OPKO

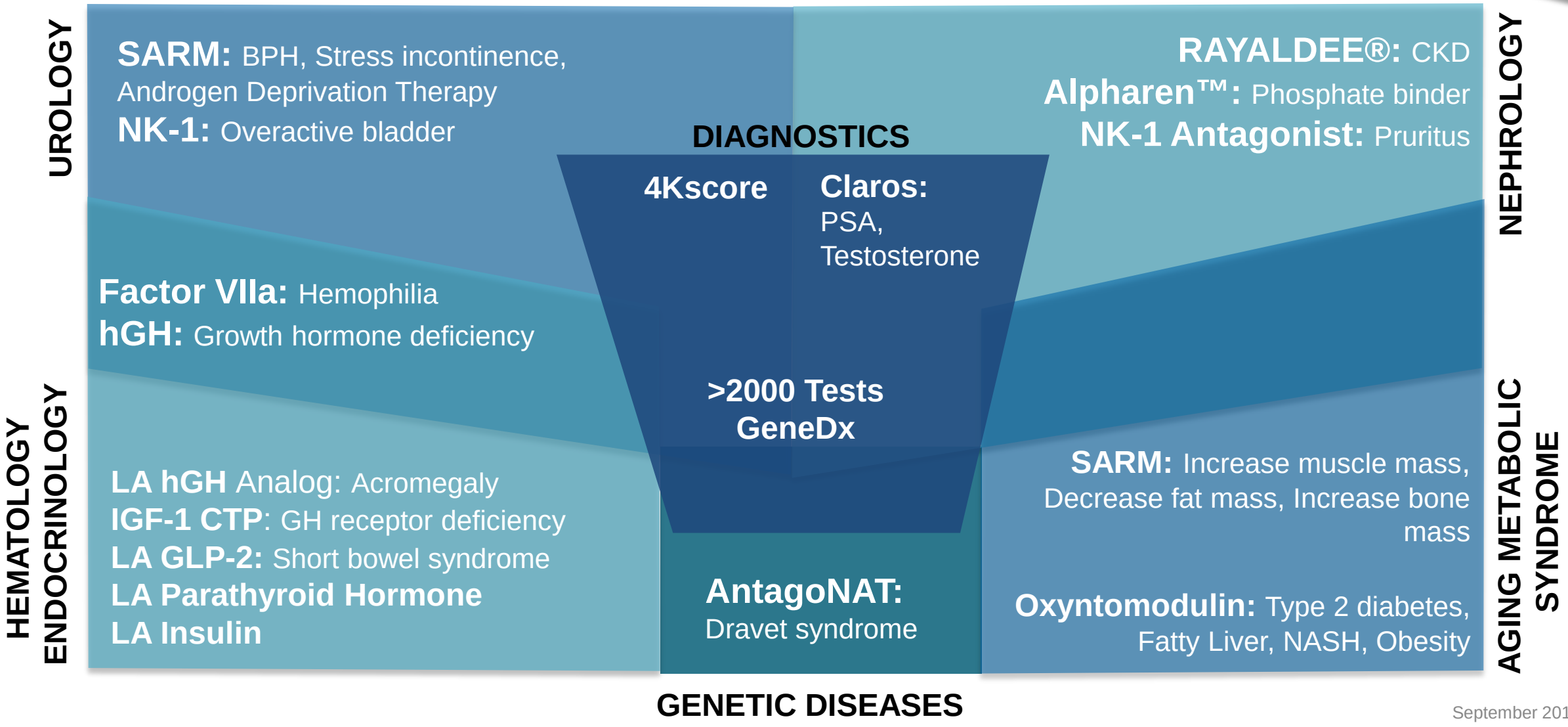
September 2018

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, including expected Rayaldee revenues for the third quarter, whether prescriptions for Rayaldee will continue to increase, 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 and whether we can develop them for certain indications, potential development opportunities for oxyntomodulin and the SARM candidate, the ability 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, Rayaldee®, hGH-CTP, the 4Kscore, Alpharen, oxyntomodulin, the SARM candidate, and our point-of-care diagnostic products, whether we can expand the menu of our point of care test offerings, 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 response date from the FDA and approval for the PMA for PSA and submission date for 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 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 develop Rayaldee for new indications including stage 5 CKD and the timeline for doing so, whether the clinical data and post hoc sensitivity analysis for hGH-CTP will support submission of a Biologics License Application (BLA) and approval for hGH-CTP in adults, whether we will submit a BLA and the timing for doing so, 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, enrolling, 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, whether Novitas and other payors will reimburse us for the 4Kscore test, 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, liquidity issues and 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.

Multinational biopharmaceutical and diagnostics company establishing important positions in large, underserved markets

Multifaceted Growth Strategy



Investment Highlights

Mature Diagnostic Business

Third largest reference lab in the U.S.

Marketed Pharmaceuticals

in early life cycle with multi $B market opportunities in large underserved markets

Robust Development Pipeline

Multiple late stage clinical trials addressing indications with large unmet medical needs

Strategy & Execution

Management team with a track record of success and commitment to opportunistic development

Diversified Assets Across Business Units

Marketed Pharmaceuticals

- RAYALDEE addresses unmet need in CKD market, ~9 million patients
- RAYALDEE partnered with Vifor Fresenius; Up to $837 million in milestones, double digit royalties
- RAYALDEE partnered in Japan with Japan Tobacco; up to $112 million in milestones with tiered double digit royalties

Robust Pipeline

- Somatrogon (hGH-CTP) partnered with Pfizer, $570 million pre-commercial milestones; double digit royalties and profit sharing,
  - ~$3 billion growing market for hGH
  - Pivotal Phase 3 pediatric somatrogon study completed enrollment August 2018
- Initiating multiple Phase 2 clinical studies in various areas of unmet need

Diagnostics

- BioReference Laboratories revenue of ~$427 million in 1H18
- 300-person sales and marketing team drives industry leading esoteric testing, ~70% of revenues
- Facilitates uptake of 4Kscore® prostate cancer test

Diversified Assets
Early Life Cycle Pharmaceuticals

Marketed Pharmaceuticals

Robust Pipeline

Diagnostics

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Chronic Kidney Disease (CKD) -The Silent Killer

- 9th Leading cause of death, ahead of breast and prostate cancer
- Prevalence expected to increase due to obesity, diabetes and hypertension
- Most patients die from cardiovascular disease, precipitated by secondary hyperparathyroidism (SHPT)
- SHPT is driven by vitamin D receptor activators (VDRAs) 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
- Updated 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

- Extended Release (1x daily) oral formulation of $25D^3$* addresses significant unmet need
- FDA-approved for SHPT (elevated iPTH) in patients with stage 3-4 CKD and VDI
- Reduces plasma iPTH 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 (~9M patients in U.S.)
- Potential for new indications including stage 5 CKD, institutionalized elderly, osteoporosis and bariatric surgery

* 25-Hydroxyvitamin D_3 or Calcifediol

RAYALDEE Sends Clear Message

RAYALDEE signals changes in treatment of SHPT

- First and only extended release prohormone of active form of vitamin D^3
- Brings 25D levels up
- Brings iPTH levels down

RAYALDEE U.S. Commercialization

- 80-Person sales and marketing team
- Increased field sales force from 35 to 64 reps (October 2017)
  - Commercial insurance under contract for >83% of U.S. covered lives
  - Medicare Part D insurance under contract for >53% of U.S. covered lives
- Total RAYALDEE prescriptions increased approximately 467% in 2Q18 compared to 2Q17, and 36% compared to 1Q18
- 2Q18 RAYALDEE revenue of $4.8M
  - Expect revenues of $5.7M-$6.5M for 3Q18
- International partnership with Vifor Fresenius; up to $837 M in milestones, double digit royalties
- Japanese partnership with Japan Tobacco; up to $112 M in milestone payments, tiered double digit royalties
- Initial line extension plans
  - Initiated phase 2 clinical trial for higher dose RAYALDEE to treat stage 5 CKD in Sept 2018

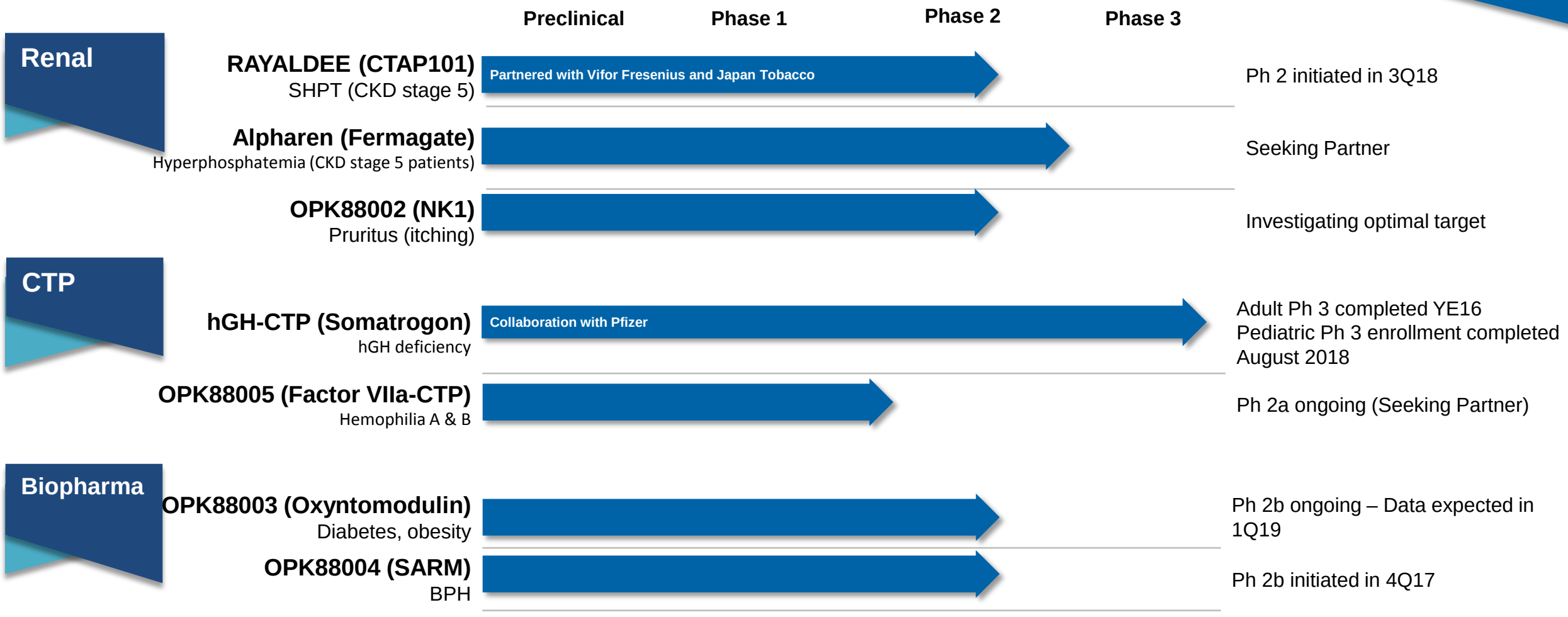
Diversified Assets
Broad Development Pipeline

Marketed Pharmaceuticals

Robust Pipeline

Diagnostics

Robust Late-Stage Pipeline



Somatrogon Competitive Advantages

New molecular entity (NME) that maintains native sequence of growth hormone

- Once weekly injection vs. current products requiring daily injections
- Phase 3 study in growth hormone deficient adults completed at the end of 2016
- Phase 3 study in naive growth hormone deficiency pediatric population enrollment completed August 2018
- Final presentation:
  - Refrigerated, liquid, non viscous formulation
  - Disposable easy-to-handle pen injection device with thin needle and small injection volume
- Orphan drug designation in the U.S. and the EU for children and adults

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Pfizer

Partnered worldwide with Pfizer

$570M Pre-commercial milestones

Double digit royalties

1 in 10K

Adults diagnosed with GHD[1]

1 in 3.8K

Children in U.S. have growth failure due to growth hormone deficiency[2]

[1] National Center for Biotechnology Information: [2] http://www.childgrowthfoundation.org/

hGH-CTP (Somatrogon) Program Status

Phase 3 pediatric somatrogon study enrollment completed August 2018

- 225 patients, non-inferiority comparison of weekly somatrogon to daily growth hormone
- Easy to use, disposable, refrigerated pen device

Phase 3 adult somatrogon

- 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 somatrogon treated group and placebo
- Completed post hoc outlier analysis in June 2017 to eliminate the influence of outliers on the primary endpoint results
- Analyses, which excluded outliers, showed a statistically significant difference between somatrogon and placebo on the change in trunk fat mass; additional analyses that did not exclude outliers showed mixed results
- No safety concerns
- Correspondence and communication with FDA indicated that:
    - There is a path for submission whereby FDA would assess the totality of the data – all relevant efficacy and safety data in adults and pediatric patients
    - The number of patients to be included in the safety database seems sufficient
    - The design of the bioequivalence study for the change from vial to pen formulation is generally acceptable
- Next Steps
    - Assess regulatory strategy for adult indication based on response from FDA

Somatrogon pediatric registration study in Japan underway

- 44 patients, comparison of weekly somatrogon to daily growth hormone
- Same pen device, dosage and formulation used in global Phase 3 pediatric study

Selective Androgen Receptor Modulator (SARM)

OPK-88004 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

Clinical Plan

- Initiated 125-patient Phase 2b trial in benign prostatic hypertrophy (BPH) or enlarged prostate to determine optimal dose
  - Complete enrollment by 1Q19
- Potential for new indications including stress urinary incontinence and androgen deprivation therapy in prostate cancer patients

50 Million

Men in the U.S. are affected by BPH

90%

Men 80 years old and older are affected by enlarged prostate[1]

14 Million

Men in the U.S. with lower urinary tract symptoms suggestive of BPH[1]

[1] Deters LA. Benign prostatic hypertrophy. Emedicine http://emedicine.medscape.com/

NK 1 Antagonist

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

OPK-88002 Clinical Development

- Investigating optimal target

[1] Medscape.com, International Society of Nephrology (ISN)/

Oxyntomodulin Analog
for Treatment of Type 2 Diabetes and Obesity

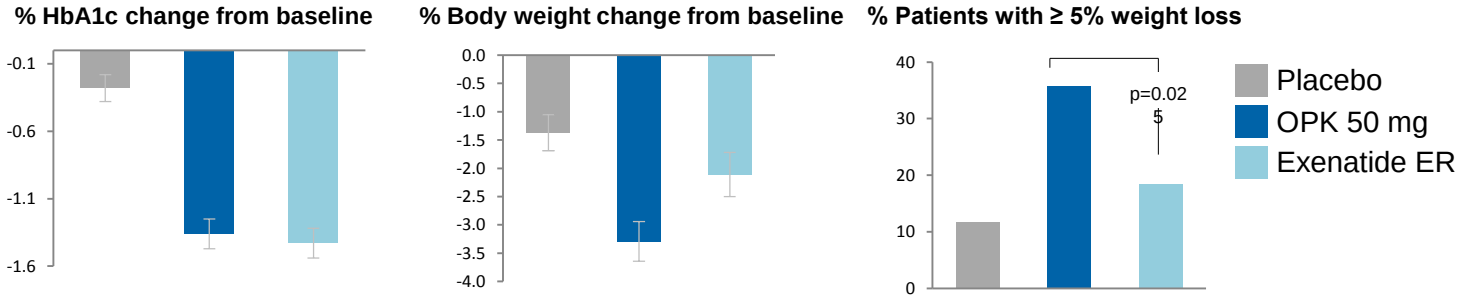
OPK-88003

- Drug development focused on: blood glucose control and reducing body weight
- Once weekly analog with potentially first to market dual GLP-1/glucagon agonist
- Data support that combining GLP-1 and glucagon activity provides superior weight loss

Clinical Development

- Phase 2b dose-escalation study enrollment completed June 2018
- Preparation of pen and formulation for Phase 3 is ongoing
- Potential additional indication for the treatment of non-alcoholic fatty liver disease (NAFLD) and non-alcoholic steatohepatitis (NASH)

Phase 2 study in 420 type 2 diabetes patients (week 12)



[1] http://www.diabetes.org; [2] https://www.cdc.gov/diabetes

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9.3%

Of the American population suffers from diabetes[1]

$176B

Direct medical costs related to diabetes in U.S.[1]

17.7M

Adults diagnosed with diabetes reported taking any medication[2]

Diversified Assets
Third Largest Reference Laboratory in the U.S.

Marketed Pharmaceuticals

Robust Pipeline

Diagnostics

OPKO Diagnostics

Near term Opportunities

Leveraging National Marketing, Sales and Distribution Resources to Drive Uptake of Diagnostic Platforms

- Revenue of ~$427M in 1H18; 2Q18 revenue of ~$216M
- GeneDx is a genomics leader known for its expertise in rare disease and whole exome testing
- Utilizing BRL commercial infrastructure to drive 4Kscore adoption and will use for Claros 1 launch and adoption upon approval

BioReference Labs is third largest full service reference laboratory in the U.S.

- ~300 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.

4Kscore® Test

Blood Test Alternative To Biopsy

More Than 2 Million Prostate Biopsies/Year WW

- 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 can predict 20 year metastasis free survival for individual patient
- Included in the 2015-2017 NCCN and 2016-2017 EAU Prostate Cancer Guidelines
- 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

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4Kscore®

Only blood test that accurately identifies risk for aggressive prostate cancer

>5K

Physicians have used 4Kscore in practice

20.5K

Tests performed during 2Q18, a 10% increase compared with 2Q17

4Kscore Clinical and Commercial Update

- Category I CPT published and effective January 1, 2017; CMS national rate for 2018 increased to $760 vs $600 in 2017
- Novitas Solutions (Medicare Administrative Contractor for OPKO Elmwood Park, NJ facility)
  - Novitas issued a draft non-coverage determination in May 2018 subject to a public comment period ended July 13, 2018
  - Novitas has been and continues to pay for 4Kscore Medicare submissions pending finalization of the draft non-coverage decision.
  - OPKO is taking a multipronged approach to address the concerns raised in the Novitas draft LCD
- 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
- Radical prostatectomy study demonstrated 4Kscore can effectively differentiate biopsy Gleason 6 cancer from those likely to harbor adverse pathology

Claros 1 Platform

Addresses Large Point of Care Test Market

25M PSA Tests in U.S. Annually

- Novel diagnostic system can provide rapid, quantitative blood test results in 10 minutes – right in the physician's office
- Modular PMA with FDA for PSA test filed in 4Q17; expect response YE18 or early 2019
- Expect testosterone 510(k) filing 1Q19
- Claros 1 point of care platform will leverage BioReference Labs distribution and marketing
- Menu expansion following initial FDA filings

Select Financial Information

June 30, 2018

Balance Sheet

- Cash, cash equivalents & marketable securities: $80.4 million
- Net investments: $45.5 million
- Current portion of line of credit and notes payable: $36.8 million
- 2023 5% convertible notes and other long term liabilities: $148.3 million

Capital Structure

- Common shares outstanding: 560.17million

Income Statement

- Consolidated revenues for 2Q18 were $263.7 million compared with $292.6 million for 2Q17
  - Revenue from services were $216.1 million Q218 compared with $233.9 million for Q217
- Net loss for 2Q18 was $6.2 million ($0.01 per share) compared with net loss of $16.9 million ($0.03 per share) for 2Q17

Upcoming Milestones

Progress Across Multiple Business Areas

✓ Phase 3 pediatric somatrogon study	Enrollment Complete Aug 2018
✓ Oxyntomodulin Phase 2b	Enrollment Complete June 2018
✓ RAYALDEE Stage 5 CKD Phase 2	Commenced 3Q18
SARM Phase 2 complete enrollment	1Q19
Claros 1 Testosterone 510(k) submission	1Q19
PMA application for Claros PSA	Under review by FDA

Thank You