



## PTC Therapeutics Announces Strategic Gene Therapy Licensing Agreement with Odylia Therapeutics

July 1, 2019

### - Collaboration expands PTC's Gene Therapy portfolio to rare ophthalmic diseases -

SOUTH PLAINFIELD, N.J. and BOSTON, July 1, 2019 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) and Odylia Therapeutics today announced a strategic collaboration to develop novel gene therapies in rare inherited retinal diseases (IRDs) utilizing the Anc80 vector system developed by researchers at Massachusetts Eye and Ear. The collaboration augments PTC's existing expertise in localized gene therapy applications in the Central Nervous System (CNS) with the deep ophthalmic experience at Odylia across a broad portfolio of IRD targets. The lead program in the collaboration will be RP-GRIP1, a ciliary protein that is defective in Leber Congenital Amaurosis 6 (LCA6), a rare early onset childhood retinal dystrophy.

"We are excited to expand our gene therapy efforts to include targets in rare, inherited retinal diseases, for which there is tremendous unmet need," said Mark Pykett, V.M.D., Ph.D., Chief Scientific Officer of PTC Therapeutics. "This partnership combines PTC's strength in developing and commercializing treatments for rare diseases with Odylia's novel vector platform and breadth of expertise in rare ophthalmic diseases. As with our CNS gene therapy programs, we will apply a targeted, micro-dosing gene therapy strategy to our IRD research and development."

Scott Dorfman, Chief Executive Officer of Odylia Therapeutics, added, "PTC is the ideal partner with their expertise in developing and commercialization of rare disorder treatments but most importantly, their commitment to patients. We look forward to the clinical translation of the Anc80 AAV platform and the RP-GRIP1 Program, as well as future programs in the partnership."

#### **About PTC Therapeutics, Inc.**

PTC is a science-led, global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. To learn more about PTC, please visit us on [www.ptcbio.com](http://www.ptcbio.com) and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.

#### **About Odylia Therapeutics, Inc.**

Odylia Therapeutics is a 501(c)(3) nonprofit corporation working to bring treatment for rare eye diseases from the lab into phase I/II/III clinical trials using AAV technology and other gene delivery techniques. Odylia was established in 2017 with support from Massachusetts Eye and Ear and the Usher 2020 Foundation, a charitable organization focused on finding cures for rare eye diseases. There are more than 250 genetic forms of Inherited Retinal Disease (IRD) that lead to blindness. Most are extremely rare, affecting only one in a million people worldwide. Today, there is pre-clinical proof-of-concept for treatments for more than 30 of these diseases, with more being discovered every day. Odylia Therapeutics was co-founded by Scott Dorfman of Usher 2020 Foundation, and Luk Vandenberghe, Ph.D., Eric Pierce, M.D., Ph.D., Joan Miller, M.D., and John Fernandez, M.S. of Massachusetts Eye and Ear. For more information, please visit [Odylia.org](http://Odylia.org)

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#### **Forward-Looking Statements:**

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements, other than those of historical fact, contained in this release, are forward-looking statements, including statements regarding the future expectations, plans and prospects for PTC; timing, results and conduct of PTC's collaboration with Odylia; expectations with respect to PTC's gene therapy platform; PTC's strategy, future operations, future financial position, future revenues or projected costs; and objectives of management. Other forward-looking statements may be identified by the words "guidance", "plan", "anticipate", "believe", "estimate", "expect", "intend", "may", "target", "potential", "will",

"would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including the factors discussed in the "Risk Factors" section of PTC's Annual Report on Form 10-K for the year ended December 31, 2018 as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

The forward-looking statements contained herein represent PTC's views only as of the date of this press release and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this press release except as required by law.

 View original content: <http://www.prnewswire.com/news-releases/ptc-therapeutics-announces-strategic-gene-therapy-licensing-agreement-with-odylia-therapeutics-300878335.html>

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