more than 70-80% end of 2017
- Initial line extension plans
  - Clinical trials for stage 5 CKD to begin 2H17

SARM–SELECTIVE ANDROGEN RECEPTOR MODULATOR
TT701 –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

- **NEXT STEP:**

Begin Phase 2 trial to determine optimal dose to treat BPH

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

ADULT hGH–CTP PHASE 3 STUDY TOPLINE DATA

- Efficacy endpoints of treating **adult** GHD patients is body fat mass, which includes trunk fat mass reduction; Differs from treating pediatric GHD patients which assesses growth height velocity
- A global multicenter study dosed in 198 patients
- Topline data means first look of unblinded data
- Primary endpoint is trunk fat mass reduction from baseline after 6 months of treatment
- Topline showed:
  - hGH-CTP group has mean change in trunk fat mass of -0.4kg and placebo group is 0
  - Does not meet statistical significance of ≤0.05 (p value)
  - 97% of hGH-CTP vs 6% of placebo group showed IFG-1 normalization
  - Safety profile is consistent with that observed with those treated with daily growth hormone
- Found an exceptional value of trunk fat mass reduction in the placebo group

NEXT STEP

Phase 3 adult hGH-CTP study

- The exceptional data point warrants an outlier sensitivity analysis of the primary endpoint and related secondary endpoints
- Developed a statistical plan for data sensitivity analysis to identify any outlier from the entire data set
- Proceed with analysis; discuss with regulatory authorities

Communicated to all investigators and CROs involved in all on-going and newly initiated adult and pediatric hGH-CTP studies

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 and Phase 2 pediatric hGH-CTP open label extension studies continue without interruption**
  - No safety concerns
  - Switching to pen device in open label extensions
- **Initiating 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
- **Orphan drug designation in the U.S. and the EU**

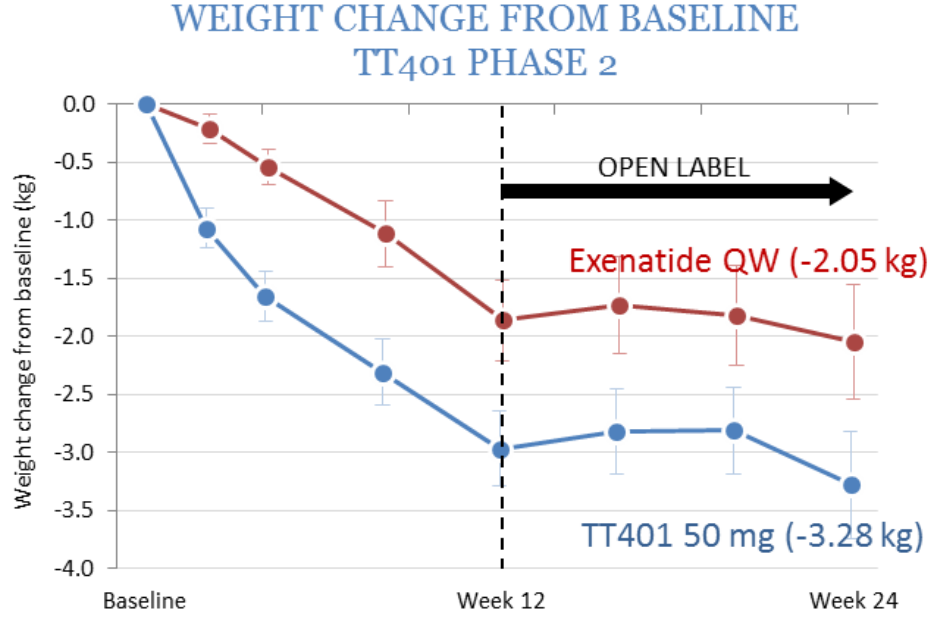
TT401 – OXYNTOMODULIN – TYPE 2 DIABETES/OBESITY

POTENTIAL FIRST TO MARKET GLP-1/GLUCAGON DUAL AGONIST

- Once weekly administered oxyntomodulin for type 2 diabetes and obesity
- Completed 420 patient Phase 2 study in type 2 diabetes patients
- Superior weight loss compared with currently approved extended release Exenatide and placebo after 12 and 24 weeks of treatment.
- Reduction in HbA1c, marker of sugar metabolism, similar to Exenatide at weeks 12 and 24.

- **NEXT STEP:**

Dose optimization Phase 2 trial



OPKO ANIMAL HEALTH

UNIQUELY POSITIONED TO OFFER PRODUCTS FOR COMPANION ANIMALS; $60 BILLION U.S.MARKET

- Minimal investment by utilizing existing product and manufacturing resources
- Launched several OTC products 1Q17
- Plan to launch select prescription products in 4Q17
- Oncology products being developed with OPKO Ireland for treatment of common cancers in pets
  - >6 million dogs and >6 million cats in the U.S. are diagnosed with cancer annually
- Developing pet friendly formulations and packaging with specific labeling for different species
- Commenced pilot study for a pet friendly Rayaldee product
  - > 1.5 million cats and dogs suffer from elevated PTH and low Vitamin D
- Marketed through a logistics partner and select national and regional distributors
- Executive team has significant experience and success in developing and marketing animal health products (DVM Pharmaceuticals division of IVAX Corp.)

SELECT FINANCIAL INFORMATION

Balance sheet at 3/31/2017

- Cash, cash equivalents & marketable securities: $131.1 million
- Net investments: $37.3 million
- Current portion of line of credit and notes payable: $11.5 million
- Senior notes (net of embedded derivatives): $39.3 million

Capital structure at 3/31/2017

- Common shares outstanding: 559,597,843

Revenue 2017 vs. 2016

- Three months ended March 31, 2017: $296.1 million vs. $291.0 million

UPCOMING MILESTONES

PROGRESS ACROSS MULTIPLE BUSINESS AREAS

▪ 4Kscore reimbursement	Coverage decisions and pricing negotiations underway
▪ $10M milestone for Varubi EU launch	Expected in 3Q17
✓ OTC animal health products	Initial launch 1Q17
▪ SARM Phase 2	Initiate in 2H17
▪ Claros 1 PSA clinical study	PMA filing 2H17
▪ Claros 1 testosterone clinical study	Initiate 2H17
▪ Oxyntomodulin Phase 2b	Initiate 1H18
✓ hGH-CTP Phase 3 Pediatric	Initiated YE16; enrollment ongoing
▪ Rayaldee Stage 5 CKD Phase 2	Initiate 2H17
▪ AntagoNAT Dravet Phase 2b	Initiate 2H17
▪ NK-1 Antagonist Pruritis Phase 2a	Initiate 2H17
▪ Pediatric hgh-CTP registration study in Japan	Initiate 2H17/1H18

THANK YOU