

Alexion and Sema4 Partner in Rare Disease Diagnostics and Systems Biology

Accelerating rare-disease diagnostics and treatments through innovation

NEW HAVEN, Conn. & STAMFORD, Conn.--(BUSINESS WIRE)-- Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) and Sema4 today announced a strategic partnership to leverage their shared experience in data science and systems biology to accelerate rare disease diagnosis and therapeutic discovery. This new partnership will combine Alexion's rare disease SmartPanel analytics with Sema4's proven success in next-generation sequencing and genomic interpretation to further enable novel diagnostic and therapeutic insights into rare diseases.

This Smart News Release features multimedia. View the full release here: http://www.businesswire.com/news/home/20170810005327/en/

"The world-class sequencing capabilities at Sema4 coupled with Alexion's SmartPanel analytics will enable a comprehensive evaluation of a patient's genome to uncover pathogenic mutations linked to rare diseases," said John Reynders, PhD, Vice President of Data Sciences, Genomics, and Bioinformatics at Alexion. "This collaboration will help accelerate an accurate diagnosis for patients with rare genetic diseases and reduce the multi-year process that many families face before receiving a diagnosis."

Alexion and Sema4 will also collaborate in searching for and decoding rare disease "genomic shields" - a buffering mechanism that enables patients with a known disease-causing mutation in their genome to resist the manifestation of the disease.

"The advanced data science and bioinformatics capabilities that Alexion and Sema4 both bring to the table will enable us to decode these unique patients who are resistant to their genetic propensity for disease," said Sema4 CEO, Eric Schadt, PhD. "By building systems biology models of the rare disease biology in these patients, we can identify novel points of therapeutic intervention for patients."

Under the partnership, Alexion will share, research, and further refine the SmartPanel, which Sema4 will leverage to optimize the design of next-generation sequencing solutions and accelerate the interpretation of genomic and phenotypic data. Both parties will combine expertise in data science and bioinformatics to decode the network biology of target systems and research the decoding of genomic shields.

About Alexion

Alexion is a global biopharmaceutical company focused on developing and delivering life-transforming therapies for patients with devastating and rare disorders. 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) and atypical hemolytic uremic syndrome (aHUS), two life-threatening ultra-rare disorders. In addition, Alexion's metabolic franchise includes two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D). Alexion is advancing its rare disease pipeline with highly innovative product candidates in multiple therapeutic areas. This press release and further information about Alexion can be found at: www.alexion.com.

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About Sema4

Sema4 is a health information company that is committed to providing open access to data and creating practical tools that help patients, clinicians, and researchers better diagnose, treat, and prevent disease. Sema4 is constructing a more comprehensive picture of health by combining a wealth of clinical experience that informs the answers that patients and providers are seeking, the world-class academic research that illuminates new directions, and the pioneering information science that puts all the pieces together. Sema4 is a venture of the Mount Sinai Health System, an integrated health system that is internationally recognized for its excellence in research, patient care, and education.

For more information, please visit <u>sema4genomics.com</u> and connect with Sema4 on <u>Facebook</u>, <u>Twitter</u> and <u>YouTube</u>.

Forward-Looking Statements

This press release contains forward-looking statements, including statements related to Alexion's diagnostic initiatives. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example, decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of our products, delays, interruptions or failures in the manufacture and supply of our products and our product candidates, failure to satisfactorily address matters raised by the FDA 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 current rates of adoption of Soliris in PNH, aHUS or other diseases are not sustained, the possibility that clinical trials of our product candidates could be delayed, the adequacy of our pharmacovigilance and drug safety reporting processes, the risk that third party payors (including governmental agencies) will not reimburse or continue to reimburse for the use of our products at acceptable rates or at all, the outcome of challenges and opposition proceedings to our intellectual property, assertion or potential assertion by third parties that the manufacture, use or sale of our products infringes their intellectual property, risks regarding 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, the risk that estimates regarding the number of patients with PNH, aHUS, HPP and LAL-D are inaccurate, the risks of changing foreign exchange rates, 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 June 30, 2017 and in our other filings with the SEC. Alexion does not intend to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

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Source: Alexion Pharmaceuticals, Inc.

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