# UNITED STATES SECURITIES AND EXCHANGE COMMISSION

Washington, DC 20549

#### FORM 8-K

#### **CURRENT REPORT**

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): June 3, 2014

# **OPKO** Health, Inc.

(Exact Name of Registrant as Specified in Charter)

Delaware (State or Other Jurisdiction of Incorporation) 001-33528 (Commission File Number) 75-2402409 (IRS Employer Identification No.)

4400 Biscayne Blvd Miami, Florida 33137 (Address of Principal Executive Offices) (Zip Code)

Registrant's telephone number, including area code: (305) 575-4100

	ck the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under of the following provisions (see General Instruction A.2. below):
	Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
	Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
	Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
П	Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240 13e-4(c))

#### ITEM 7.01. Regulation FD Disclosure.

On June 3, 2014, the Company presented at the Jefferies 2014 Global Healthcare Conference. A copy of the Company's presentation slides is furnished as Exhibit 99.1 to this Current Report on Form 8-K and incorporated herein by reference. The presentation slides are also available on the OPKO website at <a href="https://www.opko.com">www.opko.com</a> under Investor Relations. The information contained on OPKO's website shall not be deemed part of this report.

The information contained in Item 7.01 to this Current Report on Form 8-K and Exhibit 99.1 attached hereto shall not be deemed "filed" for purposes of Section 18 of the Exchange Act, or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing by the Company under the Act, unless expressly stated otherwise.

#### ITEM 9.01. Financial Statements and Exhibits

(d) Exhibits

Exhibit

Number Description

99.1 Jefferies 2014 Global Healthcare Conference Presentation.

#### **SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

OPKO Health, Inc.

By /s/ Adam Logal

Name: Adam Logal

Title: Senior Vice President, Chief Financial Officer

Date June 3, 2014

#### EXHIBIT INDEX

Exhibit

No. Description

99.1 Jefferies 2014 Global Healthcare Conference Presentation.



Jefferies 2014 Global Healthcare Conference June 3, 2014

#### **Cautionary Statement**

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, our ability to achieve high levels of growth, the potential for our products under development, the potential of our next generation prostate markers to dramatically reduce biopsies and increase detection of cancer, our ability to develop, test and launch new products, the expected timing of the studies and trials relating to our products under development, the outcome of our clinical trials and validation studies and that such outcomes will support commercialization, the expected market penetration and size of the market for our products under development, including rolapitant, Rayaldee™ (CTAP-101), hGH-CTP, the 4Kscore™, and our point-of-care diagnostic products for Total-PSA, testosterone, and Vitamin D, the potential benefits of our products under development, including whether the 4Kscore™ will improve selection of candidates for prostate biopsy and result in more than \$1 billion in savings on reduced biopsies and associated hospitalization, our ability to successfully commercialize our product candidates such as rolapitant, the 4Kscore™, Rayaldee™ (CTAP-101) and hGH-CTP, as well as products for other markets such as urology, women's health, cardiology, oncology, iPTH, and infectious disease, whether we will be able to develop Rayaldee™ (CTAP-101) for additional indications and whether Rayaldee™ (CTAP-101) will take significant market share in Stage 3 and 4 CKD patients with SHPT, whether Rayaldee (CTAP-101) will raise serum total 25-hydroxyvitamin D (25D) more effectively than any over-the-counter (OTC) or prescription (Rx) product currently marketed without the risk of hypercalcemia, expectations regarding patent coverage, the expected timing for commencing, completing and obtaining results for our clinical trials, the timing for FDA response to our submissions, the timing for release of trial data and seeking and obtaining FDA and European regulatory approvals, the life span of our patents, and the timing of commercial launch of our product candidates, as well as other non-historical statements. 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 forwardlooking statements, including risks inherent in funding, developing and obtaining regulatory approvals of new, commercially-viable and competitive products and treatments, general market factors, competitive product development, product availability, federal and state regulations and legislation, and integration issues arising from the transactions, 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 possibility of litigation, 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.



#### **OPKO**

### Important Products Available or Coming to Market Near Term

#### **Diagnostics**

- 4Kscore<sup>TM</sup> Test blood test for personalized risk of high-grade prostate cancer
- CLIA-certified urological specialty laboratory
- Claros ® 1 immunoassay system for rapid, lab quality in-office testing (PSA, Testosterone, Vitamin D)

#### Pharmaceuticals

- Vitamin D therapeutics for SHPT\*
- Platform technology to make peptides and proteins long acting to treat growth hormone deficiency, hemophilia, obesity, etc.
- Calcium-free, magnesium-based phosphate binder
- Approved third generation hepatitis B vaccine

\*Secondary Hyperparathyroidism



# **OPKO Diagnostics – Addressing Large Dx Markets**

- 4Kscore<sup>TM</sup> Test
  - Initially targeting pre-prostate biopsy market
  - PSA market 60 million tests globally
  - Recently launched in US at \$395
  - Highly significant clinical data on 1,012 patients presented at AUA plenary session May 18, 2014
  - Launch in Europe: September 2014
- Claros ® 1 Analyzer and Sangia <sup>™</sup> Microfluidic Test Card
  - In office finger-stick blood analysis
  - Initial target assays in US:
    - PSA: 30 million tests, \$750 M
    - Testosterone: 15 million tests, \$525 M
    - Vitamin D: 70 million tests, \$3.5 B





CONVENIENT

LAB QUALITY ACCURACY







# Convenience



# **Claros 1 Update**

#### Testosterone

- FDA: Pre-submission comments received from FDA
- On track to file 510(k) in 2014
- CE Mark: 4Q2014

#### • PSA

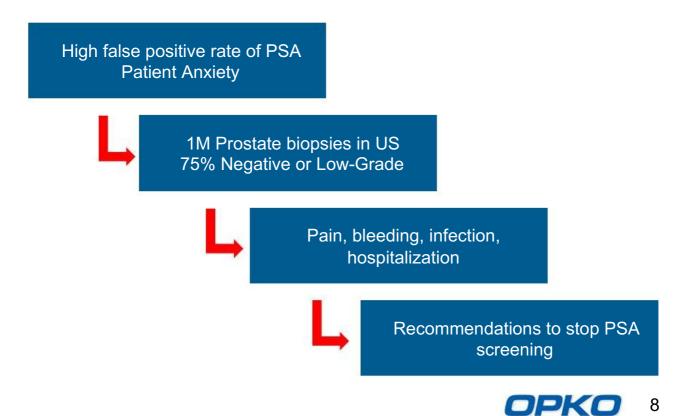
- FDA: Pre-submission response expected in August
- Timing of 510k submission based on longitudinal trial requirements
- CE Mark Update (Formulation and Chemistry): 4Q2014

#### • Vitamin D

- On track to support launch of Rayaldee 1Q2016



# **Challenges in Prostate Cancer Screening**



# **4Kscore Test – Avoiding Unnecessary Prostate Biopsies**

- The **only** test to identify men with high-grade prostate cancer from a blood sample
- Combines results of multiple biomarkers to create a patient's personal risk score
- Based on 10 years of clinical research by scientists at Memorial Sloan-Kettering Cancer Center and leading European cancer centers
- Tested in over 10,000 men in 9 separate clinical studies demonstrating a 27% 82% biopsy reduction
- Validated by OPKO in a prospective, blinded study of 1,012 men



# **4Kscore Test US Clinical Study**

# AKscore™ Test Clinical study sites

**1,012 Patients Enrolled – Prospective Clinical Trial** 



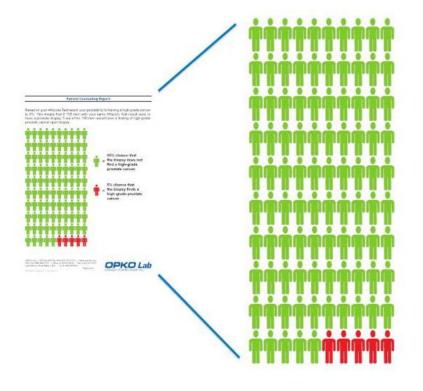
# 4Kscore Test Clinical Study Results in 1,012 Men

Summary					
77.2% 781	Negative or Gleason 6				
22.8%	High-Grade				

- Discrimination: AUC = 0.82
- Risk Calibration
- Decision Curve Analysis
- Biopsy reduction of 30% to 58%



# The 4Kscore Patient Counseling Report for a Result of 5%





95% chance that
the biopsy does not
find a high-grade
prostate cancer.



5% chance that
the biopsy finds a
high-grade prostate
cancer.



# **The 4Kscore Test Conclusions**

- Validated test based on a decade of clinical research and a prospective, multi-institutional, contemporary US clinical trial
- Convenient blood test, cost \$395
- Excellent discrimination for high-grade cancer (AUC = 0.82) and high net benefit for clinical use
- Reduces 30 58% of biopsies
- Provides a calibrated score for informed, shared decision-making between urologist and patient



# **OPKO Pharmaceuticals** — Advanced, Deep Pipeline

Product	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Milestone	Market Size
Rayaldee™ (CTAP101)	SHPT (CKD Stage 3-4 Patients)				$\Rightarrow$	Phase 3 results expected mid- 2014	\$12.0 BN
nGH-СТР	hGH deficiency						\$3.5 BN
Alpharen™ Fermagate)	Hyperphosphatemia (CKD Stage 5 Patients)				$\Rightarrow$		\$1.2 BN
Rolapitant	CINV	Outlicensed to	TESARO		$\Rightarrow$	NDA submission targeted mid-2014	\$1.5 BN
Sci-B-Vac <sup>TM</sup>	Hepatitis B (CKD Stage 5 Patients)				>		\$0.2BN
Lunacalcipol™ CTA018)	Moderate to severe SHPT (CKD Stage 5 Patients) & Psoriasis			<b>&gt;</b>			\$1.5 BN
CTAP201	Mild to moderate SHPT (CKD Stage 5 Patients)						\$1.1 BN
Factor VIIa-CTP	Hemophilia						\$1.7 BN
AntagoNAT Platform	Cancer, CV, metabolic and orphan disease						\$1.0 BN
Oxyntomodulin	Diabetes, Obesity						\$15 BN



# Rayaldee (CTAP101) - A Late-Stage Investigational Drug

#### **Product Overview**

- Oral formulation of 25D3\* addresses significant unmet need
- Safe and effective treatment for elevated PTH (SHPT) associated with low 25D levels in Stage 3-4 CKD
- Achieves reliable increases in serum 25D and reductions in plasma PTH
- Lower risk of side effects compared to active 1,25D products
- Potential for additional indications including elderly, osteoporosis & cancer
- \* 25-Hydroxyvitamin D₃\*\* 1,25-Dihydroxyvitamin D

#### **Clinical Status**

- Clinical development guided by prominent Scientific Advisory Board
- Top line phase 3 data available in mid-2014
- NDA filing in 1Q 2015

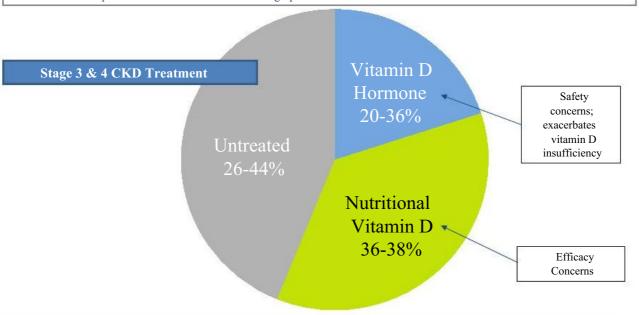
#### **Intellectual Property**

- Rayaldee US patents issued, protected through 2028
- Additional global patents allowed or pending



# Rayaldee - Commercial Opportunity

- Low serum 25D and elevated plasma PTH are prevalent in CKD Stage 3-4 patients
  - 8 million CKD Stage 3-4 patients in the US
  - 4 million patients with low serum 25D and high plasma PTH



Rayaldee is expected to take significant market share in Stage 3 and 4 CKD patients suffering from SHPT – a potential \$12 billion revenue opportunity

Source: BioTrends Research Group, Inc. December 2010



# Comparison of Vitamin D Therapies for Stage 3-4 CKD

		Effect on Blood Levels of:			
Drug	Туре	25D**	Ca	iPTH	
Rayaldee	Calcifediol (25-hydroxyvitamin D <sub>3</sub> )	Rx	1	-	1
Vitamin D	Cholecalciferol/ Ergocalciferol (vitamin D <sub>3</sub> /vitamin D <sub>2</sub> )	OTC	•	-	
Drisdol™*	Ergocalciferol (vitamin D <sub>2</sub> )	Rx		-	
Rocaltrol™	Calcitriol (1α,25-dihydroxyvitamin D <sub>3</sub> )	Rx		1	-
Hectorol™	Doxercalciferol (1\alpha-hydroxyvitamin D <sub>2</sub> )	Rx	1	1	1
Zemplar™*	Paricalcitol (19-nor-10,25-dihydroxyvitamin D <sub>2</sub> )	Rx		1	1

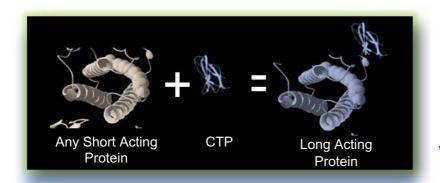
Rayaldee is expected to raise serum total 25-hydroxyvitamin D (25D) and lower plasma iPTH more effectively than any currently marketed over-thecounter (OTC) or prescription (Rx) product without the risk of hypercalcemia.

\*And generics \*\*25-hydroxyvitamin D



## CTP Technology: Clinically Validated Proprietary Platform

• CTP increases protein circulation time



CTP – a natural sequence created during evolution to enhance the longevity of peptides and proteins without increasing toxicity

- Merck's long acting FSH-CTP (Elonva <sup>™</sup>):
  - o Received EU marketing authorization in 2010; NDA filed Q3 2013
  - Single FSH-CTP injection replaces 7 daily FSH injections in fertility treatment
- Two licensees of CTP technology for human therapeutics:
  - o Merck (holds license for 4 fertility-focused proteins)
  - OPKO's Biologics (holds license for all other rights)



# **hGH-CTP Opportunity**

- \$3.5 billion market, growing 5% annually
- Once-a-week injection (current products require daily injections)
- Small needle size (31 gauge) due to low viscosity
  - Competitive long acting formulations have high viscosity
- Superb clinical, safety and immunogenicity profile
- Human growth hormone is used for:
  - Growth hormone deficient children
  - Growth hormone deficient adults
  - Short stature
  - Off label
- Orphan drug designation in the US & EU for children & adults



## **hGH-CTP Clinical Development**

#### • Adult Pivotal Phase 3 trial (ongoing)

- 189 patients
- Primary efficacy endpoint: reduction in truncal fat mass after 6 months vs. placebo
- Secondary efficacy endpoints include:
  - Reduction in total body fat
  - · Increase in lean body mass
- Single pivotal trial required by FDA for BLA submission in 2016

#### • Pediatric GHD Phase 2 trial (advanced stage)

- Enrollment completed March 2014
- 4 cohorts:
  - 3 dose levels of once-weekly hGH-CTP
  - Commercially available standard daily rhGH treatment
- Key outcome: height velocity
- Positive clinical data to be presented at ENDO meeting June 21-24, 2014
- Phase 3 to commence by 1H2015



### FVIIa -CTP: Long Acting for Treating Hemophilic Patients

- \$1.7 billion market
  - Growing 7% annually
  - 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
- Pharmacological studies in hemophilic mice and dogs FVIIa-**CTP** demonstrated:
  - Potential for substantial improvement in the quality of life of patients via subcutaneous administration
  - Reduce frequency of injection during on-demand therapy
  - Enable prophylactic treatment while reducing the frequency of injections to 2-3 times a week
- Phase 2a study in hemophilic patients: initiated H2 2014
- Orphan drug designation in the US



# **MOD-6031: Long Acting Oxyntomodulin for Obesity**

- >\$15 billion market
  - Growing rapidly
- Nature's Appetite Control Mechanism **Oxyntomodulin** 
  - Natural appetite suppressor
  - Secreted by the digestive system following food intake and induces satiety in the brain
  - Increases glucose tolerance
  - Short acting requires 3 injections per day
- **MOD-6031 Long Acting Oxyntomodulin**weekly injection studies in mutant obese mice and diet induced obese mice demonstrated:
  - Significantly inhibited food intake and reduced body weight by reducing fat
  - Reduced cholesterol levels
  - Improved glycemic control
- Phase 1 study to be initiated 1H2015
- MOD-6031 is expected to provide superior long-term therapy for obesity and diabetes type II patients



# **Rolapitant – Potential Near-term Revenue Driver**

- Rolapitant out-licensed to Tesaro in December 2010
  - Payments of up to \$121 million
  - Double-digit tiered royalties
- Differentiated cancer supportive care product with \$1.5B US Market Opportunity
  - Potent neurokinin-1(NK-1) receptor antagonist for chemotherapy-induced nausea and vomiting (CINV)
  - Opportunity to differentiate on convenience, market access and safety
    - · Single dose

- Lack of CYP 3A4 drug-drug interactions
- Long acting
- Oral and IV formulations allow full market access
- NDA submission targeted for mid-2014
  - All three Phase 3 trials (MEC \* and HEC \*\*) achieved primary endpoint
  - Primary endpoint: complete response (no emesis and no use of rescue medication)
  - Third Phase 3 trial (HEC) also achieved all secondary endpoints, including:
    - Complete response in acute (0-24 hrs) and overall (0-120 hrs) phase of CINV
    - · No significant nausea

\*Moderately emetogenic chemotherapy \*\*Highly emetogenic chemotherapy



### **Strategic Investments**

#### Proprietary Technologies with Significant Upside Potential

- **ARNO Therapeutics,** Inc. (OTC: ARNI) (~5% equity interest)
  - Anti-progestins for breast (phase 2), endometrial and prostate cancers
- **Zebra Biologics**, Inc. (~19% equity interest)
  - Combinatorial antibody libraries based on function in human cell screens
- OAO Pharmsynthez (MICE: LIFE) (~17% equity interest)
  - Russian developer and marketer of new drugs
- RXi Pharmaceuticals Corporation (NASDAQ: RXII) (~17% equity interest)
  - sRNA to prevent hypertrophic scars (phase 2)
- Cocrystal Pharma, Inc. (OTC: COCP) (~16% equity interest)
  - New anti-virals (Hepatitis C, flu, dengue fever)
- **Fabrus**, Inc. (~12% equity interest\*)
  - Antibodies against difficult targets (e.g., G protein-coupled receptor, ion channels)
- **Neovasc**, Inc. (NASDAQ: NVCN) (~6% equity interest)
  - Cardiology devices
- ChromaDex, Inc. (OTC: CDXC) (~2% equity interest)
  - New nutritional supplement APIs

(As of March 31, 2014)

\* Merger with Senesco Technologies, Inc. (OTC: SNTI) completed May 19, 2014

