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Opko Health, Inc. Announces Update on Phase III Clinical Trial of Bevasiranib; Company

Decided to Terminate
Clinical Study

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MIAMI--(BUSINESS WIRE)--OPKO Health, Inc. (NYSE Alternext US:OPK)today announced that, following the recommendation of the Independent Data Monitoring Committee (IDMC), it had decided to terminate its Phase III clinical study of Bevasiranib, a first-in-class siRNA, for the treatment of wet age-related macular degeneration (wet-AMD). Although preliminary data, needing further analysis, show activity of Bevasiranib when used adjunctively with Genentech's Lucentis®, review of the data by the IDMC indicated that the trial, as structured, was unlikely to meet its primary end point. There were no systemic safety issues identified and local ocular safety was generally unremarkable.

"While we are clearly disappointed with the preliminary results of this fully-enrolled study, the indications of activity are encouraging and we look forward to fully analyzing the data in the coming weeks," said Dr. Phillip Frost, Chairman and CEO of OPKO Health. "We remain committed to the continued development of our siRNA portfolio targeting



including our recently announced VEGFA165b sparing siRNA." These new proprietary siRNA's are designed to inhibit the angiogenic Vascular Endothelial Growth Factor A165 (VEGFA165 isoform) but spare the anti angiogenic VEGFA165b isoform.

VEGFA165 is known to play a critical role in diseases of the eye where the underlying cause of the problem is abnormal growth of blood vessels, such as in wet age-related macular degeneration. To the contrary, data show that VEGFA165b is an inhibitor of abnormal vessel growth.

About OPKO Health, Inc.

Miami-based OPKO is a specialty healthcare company engaged in the development, marketing, and sales of novel agents and both diagnostic and therapeutic devices for the management of ophthalmic diseases. For more information visit the company's website at www.opko.com.

This press release 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," and other words of



regarding preliminary data, our product development efforts, our ability to significantly improve clinical outcomes in patients, and our ability to develop a preclinical pipeline of novel agents for ophthalmic diseases, as well as other non-historical statements about our expectations, beliefs or intentions regarding our business, technologies and products, financial condition, strategies or prospects. Many factors could cause our actual activities or results to differ materially from the activities and results anticipated in forward-looking statements. These factors include those described in our filings with the Securities and Exchange Commission, as well as risks inherent in funding, developing and obtaining regulatory approvals of new, commercially viable and competitive products and treatments. In addition, forward looking statements may also be adversely affected by general market factors, competitive product development, product availability, federal and state regulations and legislation, the regulatory process for new products and indications, manufacturing issues that may arise, patent positions and litigation, among other factors. The forward looking statements contained in this press release speak only as of the date the statements were made, and we do not undertake any obligation to update forward



looking statements be subject to the safeharbor provisions of the PSLRA.

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