

## Alexion and Complement Pharma Announce Partnership to Co-Develop Pre-Clinical Complement Inhibitor for Neurodegenerative Disorders

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- · Collaboration provides opportunity to expand on Alexion's two decades of complement leadership
- Complement Pharma to receive up to a total of € 14 million in milestone-dependent payments through Phase 1b development
- Alexion has option to acquire Complement Pharma during the term of the agreement

NEW HAVEN, Conn., & AMSTERDAM--(BUSINESS WIRE)--Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) and Complement Pharma today announced a partnership to co-develop the preclinical C6 complement inhibitor CP010 for neurodegenerative disorders. C6 inhibition prevents the formation of the destructive membrane attack complex (MAC), a complex of terminal complement proteins that mediate cellular injury following complement activation, and has the potential to treat a variety of central nervous system disorders. Under the terms of the agreement, Alexion will provide Complement Pharma with up to € 14 million in milestone-dependent payments through Phase 1b development. The agreement also provides Alexion with the option to acquire Complement Pharma during the term of the agreement.

"Alexion has been a pioneer in complement biology since the development of Soliris, the world's first complement inhibitor, which has demonstrated the significant impact that C5 inhibition can have on several diseases caused by uncontrolled complement activation," said John Orloff, M.D., Executive Vice President and Head of Research & Development at Alexion. "This collaboration provides an exciting opportunity to expand on our more than two decades of complement expertise to potentially treat additional diseases by targeting C6, a different component of the complement system."

"Our understanding of the role of complement in many disorders continues to increase and many neurodegenerative diseases show activation of the complement system, even in early stages of disease," said Frank Baas, M.D., Ph.D., Chief Scientific Officer at Complement Pharma. "We believe that C6 inhibition has the potential to treat multiple neurodegenerative disorders, and we look forward to working with Alexion, a partner with a worldwide leading position in complement biology, to realize the possibilities of this approach."

Under the terms of the agreement, Alexion and Complement Pharma will collaborate on the development program for CP010. Complement Pharma will be responsible for conducting preclinical and Phase 1 studies and for manufacturing CP010. The phased agreement extends through the completion of Phase 1b development. Alexion has the option to acquire Complement Pharma during the term of the agreement.

CP010 is a humanized monoclonal antibody in preclinical development that binds to C6 in circulation to inhibit its function throughout the body by preventing MAC formation in both the periphery and the central nervous system. An end product of the activated complement cascade, MAC has been shown to play a role in neurodegeneration. CP010 has demonstrated C6 inhibition *in vitro* and *in vitro*; C6 inhibition prevents the formation of the MAC following complement activation which may allow treatment of a variety of central nervous system disorders.

## **About Alexion**

Alexion is a global biopharmaceutical company focused on serving patients and families affected by rare diseases through the innovation, development and commercialization of life-changing therapies. Alexion is the global leader in complement inhibition and has developed and commercializes the first and only approved complement inhibitor to treat patients with paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), and anti-acetylcholine receptor (AchR) antibody-positive generalized myasthenia gravis (gMG). In addition, Alexion has two highly innovative enzyme replacement therapies for patients with life- threatening and ultra-rare metabolic disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D). As the leader in complement biology for over 20 years, Alexion focuses its research efforts on novel molecules and targets in the complement cascade, and its development efforts on the core therapeutic areas of hematology, nephrology, and metabolic disorders. This press release and further information about Alexion can be found at: <a href="https://www.alexion.com">www.alexion.com</a>.

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## **About Complement Pharma**

Complement Pharma is a biotech company focusing on complement inhibition to modify neurodegenerative disease progression. The Company was founded in 2017 by Prof. Frank Baas and Robert Jan Lamers with the goal of translating the opportunities of C6 complement inhibition into clinical products for patients with acute and chronic neurodegenerative diseases. For more information, please visit <a href="https://www.complementpharma.com">www.complementpharma.com</a>.

## **Forward-Looking Statements**

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, including statements related to drug discovery, clinical development, development and regulatory milestones resulting from the agreement between Alexion and Complement Pharma, the potential benefits of CP010 (including the potential to treat a variety of disorders), the potential benefits of the inhibition of C6 and the potential benefits of the transaction to Alexion (including the expansion of complement experience). Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ materially from those indicated in these forward-looking statements, including for example, decisions of regulatory authorities regarding the adequacy of the research regarding CP010, the failure to reach the contractual milestones for CP010 set forth in the agreement, the failure to obtain marketing approval or material limitations on the marketing of our products or the potential products based upon CP010, delays, interruptions or failures in the manufacture and supply of such products and related product candidates, failure to satisfactorily address matters raised by the U.S. Food and Drug Administration and other regulatory agencies, the possibility that results of clinical

trials are not predictive of safety and efficacy results of our products in broader patient populations, the possibility that clinical trials of applicable product candidates could be delayed, the adequacy of our pharmacovigilance and drug safety reporting processes, uncertainties surrounding legal proceedings, company investigations and government investigations, including investigations of Alexion by the U.S. Securities and Exchange Commission (SEC) and U.S. Department of Justice, the risk that anticipated regulatory filings are delayed, risks relating to the potential effects of the Company's restructuring, and a variety of other risks set forth from time to time in Alexion's filings with the SEC, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended March 31, 2018 and in our other filings with the SEC. Alexion undertakes no obligation to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.