

---

---

**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): September 24, 2015**

---

**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

---

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 September 24, 2015, the Company posted an updated investor presentation on its website. A copy of the Company's presentation is furnished as Exhibit 99.1 to this Current Report on Form 8-K and incorporated herein by reference. The presentation is also available on the OPKO website at [www.opko.com](http://www.opko.com) 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*

<u>Exhibit Number</u>	<u>Description</u>
99.1	Corporate Presentation of September 24, 2015.

---

**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 September 24, 2015

---

## EXHIBIT INDEX

<u>Exhibit No.</u>	<u>Description</u>
99.1	Corporate Presentation of September 24, 2015.

# ***OPKO***

---

SEPTEMBER 2015

**Advancing Our Deep Pipeline  
of Pharmaceuticals and  
Diagnostics**



## 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>®</sup> to reduce prostate biopsies 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 approval for 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, Rayaldee<sup>™</sup>, hGH-CTP, the 4Kscore, Factor VIIa-CTP, oxyntomodulin, and our point-of-care diagnostic product for PSA, 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 40-55% cost savings, the expected PMA submission date for PSA, expected per patient savings, that 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, hGH-CTP and Rayaldee 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) product currently marketed without the risk of hypercalcemia, whether we can reach more than half of the CKD population with a small sales force, our ability to establish a sales and marketing and clinical support infrastructure for Rayaldee and the timeline for doing so, the expected PDUFA date and launch date for Rayaldee, 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 as well as reimbursement coverage, and the timing of commercial launch of our product candidates, expectations about near term profitability, that EirGen will manufacture our current and future products resulting in higher margins, that Bio-Reference's vast array of genetics and genomics data will benefit OPKO, 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 integration challenges with Bio-Reference, 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, 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 is a multinational biopharmaceutical and diagnostics company that seeks to establish industry-leading positions in large growth markets by leveraging its discovery, development, and commercialization expertise

**OPKO is positioned for strength and growth in large target markets with significant unmet needs**



## VALUE DRIVERS



### GLOBAL AGREEMENT WITH PFIZER

OPKO and Pfizer entered Global Agreement for long acting human growth hormone (hGH-CTP) in December 2014

\$295 million up front payments to OPKO;  
Up to an additional \$275 million in milestones prior to profit sharing

Pfizer's Genotropin represents about 23% of the world market and has annual revenues exceeding \$700 million; worldwide market is \$3+ billion and growing

Profit sharing shall commence upon commercial launch for Pediatric GHD encompassing combined sales for all indications of OPKO's hGH-CTP and Pfizer's Genotropin

### TRANSFORMATIVE DIAGNOSTICS

Acquisition of Bio Reference Labs creates near term opportunities for vertical integration with OPKO's diagnostic pipeline

Bio Reference Labs reaches 10 million patients annually and has grown over the past 20 years at a CAGR over 20%

Generated \$832 million in profitable revenue last year as the third largest full service reference laboratory in U.S.

Will provide national marketing and distribution for OPKO's diagnostic pipeline with an initial focus on launching the 4Kscore and Claros 1® platform

### RAYALDEE'S PDUFA DATE SET

OPKO's Rayaldee PDUFA date set for March 29, 2016

Addresses significant unmet market need in a potential \$12 billion market

Rayaldee targets Stages 3-4 CKD, which has an estimated patient population of 20 million in the U.S.

### TESARO RECEIVES FDA APPROVAL

OPKO licensee Tesaro receives FDA approval for VARUBI (rolapitant)

OPKO to receive up to \$110 million of additional milestone payments

Double digit tiered royalties with a product launch targeted for Q4 2015

U.S. market opportunity exceeds \$1 billion annually



## NEAR TERM CATALYSTS



DATE	POTENTIAL CATALYST	SIZE OF ADDRESSABLE MARKET	COMPETITIVE ADVANTAGE
2015 / 2016	Coverage decisions on Reimbursement for 4Kscore test	> \$1 billion annual sales	Greater accuracy; Only predictor of aggressive prostate cancer; Simple non-invasive blood test
Q4 2015	Commercial launch of VARUBI™ (rolapitant)	> \$1 billion annual sales	Longer acting; sustained coverage; and IV; Allow full market access
Q4 2015	Begin patient dosing in Phase 2a study of Factor VII-CTP	\$1.7 billion	Only 25% of patients are currently treated; Orphan Drug designation in U.S. and EU; reduced frequency of injection
March 29, 2016	FDA Approval of Rayaldee	\$12 billion	Significant unmet market need for Stages 3-4 CKD (20 million patients)
2H 2016	Phase 3 clinical trial results for hGH-CTP for adults	\$3 billion	Once a week injection versus daily injections; orphan drug designation in the U.S. and EU

## OPKO PHARMACEUTICALS' ADVANCED, DEEP PIPELINE



PRODUCT	INDICATION	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	MILESTONE	MARKET SIZE
<b>Royaldee™ (CTAP101)</b>	SHPT (CKD Stage 3-4 Patients)					<b>PDUFA date March 29, 2016</b>	<b>\$12.0 BN</b>
<b>hGH-CTP</b>	hGH deficiency						<b>\$3.0 BN</b>
<b>Alpharen™ (Fermagate)</b>	Hyperphosphatemia (CKD Stage 5 Patients)						<b>\$1.2 BN</b>
<b>VARUBI (rolapitant)</b>	CINV					<b>Product Launch Q4 2015</b>	<b>\$1.0 BN</b>
<b>CTAP201</b>	Mild to moderate SHPT (CKD Stage 5 Patients)						<b>\$1.1 BN</b>
<b>Factor VIIa-CTP</b>	Hemophilia					<b>Phase 2a trial in 4Q 2015</b>	<b>\$1.7 BN</b>
<b>Oxyntomodulin</b>	Diabetes, Obesity					<b>Phase I trial targeted for Q1 2016</b>	<b>\$15 BN</b>
<b>AntagoNAT Platform</b>	Cancer, CV, metabolic and orphan disease						<b>\$1.0 BN</b>

**PDUFA DATE MARCH 29, 2016; MET ALL PHASE 3 ENDPOINTS**

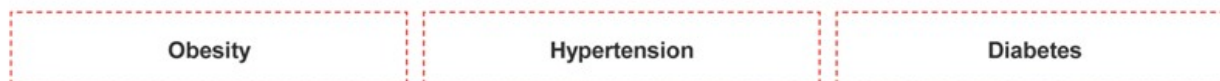
- Product launch estimated 2H 2016 in a \$12 billion market
- Rayaldee is expected to take significant market share in Stage 3 and 4 CKD patients suffering from SHPT
  - 20 million patients in the U.S.
- Modified-release (MR) oral formulation of 25D3\* addresses significant unmet market need
- Safe and effective treatment for elevated PTH (SHPT) associated with low 25D levels in Stages 3–4 CKD
- Existing therapies have efficacy and safety concerns
- Achieves more reliable increases in serum 25D and reductions in plasma PTH than nutritional vitamin D
- Lower risk of side effects compared to active 1,25D\*\* products
- Additional potential for new indications including stage 5 CKD, institutionalized elderly, osteoporosis & cancer

\* 25-Hydroxyvitamin D<sub>3</sub> or Calcitriol  
\*\* 1,25-Dihydroxyvitamin D<sub>3</sub> or Calcitriol

## RAYALDEE – STEPS TO COMMERCIALIZATION



The Chronic Kidney Disease (CKD) patient population is large and growing as a result of:



Stage	Kidney Function	CKD Prevalence	% of CKD Patients with		
			Vitamin D Insufficiency (↓25D)	SHPT (↑ PTH)	Hyperphosphatemia (↑ Phosphorus)
3	Moderate impairment	18.7 Million*	71%	40%	37%
4	Severe impairment	1.4 Million*	83%	82%	50%
5	Failure	0.5 Million*	97%	95%	70%

\*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; Tentori, F et al., Clin J Am Soc Nephrol 2015; 10:98-109

			Effect on Blood Levels of:		
Drug	Active	Type	25D**	Ca	iPTH
<b>Royaldee</b>	<b>Calcifediol</b> (25-hydroxyvitamin D <sub>3</sub> )	<b>Rx</b>	<b>Significant Increase</b>	<b>No Change</b>	<b>Significant Decrease</b>
Vitamin D	Cholecalciferol/Ergocalciferol (vitamin D <sub>3</sub> /vitamin D <sub>2</sub> )	OTC	Minor Increase	No Change	Minor Decrease
Drisdol™*	Ergocalciferol (vitamin D <sub>2</sub> )	Rx	Minor Increase	No Change	Minor Decrease
Rocaltrol™*	Calcitriol (1α,25-dihydroxyvitamin D <sub>3</sub> )	Rx	Decrease	Increase	Significant Decrease
Hectorol™*	Doxercalciferol (1α-hydroxyvitamin D <sub>2</sub> )	Rx	Decrease	Increase	Significant Decrease
Zemplar™*	Paricalcitol (19-nor-1α,25-dihydroxyvitamin D <sub>2</sub> )	Rx	Decrease	Increase	Significant Decrease

\*And generics

\*\*25-hydroxyvitamin D

## LONG-ACTING HUMAN GROWTH HORMONE (hGH-CTP)



### Terms of Global Collaboration with Pfizer:

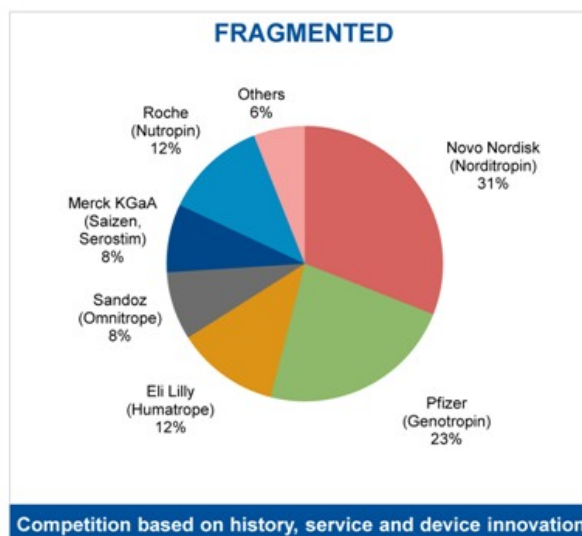
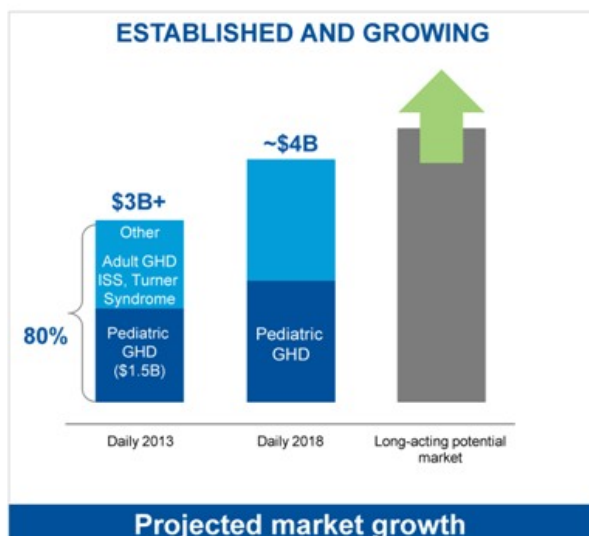
FINANCIAL	<ul style="list-style-type: none"><li>▪ \$295 million up-front payments to OPKO</li><li>▪ \$275 million to OPKO upon achievement of regulatory based milestones</li></ul>
DEVELOPMENT	<ul style="list-style-type: none"><li>▪ OPKO responsible for funding development program for the key indications:<ul style="list-style-type: none"><li>• Adult and Pediatric Growth Hormone Deficiency (GHD)</li><li>• Pediatric Short for Gestational Age</li></ul></li><li>▪ Pfizer responsible for funding:<ul style="list-style-type: none"><li>• Development programs for additional indications</li><li>• All Post Marketing Studies</li><li>• All Commercialization Activities</li></ul></li></ul>
COMMERCIAL	<ul style="list-style-type: none"><li>▪ Initial double digit tiered royalties on sales of Adult GHD</li><li>▪ Profit sharing commencing upon launch for Pediatric GHD encompassing combined sales for all indications of OPKO's hGH-CTP and Pfizer's Genotropin</li><li>▪ Pfizer Genotropin represents about 23% of the world market with annual sales exceeding \$700 million; total worldwide market is \$3+ billion and growing</li></ul>

### PHASE 3 ENROLLMENT COMPLETED FOR ADULTS IN JULY 2015

- **New molecular entity that maintains natural native sequence of Growth Hormone**
- **A key differentiator is a once-a-week injection (current products require daily injections)**
- Human growth hormone is used for:
  - Growth hormone deficient children and adults
  - SGA, PWS, ISS
- Final Presentation:
  - The drug product will be a refrigerated, liquid non viscous formulation
  - Injected using a disposable easy to handle pen device with a thin needle and low injection volume
- Phase 2 study in naive growth hormone deficiency pediatric population has been completed
- Orphan drug designation in the U.S. & EU for the treatment of children and adults with GHD



## DAILY hGH MARKET IS \$3B, UNDIFFERENTIATED, AND GROWING



### COMMERCIAL LAUNCH ANTICIPATED Q4 2015; FDA APPROVED ON SEPTEMBER 2, 2015

- Rolapitant out-licensed to Tesaro in December 2010
  - **Additional milestone payments of up to \$110 million to OPKO**
  - **Double-digit tiered royalties for OPKO**
- Differentiated cancer supportive care product with >\$1 billion U.S. 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
    - Long acting, sustained coverage
    - Lack of CYP 3A4 drug-drug interactions
    - Oral and IV formulations allow full market access

<sup>1</sup> Moderately emetogenic chemotherapy

<sup>2</sup> Highly emetogenic chemotherapy

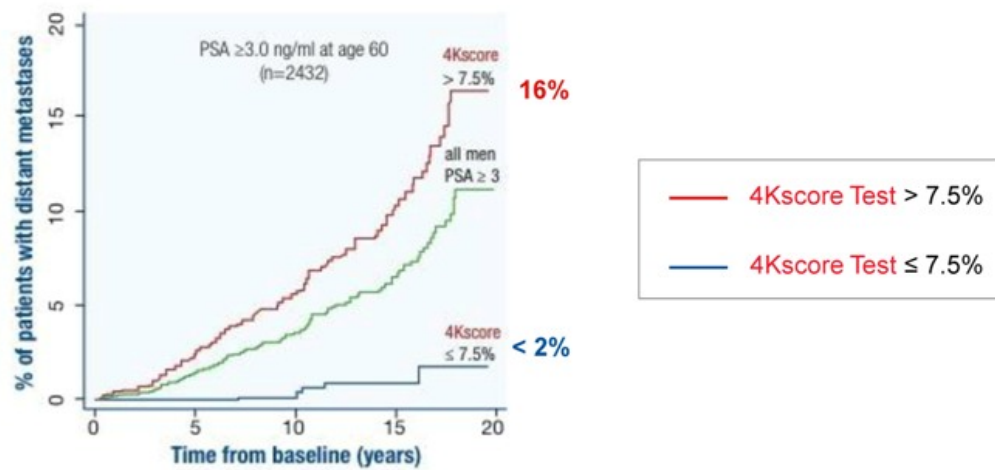
### **LEVERAGE BIO REFERENCE'S CHANNELS TO ACCELERATE ADOPTION OF OPKO'S DIAGNOSTIC PRODUCTS**

- Bio Reference Labs is the third largest full service reference laboratory in the U.S.
- Leverage the national marketing, sales, and distribution resources of Bio Reference Labs to enhance sales of OPKO's diagnostic platforms
  - ~420 sales and marketing personnel
  - ~5,000 people working together to support the needs of clients and patients
  - ~175 BRLI patient service centers located throughout the U.S.
- Bio Reference Labs generated \$832 million in revenue at 44% gross margins in FY 2014
  - Has grown at a 20% CAGR over the past 20 years
  - Will serve as a launching pad for the 4Kscore as reimbursement for the 4Kscore continues to be expanded

**MORE THAN 2 MILLION BIOPSIES PER YEAR WORLDWIDE**

- **4Kscore is the only blood test that accurately identifies risk for aggressive prostate cancer**
- Reimbursement to be expanded throughout the remainder of 2015 and 2016
- Clinical utility is based on three decades of clinical biomarker research and over 20,000 men tested in Europe and the U.S.
- Over 1,000 urologists have used the 4Kscore in routine practice
- Identifies the actual risk of aggressive prostate cancer for the individual patient with high grade prostate cancer pathology and poor prostate cancer clinical outcomes within 20 years
- 40–55% cost savings to potentially avoid unnecessary MRI and prostate biopsies

## THE 4KSCORE TEST PREDICTS METASTASES WITHIN 20 YEARS<sup>1</sup>



<sup>1</sup> Improving the Specificity of Screening for Lethal Prostate Cancer Using Prostate-specific Antigen and a Panel of Kallikrein Markers: A Nested Case-Control Study European Urology (in press)

## CLAROS 1 PLATFORM ADDRESSES LARGE TESTING MARKETS

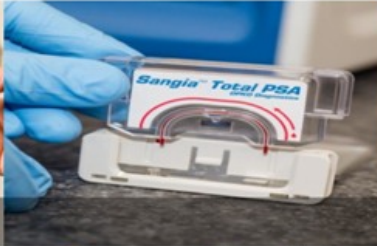


### 30 MILLION PSA TESTS IN THE U.S. GENERATING \$750 MILLION PER YEAR

- Modular PMA filing with the FDA expected in 2016
- Claros 1 Platform will leverage Bio Reference Labs' distribution and marketing
- Rapid Testing in the Physician Office with simple user steps:



Finger stick blood sample



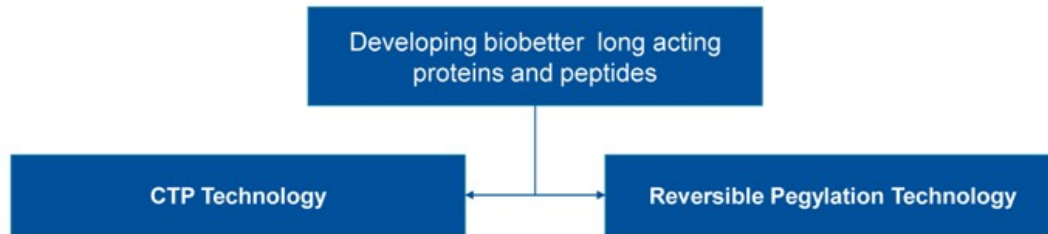
Snap cartridge into Sangia™ cassette



Insert cassette into Claros®1 analyzer



Quantitative Results



- Significant reduction in injection frequency
- CTP Technology safety profile is comparable to non-modified active therapeutic agents
- Potential expanded prophylactic uses with longer half life agents, in addition to short-term treatment
- Maintain drug bio-activity

**PHASE 2a STUDY TO COMMENCE Q4 2015 IN \$1.7 BILLION MARKET**

- **Market is 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, FVIIa-CTP demonstrated:
  - Potential for subcutaneous administration
  - Reduced frequency of injection during on-demand therapy
  - Enable prophylactic treatment while reducing the frequency of injections to 2-3 times a week
- **Orphan drug designation in the U.S. and EU**



MARKET SIZE FOR DIABETES AND OBESITY IS \$15 BILLION

MOD-6031  
(in animal studies)

SIGNIFICANTLY INHIBITS FOOD INTAKE AND REDUCES  
BODY WEIGHT BY REDUCTION IN FAT

IMPROVES GLYCEMIC CONTROL BY INDUCING GLUCOSE  
DEPENDENT INSULIN SECRETION (DIRECT MECHANISM)  
AND BY REDUCING FAT (INDIRECT MECHANISM)

IMPROVES LIPID PROFILE

IS EXPECTED TO PROVIDE EFFECTIVE LONG-TERM  
THERAPY FOR OBESE AND TYPE II DIABETES PATIENTS

- Phase 1 study to be initiated in Q1 2016
- Phase 1 study will evaluate the safety and pk-pd profile of MOD-6301 in overweight or obese healthy volunteers
- A battery of comprehensive toxicological studies have been completed confirming the safety of MOD-6031 following a single injection

## MOD-6031 – OPKO'S SOLUTION

**OPKO**

- Oxyntomodulin is a natural appetite suppressor and a dual GLP-1/Glucagon receptor agonist
- Secreted by the digestive system following food intake and induces satiety in the brain
- Crosses blood-brain barrier to induce satiety
- Increases glucose tolerance in insulin resistance pre-diabetic state associated with obesity

### DEVELOPMENT CHALLENGE:

Oxyntomodulin has short half-life which requires multiple daily injections

### OPKO SOLUTION:

MOD-6031, a reversible PEG<sub>30</sub> formulation which provides for once-a-week dosing frequency

