
OPKO

# September 2020
# 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 expected financial performance and expectations regarding the market for and sales of our products, expectations about COVID-19 testing, the demand for testing, our capacity for testing and expected turnaround time, the impact of COVID-19 on all of our businesses, positively and negatively, our ability to expand our capacity should there be additional demand, the availability of resources, including labor, equipment and supplies, to meet demand for testing and the potential impact on us should these resources be constrained, whether our turnaround time will be as expected or our performance quality decline, our product development efforts and the expected benefits of our products, our estimated revenues and financial projections, including expected Rayaldee and BioReference revenues, whether we will maintain profitability or continue growth at BioReference, whether prescriptions for Rayaldee will stabilize or increase, expected milestones and royalties from the outlicense of our products, 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, our ability to successfully commercialize our product candidates such as hGH-CTP, Factor VII-CTP, and our rare disease product candidates, 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 for hGH-CTP will support submission of a Biologics License Application (BLA), the timing of such submission, and approval for hGH-CTP in adults and pediatric patients, whether we will be required to make any changes to our development plans for hGH-CTP and increase our expenditures, 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. 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 the continued prevalence of COVID-19, 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 and relationship with Pfizer and our other commercial partners, 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 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, 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

# Diversified Assets Across Business Units

## Marketed Pharmaceuticals

- Rayaldee addresses unmet need in CKD market
- Rayaldee revenue of $8.6 million in 2Q 2020
- Rayaldee partnered with Vifor Fresenius; Up to $787 million in milestones with tiered double digit royalties
- Rayaldee partnered in Japan with Japan Tobacco; up to $112 million in milestones with tiered double digit royalties

## Pipeline

- Somatrogon once weekly (hGH-CTP) partnered with Pfizer
  - Successful pivotal phase 3 pediatric somatrogon study completed; positive topline data announced October 2019
  - Successful Japanese phase 3 pediatric somatrogon study completed; positive top line data announced in June 2020
- Initiating multiple drug study trials in various areas of unmet need

## Diagnostics

- BioReference Laboratories revenue of
- ~$251 million in 2Q 2020
- Significant response to COVID-19 pandemic; ~5.2 million PCR tests performed as of September 13 2020; leader in mobile testing sites and federal, state and local partnerships
- 300-person sales and marketing team drives industry leading esoteric testing
- Recognized leader in genetic testing

# Somatrogon© Summary

## Achieved Primary Endpoint

- Somatrogon© (hGH-CTP), a once weekly growth hormone replacement therapy, was proven non-inferior to daily Genotropin® (somatropin) with respect to height velocity after 12 months
- Height velocity at 12 months of treatment was higher in the somatrogon© group (10.12 cm/year) than in the somatropin group (9.78 cm/year)

## Secondary Endpoints Achieved

- Change in height standard deviation scores at six and 12 months were higher with somatrogon© in comparison to somatropin
- At six months, change in height velocity was higher with somatrogon© in comparison to somatropin
- Somatrogon was generally well tolerated in the study and comparable to that of somatropin dosed once-daily with respect to the types, numbers and severity of the adverse events observed between the treatment arms.

## Global Partnership with Pfizer

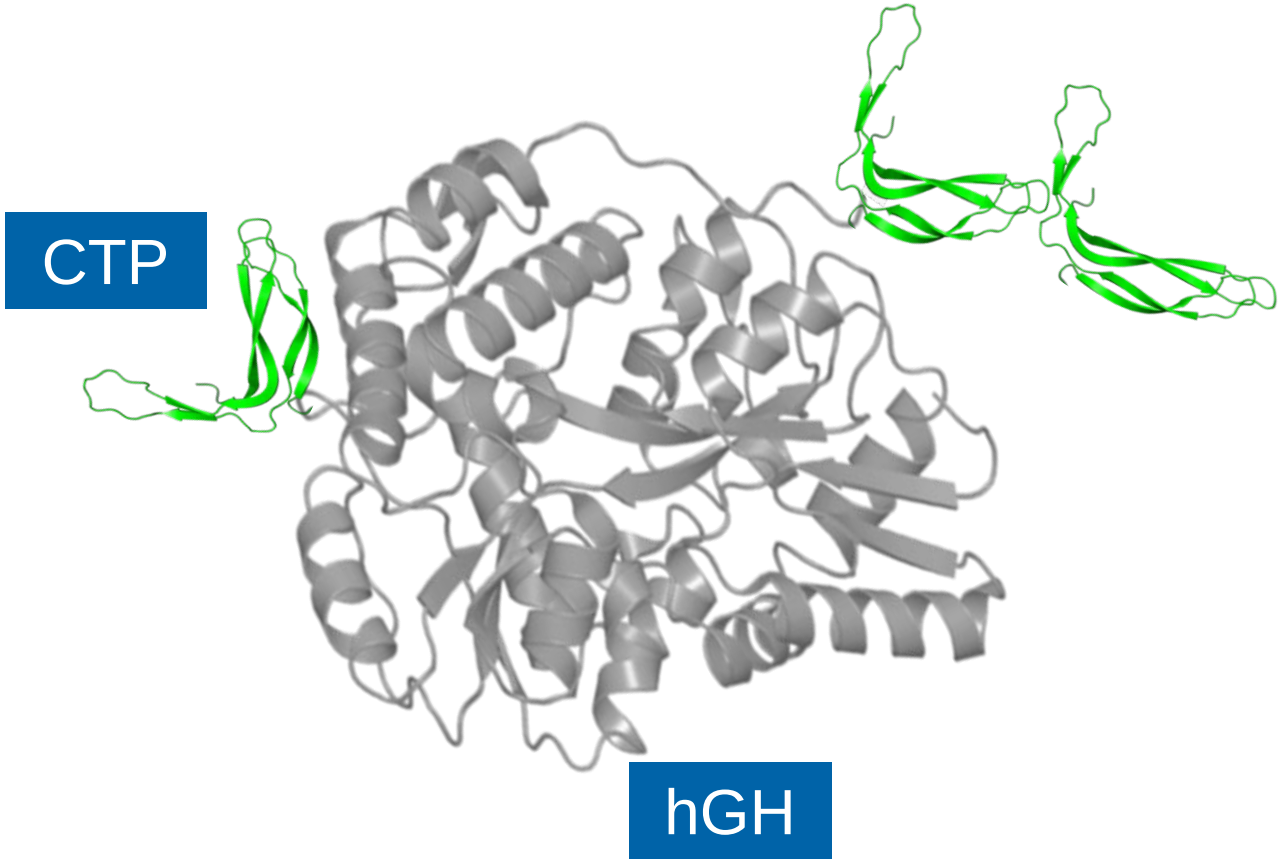
- Pfizer to commercialize somatrogon©
- Highly committed to maintaining global hGH franchise

## Successful Japanese Registration Study

- Height velocity at 12 months of treatment was higher in the somatrogon© group (9.65 cm/year) than in the somatropin group (7.87 cm/year)
- Study met all primary and secondary endpoints and together with the global Phase 3 clinical trial a NDA submission is anticipated in 1H 2021

[1]Pfizer 2018 Annual Report

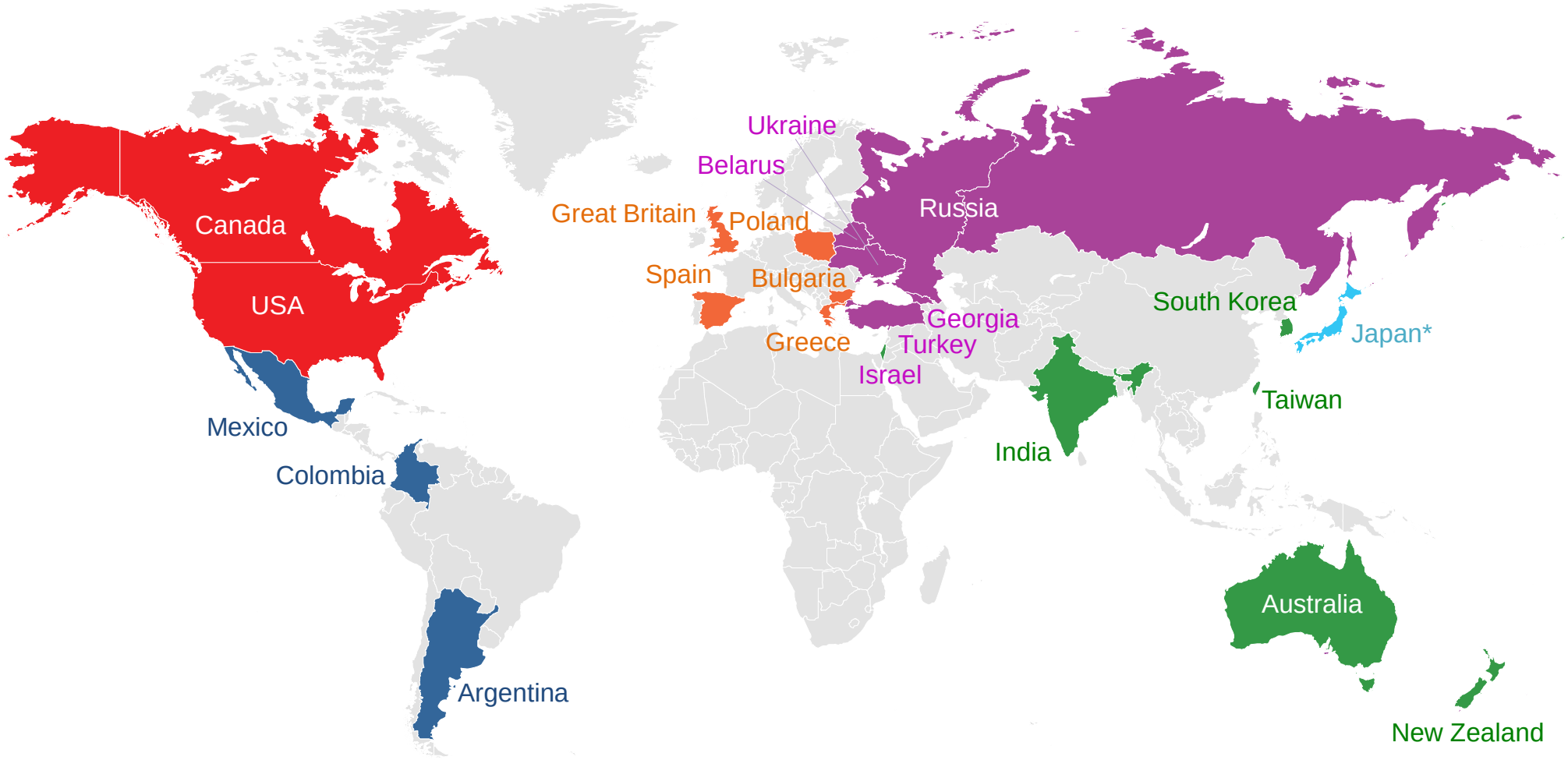
# Somatrogon© CTP Technology




Somatrogon© (hGH-CTP) consists of the natural peptide sequence of native growth hormone and the 28 amino acids of the C-Terminus Peptide of human chorionic gonadotropin hormone. This molecule, compared to current GH replacement therapies, is designed to reduce the injection frequency from daily to once a week in adults and children with GH deficiencies.

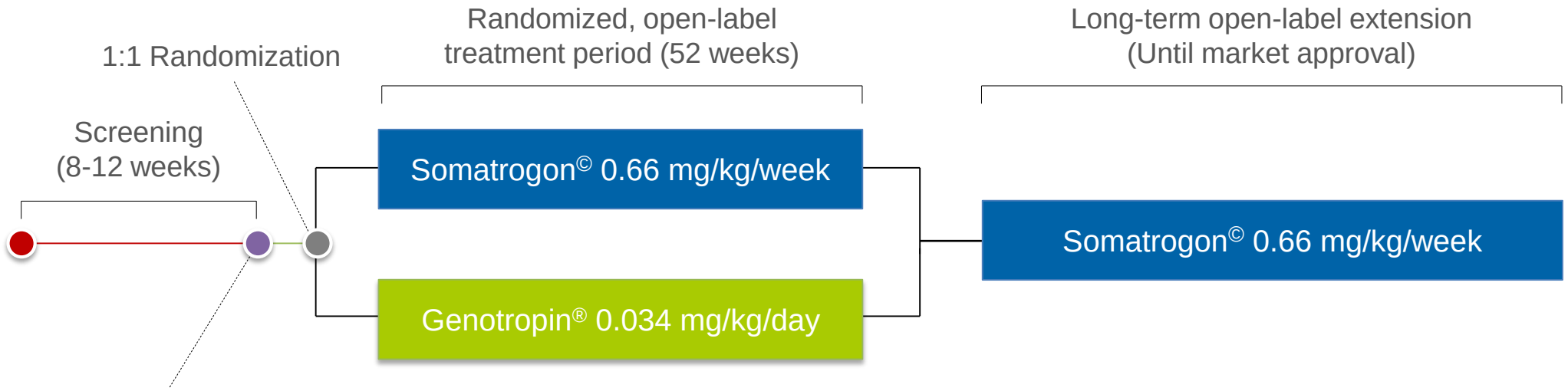
# Somatrogon© Phase 3 Participation

## 106 CLINICAL SITES ACROSS 22 COUNTRIES




* Additional separate Phase 3 clinical trial conducted in Japan

# Somatrogon© Phase 3 Study Design




- 224 subjects enrolled in the main study
- 212 subjects currently in long-term open-label extension

| | |
|---|---|
| Primary Endpoint | • Annual height velocity at month 12 |
| Secondary Efficacy Endpoints | • Height velocity at month 6<br>• Change in height standard deviation score (SDS) at months 6 and 12 |

# Somatrogon Path To Approval

- BLA submission in US anticipated 2H 2020
  - Completion of analysis of immunogenicity and safety data from pivotal Phase 3 study and open label extension study
- Data from two abstracts was presented at the Endo Society's "ENDO Online 2020" on June 8th, 2020
  - "Somatrogon Growth Hormone in the Treatment of Pediatric Growth Hormone Deficiency:  Results of the Pivotal Phase 3"
  - "Interpretation of Insulin-like Growth Factor (IGF-1) Levels Following Administration of somatrogon (a long-acting Growth Hormone-hGH-CTP"
- MAA submission in Europe to follow upon completion of open label study demonstrating benefit and compliance with reduced treatment burden
  - Study completed in September 2020
- NDA submission in Japan anticipated 1H 2021
  - Japanese registration study successful completion announced in June 2020

# Diversified Assets Nephrology



Rayaldee®
calcifediol ER capsules
30 mcg

## First and only extended-release prohormone of active form of vitamin $D_3$

- Once daily oral formulation of the prohormone $25D_3$* addresses significant unmet need
- Only product approved by FDA to treat secondary hyperparathyroidism (SHPT) in patients with stage 3-4 CKD and vitamin D insufficiency
- Reduces plasma iPTH and increases serum 25D, with safety profile similar to placebo
- Minimal adverse effects on serum calcium or phosphorus, key drivers of vascular calcification
- Total prescriptions of Rayaldee increased 54%** in 2Q 2020 compared to 2Q 2019 and was consistent with the first quarter 1Q 2020 despite COVID-19 restrictions

## Healthcare providers have no good options to treat SHPT in stage 3-4 CKD except for Rayaldee

## 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
- Elevated blood levels of intact parathyroid hormone (iPTH) arise from vitamin D insufficiency
- High PTH levels promote vascular calcification, a major cause of CKD morbidity and mortality
- Updated KDIGO practice guidelines recommend against routine use of vitamin D receptor activators and highlight unproven effectiveness of vitamin D supplementation

** As reported by IQVIA

* 25-Hydroxyvitamin $D_3$ or Calcifediol

# Rayaldee for Patients with Stage 5 CKD and Vitamin D Insuffiency

- Phase 2 open label trial to evaluate the safety, efficacy, pharmacokinetics and pharmacodynamics of higher strength Rayaldee in subjects with Vitamin D insufficiency and CKD requiring regular hemodialysis
- Commenced September 2018
  - Approximately 44 patients being treated for 26 weeks
    - Interim data released in  March 2020
    - Topline data expected in 2Q 2021
  - Costs shared with Vifor Fresenius and Japan Tobacco

# FDA-Approved Phase 2 Study with Rayaldee in COVID-19 Patients

**Title:** A Randomized, Double-Blind Placebo-Controlled Study to Evaluate the Safety and Efficacy of **R**ayaldee (calcifediol) **E**xtended-relea**s**e **C**aps**u**l**e**s to Treat Symptomatic Patients Infected with SARS-CoV-2 (**REsCue**)


**Outcome:**
- Prevention of more serious symptoms
- Faster recovery

~160 Patients with mild to moderate COVID-19 (many with stage 3 or 4 CKD)

1:1 randomization (~80 per treatment group)

Loading Dose:
300 mcg/day for 3 days

Maintenance Dose:
60 mcg/day for 24 days

Observation only for 14 days

Endpoints: Time to resolution of symptoms and attainment/maintenance of serum 25D levels within 50-100 ng/mL.
Timeline: Patients screening to commence later in September or in October 2020; completion expected prior to EOY 2020.
Sites: Multiple US sites located in southern Florida, the Central Gulf coast, the Midwest and the Southwest.

# Diversified Assets Broad Development Pipeline



# Rare Disease Pipeline In Development

| INDICATION | PRODUCT | MARKET SIZE | PRECLINICAL | PHASE 1 | PHASE 2 | PHASE 3 |
|---|---|---|---|---|---|---|
| Growth Hormone Deficiency (GHD) | hGH-CTP (Adults) | Pfizer | Once-weekly injection → | → | → | → |
| | hGH-CTP (Children) | | Once-weekly injection → | → | → | → |
| Hemophilia | Factor VIIa-CTP (IV) | $0.6B | → | → | | |
| | Factor VIIa-CTP (SC) | | Once-/twice-weekly injection → | → | | |
| iGF-1 Deficiency | iGF-1-CTP | $0.2B | Once-/twice-weekly → | | | |
| Bowel Disorders | GLP-2 Agonist | $2.0B | Once-weekly → | | | |

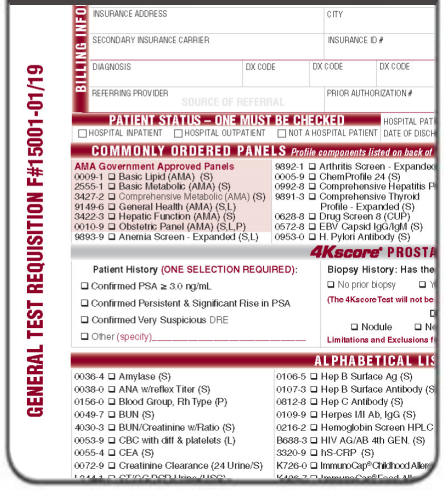
# Diversified Assets
# One of the Nations Largest Reference Laboratories



# BioReference Laboratories Overview

## Leading National Clinical Laboratory


## 80,000 Requisitions Daily


GENERAL TEST REQUISITION F#15001-01/19

BILLING INFO

INSURANCE ADDRESS | CITY

SECONDARY INSURANCE CARRIER | INSURANCE ID #

DIAGNOSIS | DX CODE | DX CODE | DX CODE

REFERRING PROVIDER | PRIOR AUTHORIZATION #

SOURCE OF REFERRAL

PATIENT STATUS – ONE MUST BE CHECKED

☐ HOSPITAL INPATIENT ☐ HOSPITAL OUTPATIENT ☐ NOT A HOSPITAL PATIENT

COMMONLY ORDERED PANELS *Profile components listed on back of*

AMA Government Approved Panels

0009-1 ☐ Basic Lipid (AMA) (S)
2555-1 ☐ Basic Metabolic (AMA) (S)
3427-2 ☐ Comprehensive Metabolic (AMA) (S)
91489-6 ☐ General Health (AMA) (S,L)
3422-3 ☐ Hepatic Function (AMA) (S)
0010-9 ☐ Obstetric Panel (AMA) (S,L,P)
9893-9 ☐ Anemia Screen - Expanded (S,L)

9892-1 ☐ Arthritis Screen - Expanded
0005-9 ☐ ChemProfile 24 (S)
0992-8 ☐ Comprehensive Hepatitis P
9891-3 ☐ Comprehensive Thyroid Profile - Expanded (S)
0628-8 ☐ Drug Screen 8 (CUP)
0572-8 ☐ EBV Capsid IgG/IgM (S)
0953-0 ☐ H. Pylori Antibody (S)

4Kscore® PROSTA

Patient History (ONE SELECTION REQUIRED):

☐ Confirmed PSA ≥ 3.0 ng/mL
☐ Confirmed Persistent & Significant Rise in PSA
☐ Confirmed Very Suspicious DRE
☐ Other (specify)

Biopsy History: Has the
☐ No prior biopsy ☐ Y
(The 4Kscore Test will not be
☐ Nodule ☐ N
Limitations and Exclusions f

ALPHABETICAL LIS

0036-4 ☐ Amylase (S)
0038-0 ☐ ANA w/reflex Titer (S)
0156-0 ☐ Blood Group, Rh Type (P)
0049-7 ☐ BUN (S)
4030-3 ☐ BUN/Creatinine w/Ratio (S)
0053-9 ☐ CBC with diff & platelets (L)
0055-4 ☐ CEA (S)
0072-9 ☐ Creatinine Clearance (24 Urine/S)

0106-5 ☐ Hep B Surface Ag (S)
0107-3 ☐ Hep B Surface Antibody (S)
0812-8 ☐ Hep C Antibody (S)
0109-9 ☐ Herpes I/II Ab, IgG (S)
0216-2 ☐ Hemoglobin Screen HPLC
B688-3 ☐ HIV AG/AB 4th GEN. (S)
3320-9 ☐ hS-CRP (S)
K726-0 ☐ ImmunoCap® Childhood Aller

## 4,000 Employees - 300+ Medical Staff

## Serving 11 Million Patients Annually

## First Class Logistics

- 1.5M+ pick-ups annually
- 17M+ miles driven annually
- 475+ cars in the fleet
- 450+ drivers in logistics

# Areas of Focus

**Targeted Expertise for Physicians**

**Strategic Partnerships**

**Patient Empowerment**

# Targeted Areas of Expertise for Physicians

# Strategic Laboratory Collaboration

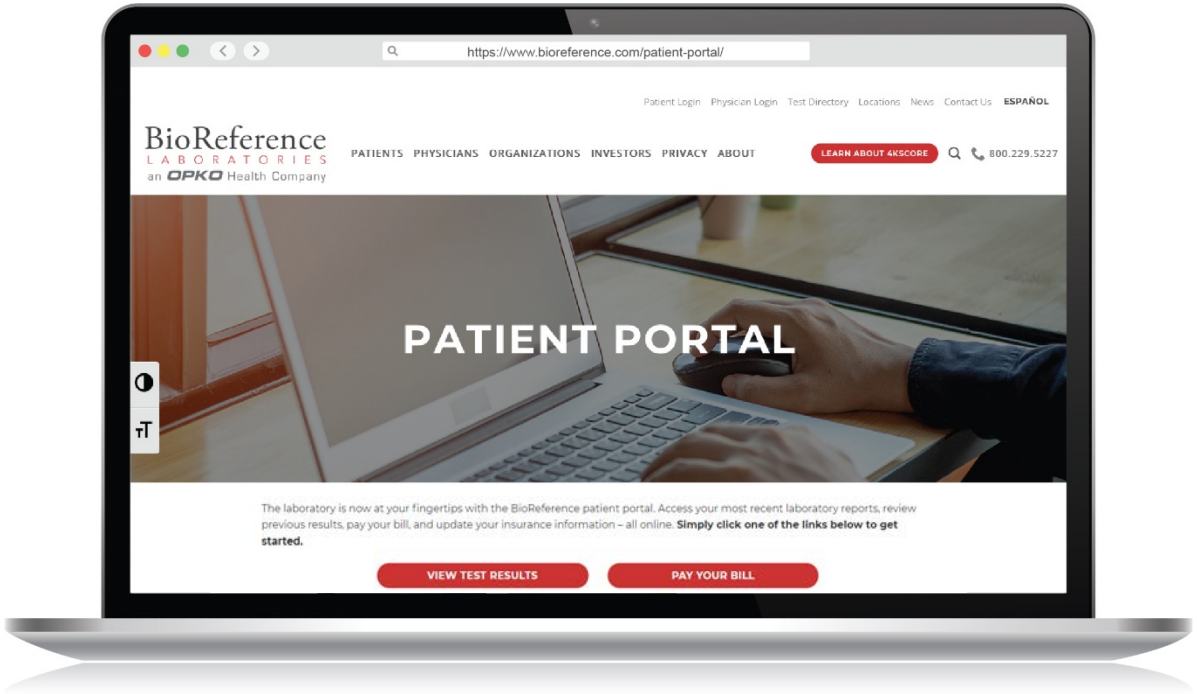
**OPKO Health's BioReference Laboratories and Westchester Medical Center Health Network Enter Into Strategic Laboratory Collaboration to Deliver Operational and Diagnostic Services, May 13, 2020**

- WMCHealth, 10 hospitals through out the Hudson Valley performing over **7 million tests a year**
- Provide administrative services, laboratory equipment, supplies, reference testing and outreach services

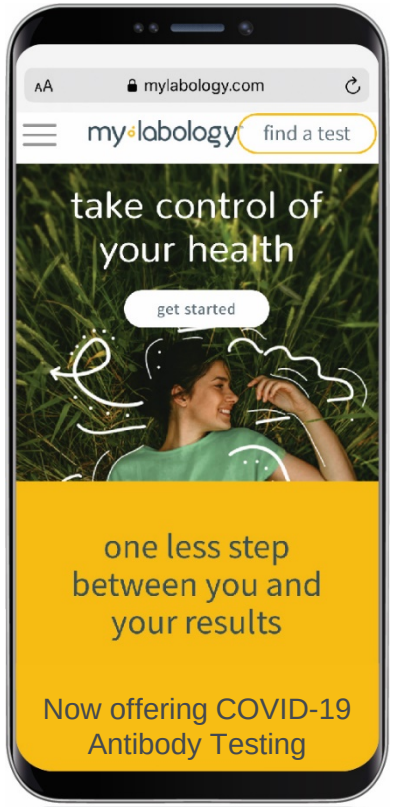
# Patient Empowerment

*"BioReference: The Lab Patients Choose"*

## Patient Portal




## Consumer-initiated Testing



# BioReference's COVID-19 Response

**Serving all 50 states with testing locations in: Elmwood Park, NJ; Melbourne, FL; Gaithersburg, MD; Houston, TX; Campbell, CA**

- Currently, PCR and/or Antibody Testing is being performed out of NJ, FL, MD, TX and CA
- PCR & Antibody turnaround time for "Priority" account is 6 hours once in the lab
- Performed ~5.3 million COVID-19 PCR tests as of September 13, 2020
- Capacity to run more than 70,000 PCR tests per day
- Capacity to run 400,000 antibody tests per day
- Partnering with New York, New Jersey and Florida, multiple cities, municipalities and hospital systems and operating more PCR collection sites than any other commercial laboratory
- Highly visible back to work solutions including partnering with NFL, NBA and MLS

# Leverage Strategic Partnerships to Optimize COVID-19 Testing Access

Westchester Medical Center Health Network

Multiple Large Medical Groups

Municipalities and Government

# Looking Forward

- Many factors continue to drive demand for COVID-19 testing at BioReference Laboratories.
  - Hospitals desire more PCR testing for patients undergoing elective in-patient and outpatient procedures.
  - Hospitals will continue to test their employees for both PCR and antibody presence.
  - Physicians will continue to test their patients for COVID-19 disease.
  - The general public continues to desire to know their antibody status.
- The return of the flu season this fall and winter may have an impact on COVID-19 testing and other respiratory infection testing.
- Employers are working through their strategies for employees to return to work which includes both PCR and antibody testing programs.

# Select Financial Information

**June 30, 2020**

## Balance Sheet

- Cash, cash equivalents & marketable securities:  $21.6 million
- Un-utilized $100m unsecured credit facility and $15m available under ABL with J.P. Morgan Chase resulting in more than $136m of capital available
- Net investments: $26.3 million
- Convertible notes, net:  $216.5 million

## Capital Structure

- Common shares outstanding: 669.8 million

## Income Statement

- Consolidated revenues for 2Q 2020 were $301.2 million compared with $226.4 million for 2Q 2019
  - - Revenue from services were $251.0 million in 2Q 2020 compared with $178.5 million for 2Q 2019
- Net income for 2Q 2020 was $33.7 million ($0.05 per share) compared with net loss of $59.8 million ($(0.10) per share) for 2Q 2019