### **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): January 13, 2015

# OPKO Health, Inc.

(Exact Name of Registrant as Specified in Charter)

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

> 4400 Biscayne Blvd Miami, Florida 33137

(Address of Principal Executive Offices) (Zip Code)

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

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under

any	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))
	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 January 13, 2015, the Company made a presentation at the 33rd Annual J.P. Morgan 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 33rd Annual J.P. Morgan 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 January 13, 2015

#### EXHIBIT INDEX

Exhibit No.

Description

99.1 33rd Annual J.P. Morgan Healthcare Conference Presentation.



33 <sup>rd</sup> Annual J.P. Morgan Healthcare Conference January 2015

### **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 the 4Kscore<sup>TM</sup> to reduce prostate biopsies by 30-58% and predict the risk of aggressive prostate cancer, our ability to develop, test and launch new products, the expected timing of the clinical studies and regulatory submissions 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 without limitation, Rolapitant  $^{TM}$ , Rayaldee  $^{TM}$  (CTAP-101), hGH-CTP $^{TM}$ , 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, predict the risk of distant metastases, and result in \$2 to 4 billion in healthcare savings, whether MOD-6031 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, 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, whether we can reach more than half of the CKD population with a small sales force, expectations regarding patent coverage, the expected timing for commencing, completing and obtaining results for our clinical trials, the timing for release of trial data and seeking and obtaining FDA and European regulatory approvals, 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 forward-looking 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 Health Focus on Large Market Potential Products**

Diagnostics	<ul> <li>Claros ®1 immunoassay system for rapid, lab quality in-office testing</li> <li>4Kscore™ blood test for aggressive prostate cancer risk</li> </ul>					
Pharmaceuticals	<ul> <li>New Vitamin D products for SHPT</li> <li>Platform technologies to make peptides and proteins long-acting for growth hormone deficiency, hemophilia, obesity</li> <li>Calcium-free, magnesium-based phosphate binder</li> <li>Approved third generation hepatitis B vaccine</li> </ul>					
International Markets	<ul> <li>Established businesses in:</li> <li>Mexico</li> <li>Spain</li> <li>Israel</li> <li>Chile</li> <li>Brazil</li> <li>Uruguay</li> </ul>					
Opportunistic Investments	<ul> <li>Innovative technologies</li> <li>Antibodies</li> <li>RNAi</li> <li>Anti-virals</li> <li>Cardiovascular devices</li> </ul>					



# 4Kscore Test to Identify Risk for Aggressive **Prostate Cancer**

- Two types of prostate cancer:
  - Low grade, non-lethal
  - Aggressive, lethal
- 4Kscore: ONLY blood test to predict risk of aggressive form
- Backed by 10 years of clinical research at Memorial Sloan-**Kettering Cancer Center**
- Validated by OPKO in a prospective, blinded study of 1,012
- Global market for pre-biopsy test (not screening): \$800M
- Use of 4Kscore would reduce 30-58% of unnecessary prostate biopsies
- Healthcare savings of \$2-4 billion in the US



# **4Kscore Commercial Update**

- 4Kscore US clinical trial manuscript in press (Eur. Urol.)
- 4Kscore CPT code application accepted by AMA CPT Editorial Board (MAAA Category 3)
- 4Kscore was discussed at NCCN Guidelines meeting Nov. 2014 for potential inclusion in 2015 guidelines
- Over 465 US urologists have used the 4Kscore test in routine practice
- Launched by OPKO in Europe September 15, 2014; samples tested at University of Barcelona barnaclinic+
- Mexico launch in January 2015; samples will be tested at OPKO Lab in Nashville, TN



Claros ®1: Rapid Testing in the Physician Office



# **Claros 1 Platform Addresses Large Dx Markets**

### • Testosterone

- 15 million US tests: \$525 M
- 510(k) filing in 2Q2015

#### • PSA

- 30 million US tests: \$750 M
- Intended use to focus on screening (early detection) claim
- PMA filing in 1Q2016

### • Vitamin D

- 70 million US tests: \$3.5 B
- On track to support launch of Rayaldee 1Q2016

### • Lyme Disease

- Claros 1 selected as platform for NIH funded development
- OPKO has exclusive option to diagnostic test (\$120M US Market)



# OPKO Pharmaceuticals — Advanced, Deep Pipeline

Product	Indication	Preclinical	Phase 1	Phase 2	Phase 3	Milestone	Market Size
Rayaldee <sup>TM</sup> (CTAP101)	SHPT (CKD Stage 3-4 Patients)					Phase 3 results reported 3Q 2014	\$12.0 BN
hGH-CTP	hGH deficiency	Collaboration	with Pfizer				\$3.5 BN
Alpharen™ (Fermagate)	Hyperphosphatemia (CKD Stage 5 Patients)					8	\$1.2 BN
Rolapitant	CINV	Outlicensed to	TESARO			NDA accepted November, 2014	\$1.5 BN
Sci-B-Vac™	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	<b></b>					\$1.7 BN
AntagoNAT Platform	Cancer, CV, metabolic and orphan disease	<b></b>					\$1.0 BN
Oxyntomodulin	Diabetes, Obesity	<b></b>					\$15 BN



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

#### **Product Overview**

- Oral formulation of 25D 3\* addresses significant unmet need
- Achieves reliable increases in serum 25D and reductions in plasma PTH in Stage 3-4 CKD
- Lower risk of side effects compared to active 1,25D
   products
- Phase 1 cancer trail begun
  - 25-Hydroxyvitamin D<sub>3</sub>
- \*\* 1,25-Dihydroxyvitamin D

#### **Clinical Status**

- Clinical development guided by prominent Scientific Advisory Board
- Positive Phase 3 data
- NDA submission target: 1Q2015

#### **Intellectual Property**

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



# Market Opportunity: Chronic Kidney Disease (U.S.)

The CKD patient population is large and growing as a result of:

- **Obesity**
- Hypertension
- Diabetes

·	naveles		% of CKD Patients with:				
Stage	Kidney Function	CKD Prevalence	Vitamin D Insufficiency(↓ 25D)	SHPT ( PTH)	Hyperphosphatemia ( Phosphorus)		
3	Moderate impairment	18.7 Million*	70%	56%	37%		
4	Severe impairment	1.4 Million*	80%	60%	50%		
5	Failure	0.5 Million*	90%	90%	70%		

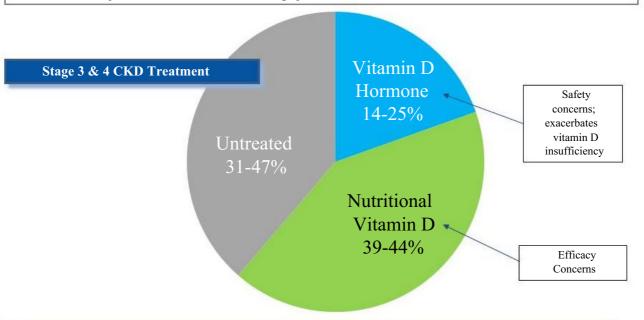
Over half of the CKD population can be reached with a small sales force targeting nephrologists and endocrinologists

\*US Renal Data Service 2013 Annual Data Report Sources: Levin, A et al., Kidney International 2007; 71: pp.31-38.
Gonzalez, E et al. Am J Nephrol 2004;24:503-510.
LaClair, R et al. Am J Kidney Dis 2005;45:1026-1033.



# Rayaldee - Commercial Opportunity

- Low serum 25D and elevated plasma PTH are prevalent in CKD Stage 3-4 patients
  - 20 million CKD Stage 3-4 patients in the US
  - 8 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 2013



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

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

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



# **OPKO Biologics:** Leader in BioBetter Drugs

Developing biobetter long acting proteins and peptides





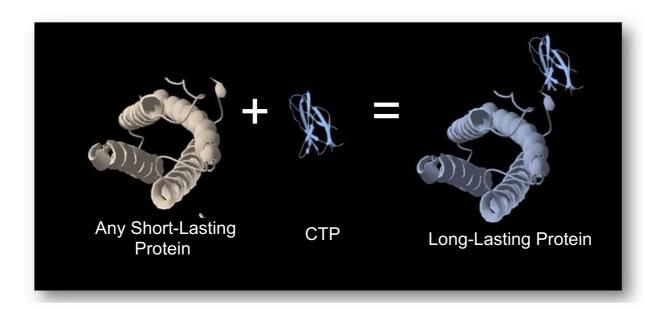
**Reversible Pegylation Technology** (Peptides and small molecules)

**CTP Technology** (Recombinant Proteins)

- Dramatically reduce injection frequency
- · Reduce drug load
- Reduce potential side-effects for most proteins, peptides and small molecules
- Maintain drug bio-activity
- Validated platform technologies safe and effective
  - Preclinical & clinical proof-of-concept in multiple compounds



# hGH-CTP – The Long Acting hGH



Natural sequence No need for linker



# Global Collaboration with Pfizer for OPKO's Long-Acting Human Growth Hormone (hGH-CTP)

#### **Collaboration Terms:**

#### **Financial**

- \$295 M up-front payment
- \$275 M for achievement of regulatory based milestones

#### **Development**

- OPKO responsible for funding development program for the key indications:
  - Adult and Pediatric Growth Hormone Deficiency (GHD)
  - Pediatric Short for Gestational Age
- Pfizer responsible for funding:
  - Development programs for additional indications
  - All Post Marketing Studies
  - · All Commercialization Activities

#### Commercial

- Initial double digit tiered royalties on sales of Adult GHD
- Profit sharing commencing upon launch for Pediatric GHD encompassing combined sales for all indications of OPKO's hGH-CTP and Pfizer's Genotropin
- Pfizer Genotropin represents about 25% of the world market with annual revenues exceeding \$700 M



# **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)

- 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



# **hGH-CTP Clinical Development** Pediatric GHD Phase 2 trial (advanced stage)

- 4 Cohorts:
  - 3 dose levels of once-weekly hGH-CTP
  - Commercially available standard daily rhGH treatment
- Key outcome: height velocity
- Positive clinical data presented at ENDO meeting June 2014
  - All doses provided excellent growth response compared to control group and historical controls
  - Promising safety profile
  - hGH-CTP mean annualized height velocity at 6m ranged from 12.25-14.37cm, compared to annual height velocity of ~10cm as published by Bakker (2008) and Ranke (2010) for the same GHD patient population (peak GH, age)



### **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
- studies in hemophilic mice and dogs FVIIa-CTP Pharmacological demonstrated:
  - Reduce injection frequency during on-demand therapy
  - Enable prophylactic treatment while reducing the frequency of injections to 2-3 times a week
  - Potential for substantial improvement in the quality of life of patients via subcutaneous administration
- Phase 2a study in hemophilic patients to be initiated 1Q 2015
- Orphan drug designation in the US and EU



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

- WHO estimates 500 million severely overweight or obese people
- >\$15 billion market growing rapidly
- Oxyntomodulin: Nature's Appetite Control Mechanism
  - Natural appetite suppressor
  - Secreted by the digestive system following food intake and induces satiety
  - Increases glucose tolerance
  - Short acting requires 3 injections per day
- MOD-6031 Long Acting Oxyntomodulinweekly 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 mid 2015
- MOD-6031 is expected to provide SAFE 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 chemotherapyinduced nausea and vomiting (CINV)
  - Opportunity to differentiate on convenience, market access and safety
    - Single dose

• Lack of drug-drug interactions

· Long acting

- Oral and IV formulations allow full market access
- NDA accepted November 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
- Also effective to prevent nausea \*Moderately emetogenic chemotherapy \*\*Highly emetogenic chemotherapy



### **Strategic Investments**

### Proprietary Technologies with Significant Upside Potential

- ARNO Therapeutics, Inc. (OTC: ARNI) (~4% 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) (~11% equity interest)
  - sRNA to prevent hypertrophic scars (phase 2)
- Cocrystal Pharma, Inc. (OTC: COCP) (~15% equity interest)
  - New anti-virals (Hepatitis C, flu, dengue fever)
- Sevion Therapeutics, Inc. (OTC: SVON) (~4% 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 September 30, 2014)

